

SICKLE CELL DATA COLLECTION PROGRAM REPORT

DATA TO ACTION



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Centers for Disease
Control and Prevention
National Center on Birth Defects
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INTRODUCTION

The Sickle Cell Data Collection (SCDC) program gathers health data from multiple sources to determine how many people live with sickle cell disease (SCD) in the United States and to learn about their use of healthcare services and health outcomes over time. The SCDC program shares this information with audiences who can drive improvements in SCD health care, treatment, and policy.

The primary goal of the SCDC program is to improve quality of life, life expectancy, and health among those living with SCD. Accomplishing this goal requires a joint effort across a variety of SCD stakeholders including providers, healthcare administrators, pharmaceutical companies, SCD community organizations, policymakers, public health organizations, and most importantly, patients and their advocates.

Continue reading to learn about the SCDC program, how the program has helped use data to drive action, and how it can equip SCD stakeholders with the evidence necessary to improve the lives of people with SCD.

ABOUT SCD

SCD is the most common inherited blood disorder in the United States, and affects an estimated 100,000 Americans.¹ People with SCD have abnormal red blood cells that prevent blood from reaching the body's tissues and organs, causing crippling pain, infection, and in some cases, stroke and other serious [health problems](#). Although improvements in health care and treatment 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.²

These poor health outcomes are likely influenced by

- *Limited or no access to quality, comprehensive care.* The number of medical centers across the country that specialize in treating SCD are limited. Furthermore, most people with SCD do not live near these centers. The number of physicians trained and willing to treat patients with SCD, especially adult patients, is also limited. The majority of patients with SCD are on Medicaid — a 2004 study found that 66 percent of hospital stays for SCD were paid by Medicaid.³ Fewer than 70 percent of doctors in the United States accept new Medicaid patients.⁴
- *Limited access to treatments.* Hydroxyurea (HU), the most common drug used to treat SCD, is often under-prescribed to patients who may need it most.⁵
- *Mistaken beliefs about people with SCD.* Healthcare providers may wrongly believe that patients with SCD are drug-seekers and may doubt patient reports of severe pain.⁶ As a result, those with SCD often wait longer than those without SCD to see a doctor and to get pain medicines when visiting the emergency department.⁷

SCD is also a significant public health issue globally. In many African countries where SCD is common, up to 3 percent of newborns may have the disease.⁸ Studies suggest that 50 percent to 90 percent of children born with SCD in resource-poor regions die before age 5.⁸ Data indicate that up to 16% of the deaths of children younger than age 5 in some of these areas may be due to SCD.⁹ However, SCD is often not diagnosed, so it is rarely listed as the underlying cause of death among children. For these reasons, not enough attention is given to finding SCD early and properly managing the condition. The international burden of SCD has a domestic impact because many immigrants and refugees may move to the United States from countries where the disease is most common, but potentially undiagnosed.

The estimated cost of care for SCD in the United States is about \$1.1 billion dollars per year,¹⁰ the majority of which is paid for through public insurance programs like Medicaid and Medicare. By understanding more about SCD through the SCDC program, the improvements to quality of life, life expectancy, and health may also result in reduced costs and less financial problems related to the disease.

GAPS IN KNOWLEDGE

Research has shown a great need for SCD data collection and analysis: 1 of the 5 recommendations of the 2007 American Society of Pediatric Hematology/Oncology Sickle Cell Summit was to use population-based surveillance (identifying all new cases of disease through monitoring of a defined population or group) to measure changes in health called 'health outcomes'.¹¹ The following year, the National Institutes of Health convened a State-of-the-Science Conference. An identified need from that conference was

“a surveillance system ... for patients with sickle cell disease who will be followed prospectively [their health will be monitored into the future]. This system should contain demographic [such as sex, education level, income level, marital status, etc], laboratory, clinical, treatment, and outcome information.”¹²

Although SCD is the most common inherited blood disorder in the United States, remarkably little is known about people affected by SCD. The SCDC program aims to address gaps in knowledge about SCD including the ones below.

How many people live with SCD?

Centers for Disease Control and Prevention (CDC) estimates that SCD affects more than 100,000 people in the United States, but the exact number of people with SCD is unknown because no national data collection system exists for the disease. The estimated number of people affected by SCD is often based on newborn screening and life expectancy data (taken from other data). In the United States, there are data systems for specific diseases, such as the National Program of Cancer Registries; however, there is no similar system for SCD, which could determine the actual or true number of people living with SCD and how SCD affects their health.

An incomplete picture of SCD

Much of the currently available SCD research relies on one of two sources of data: clinical data (data collected for research purposes such as data from electronic health records) or administrative data (data collected for record-keeping rather than research purposes). Clinical data includes reporting only on patients who seek care at SCD clinics (clinical care centers that specialize in care for people with SCD) but many patients, especially adults, with SCD do not have access to a SCD clinic and must receive care elsewhere such as the emergency department (ED). Therefore, research based on clinical data alone likely excludes a large number of people with SCD.

Administrative data, such as hospital discharge or state Medicaid data may include inaccurate reporting of SCD and related issues because the records may include patients who were miscoded or not coded for SCD. Research suggests that administrative data alone are unreliable because they may dramatically overestimate the number of people with SCD (by including patients who were miscoded for SCD) and thereby underestimate the use of healthcare services by true cases of SCD.¹³



Limited information about the course of disease

In the United States, all states have had universal screening for SCD since 2006 as part of state-administered newborn screening programs. However, little is known about what happens to these infants after the positive screening results are shared with the family and pediatrician. More SCD research is needed to better understand patterns around their

- Use of healthcare services (location of care and use of outpatient, emergency department, and hospital services)
- Uptake of recommended treatments (transcranial Doppler screening, transfusions, hydroxyurea, penicillin, immunizations)
- Healthcare outcomes (coexisting or co-occurring conditions, surgeries, stroke, death).

Beginning in the later teen years and into adulthood, patients often suffer from more frequent SCD-related complications than younger pediatric patients.¹⁴ These complications include pain crises, chronic pain, and avascular necrosis (death of bone tissue due to a lack of blood supply).¹⁴ The increase in complications during this time is likely due in part to a lack of access to proper care during transition from pediatric to adult care.¹⁵ More research is needed to gain a better understanding of the complex health needs of patients as they undergo this change or transition in care.

In addition, few studies have been carried out on older patients with SCD. Advancements in treatment and health care have extended life expectancy and changed the disease course for people with SCD. Recently, there has been enough people with SCD to study into older age. Consequently, little has been published about complications, coexisting (2 or more conditions the patient has at the same time) or co-occurring conditions (presence of both a mental health and a substance-use disorder), and the use of healthcare services among patients over the age of 45.

THE SCDC PROGRAM

The SCDC program, which collects health information about people with SCD to study long-term trends in diagnosis, treatment, and healthcare access, seeks to address gaps in knowledge. The SCDC program is the first data collection system of its kind, collecting population-based data from multiple sources over many years to present a comprehensive picture of the SCD population.



The SCDC program builds on previous SCD surveillance carried out under the CDC and National Heart, Lung, and Blood Institute's Registry and Surveillance System for Hemoglobinopathies (RuSH) pilot project; and the CDC's Public Health Research, Epidemiology, and Surveillance in Hemoglobinopathies (PHRESH) initiative.

Goals

The goal of the SCDC program is to improve quality of life, life expectancy, and health among those living with SCD. The SCDC program accomplishes this goal by collecting, summarizing, and reporting multisource, population-based, longitudinal data (data collected over time) for people with SCD to

- Establish a health profile of those with SCD
- Track changes in SCD health outcomes over time
- Ensure credible, scientifically sound information to improve standards of care
- Provide information to improve policy and healthcare practices.

California and Georgia have implemented the SCDC program; the long-term goal is to expand the program to multiple states and to establish SCD surveillance at the national level.

Role of SCDC partners

Centers for Disease Control and Prevention's National Center on Birth Defects and Developmental Disabilities (CDC/NCBDDD)

CDC/NCBDDD provides technical guidance and oversight for all SCDC activities, fostered by its extensive experience in designing, implementing, and analyzing data from population-based surveillance systems and disease-specific registries. Additionally, CDC/NCBDDD can leverage the knowledge of experts and partnerships with other federal agencies to ensure that SCDC meets the research, public health, and policy needs of the larger community. CDC/NCBDDD is responsible for the control of the content, resources, materials, and associated components of the project.

Participating States

SCDC funds two programs to carry out its surveillance program: Public Health Institute's 'Tracking California' program and Georgia State University's Georgia Health Policy Center (GHPC). These entities have the public health authority to legally access individual-level demographic, clinical, and healthcare visits information from a wide range of sources. They are responsible for collecting, analyzing, and housing the data. Tracking California and GHPC also convene statewide, multidisciplinary partnerships that help guide the focus, content, and information dissemination for the project.



CDC Foundation

CDC Foundation administers the memorandum of agreement with CDC/NCBDDD that defines the scope, responsibilities, and budget for activities related to this project. CDC Foundation has more than 22 years of experience successfully administering public-private partnerships across sectors on behalf of CDC's mission and priorities.

Funding partners

CDC Foundation currently receives funding for SCDC from four partners: Bioverativ (spin off of Biogen), Global Blood Therapeutics, Pfizer, Inc., and Doris Duke Charitable Foundation. Support from partners provides funding for field staff, partnerships with California and Georgia, and project-related travel and meetings.

Data

Data sources

Health scientists in participating states work with organizations to access

- Newborn screening records
- Death certificates
- State Medicaid claims
- Hospital and ED discharge data.
- Clinical data from SCD clinics

Once health scientists have access to these data, they link the datasets and de-duplicate patients appearing in multiple datasets; then they create a case file for each individual in the data system.

At this time, SCDC data do not include government data other than those from health agencies (no school records or immigration status), data directly from people with SCD (no surveys or biological information), or private sector data (no employment records).



Data storage

At this time, health scientists in their respective states maintain all individual level, identifiable data. Data-use agreements within each state and across state agencies require that these data are treated as any state-level protected health information (such as information about infectious disease or cancer registry data). This means

- Strict requirements regarding hardware, networks, and procedures for data storage and access
- Training and certification for personnel working with the data
- Commitment to data security and patient privacy at all levels of these state projects.

Identifiable data are not shared with other entities (including external researchers and federal agencies, such as CDC), but SCDC health scientists may work with external researchers to analyze these data as long as the data are only directly accessed by SCDC health scientists and results are reported in summary form.

Priorities

Based on feedback from an extensive number of stakeholders including national medical professional organizations, federal partner agencies, community-based organizations, clinicians, state agency staff, and patients and their advocates, the SCDC program focuses on 5 priority areas. These 5 areas were selected as priorities because they could be studied using SCDC data to help drive changes in policy or health care.

Geography of the patient population

SCDC data can be used to map the demography (size, structure, and distribution of a population) and geography (where people live) of people with SCD by county and subcounty. Healthcare providers or facilities visited by patients can also be mapped to identify the geographic challenges in gaining access to care.

Transition from pediatric to adult care

During the transition from pediatric to adult care, people with SCD often experience more frequent complications related to the disease. SCDC data can help researchers understand why people with SCD often have the most severe symptoms and use healthcare services most frequently during this transition.

Hispanic patients with SCD

1 in 10 people with SCD in the United States are Hispanic¹⁶. SCDC information can shed light on the challenges of identifying SCD among Hispanics, which includes Hispanics born outside of the United States.



Older patients with SCD

People with SCD are living longer, making it more important to use SCDC information to study common health problems—not just those related to SCD—that patients experience as they age.

The use of healthcare services

High use of healthcare services, including those in the hospital and emergency department, occurs among people with SCD. SCDC information can identify how often people with SCD use healthcare services and what factors may lead to periods of high use of these services.



IMPACT OF SCDC DATA

SCDC data are used to improve policy, healthcare practices, and treatments that in turn, improve the health outcomes and healthcare access for people with SCD. Findings from the SCDC program are shared with the public including providers, healthcare administrators, pharmaceutical companies, SCD community organizations, policymakers, public health organizations, and patients and their advocates. Improving the lives of people with SCD requires the combined efforts of these partners to put SCDC data into action. Examples of how SCDC data are helping and can continue to help partners serve the needs of people with SCD are described below.

Healthcare providers

The SCDC program offers valuable insights to healthcare providers about improving care for SCD. Ongoing SCD surveillance in Georgia has determined where patients with SCD live within the state and what services they are receiving, which is essential to identifying and addressing gaps. For example, SCDC data have highlighted disparities (differences) in services between urban and rural areas and between services for pediatric and adult patients.

County-specific data enable the SCDC program in Georgia to identify SCD “hot spots” (areas with higher concentrations of residents with SCD) and to make healthcare providers aware of them. “The data highlight that SCD is a significant problem in providers’ hometowns. In my presentations to these providers, these are the data that get their attention,” said James Eckman, MD, Professor Emeritus, Hematology and Medical Oncology, Emory University School of Medicine.

Dr. Eckman also uses data on death rates among people with SCD in presentations to dispel the myth that all patients with SCD still die young. “SCDC data show that people with SCD are now living longer than ever before and that providers need to think about health problems unrelated to SCD that older patients may develop.”

SCDC data are useful in the clinical care setting when interacting with patients with SCD and their families. Dr. Peter Lane, the director of the SCD clinic at Children’s Healthcare of Atlanta, has used SCDC death rate data to demonstrate the potential consequences of declining hydroxyurea (HU) use. Dr. Lane uses a chart that shows poorer health outcomes over time for patients who do not take HU compared with patients who do. This information shows teens with SCD how they can prevent complications such as organ damage and emphasizes the need for teens to take control of their health early on. By empowering patients with information about their own health and disease course, they are better prepared to self-advocate and to play an active role in decisions about their care.

“ The data highlight that SCD is a significant problem in providers’ hometowns. In my presentations, these data get providers’ attention.”

- James Eckman, MD



Healthcare administrators

SCDC data, which include both administrative and clinical data, can help provide a more complete picture of clinical practice to healthcare administrators who may rely on administrative data alone. In a California study, administrative data suggested, "...the rate of transfusion for acute chest syndrome in SCD was low when, in fact, the rate was much higher..." at the three institutions studied, providing an inaccurate depiction of clinical practice¹². The SCDC program uses multiple sources of data and more accurately reflects diagnoses related to SCD and the relevant procedures performed in clinical care settings.

Pharmaceutical companies

The landscape of care and treatment for people with SCD is expected to shift as pharmaceutical companies invest in testing SCD-related drugs in clinical trials. The SCDC program tracks people living with SCD and their health outcomes, which can help forward-thinking pharmaceutical companies determine

- Markets for new drugs and where these markets are located
- Uptake of new drugs at the population level
- Impact of new drugs on use of healthcare services
- Changes in health outcomes as a result of new drugs.

The SCDC program will establish a health profile of people with SCD living in Georgia and California before pharmaceutical companies introduce new drugs, providing a baseline for comparison with findings determined after these drugs hit the market. The wealth of information provided by the data helps these companies understand the unmet needs of the SCD population, how to allot funds for clinical trials, and how trends like mass migration are changing the landscape of the disease.

“Data is everything, it drives every resourcing decision we make,” says Jung Choi, Chief Business and Strategy Officer at Global Blood Therapeutics, a funding partner of the SCDC program. “We found out through the data that access to care was a big issue, a big hurdle. We realized that we needed to quickly increase our investment in health policy and government affairs.”

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**- Jung Choi,
Global Blood
Therapeutics**

SCD community organizations

Community organizations for people with SCD often need grants and funding for program-building. SCDC data can be an incredibly valuable addition to grant proposals to help make a more robust argument for funding support. When writing these proposals, an organization’s mission and funding needs are often communicated, but it is also important to write from the funder’s perspective. Funders are often interested in how their investments will help fulfill unmet needs. Organizations can use SCDC data in grant proposals as evidence of the problems that the funding will help solve by including information such as burden of disease, health outcomes, and health disparities.

SCDC data are also valuable in advocating for better healthcare access for people with SCD. For example, the SCDC program found that 1 in every 2 adults with SCD in California (about 1,500 people) live in Los Angeles (LA) County. The data showed no places in the county where adults with SCD could receive quality, comprehensive, and coordinated care. A team of SCD experts used these data to inform LA County’s Department of Health Services about the urgent needs of the SCD community. SCDC data, together with the determination and hard work of many partners, led to the establishment of the Martin Luther King, Jr. Outpatient Center, located within 5 miles of most patients with SCD in LA County, in order to address those needs.



Policymakers

Organizations such as the American Society of Hematology (ASH) meet with policymakers on Capitol Hill to raise awareness about SCD and to explain the health disparities faced by the SCD population. SCDC data can equip these organizations with answers to common questions asked by policymakers

- What does access to care look like for people with SCD?
- What are the costs associated with the health disparities of SCD?
- What are the gaps in care?
- Where do people with SCD live and how closely located are they to providers and healthcare services?

In order to address policy that can lead to tangible outcomes, such as improved reimbursement for care and treatment of SCD and increased SCD expertise among healthcare providers, policymakers must realize how SCD is affecting their citizens. SCDC data can be used to develop educational materials, such as maps based on an elected official's jurisdiction, tailored to policy audiences that demonstrate the landscape of SCD.



Other Department of Health and Human Services projects

The public health community is responsible for providing evidence of the important problems faced by people with SCD. SCDC data are critical to demonstrating these problems to the healthcare industry, policymakers, the SCD community, and the public. SCD issues, such as the peak in complications during young adulthood and inadequate primary care among older patients, must be widely shared with audiences who are able and willing to address these issues.

SCDC data can be incredibly useful in evaluating the impact of public health programs. Several federal initiatives require health outcomes data to document how well their activities are working. One such initiative is *Healthy People*, which provides science-based, 10-year national objectives to improve the health of all Americans. The *Healthy People 2020* SCD objectives, released in 2010, included

- Increasing the proportion of children with SCD who receive routine penicillin treatment called prophylaxis (has lowered the risk of death in children with SCD) from 4 months to 5 years of age.
- Reducing hospitalizations due to preventable complications of SCD among children 9 years of age and under.

These objectives were archived in 2015 due to a lack of a national-level data source. Once the SCDC program expands to include more states, it will close this data gap and prove incredibly valuable in demonstrating how well public health activities that aim to reach these objectives are working.

Additionally, the Health Resources and Services Administration's Sickle Cell Disease Treatment Demonstration Regional Collaboratives Program (SCDTDP), funds 5 regional networks to improve the prevention and treatment of SCD. The National Institutes of Health's Sickle Cell Disease Implementation Consortium (SCDIC) works to improve the care of adolescents and adults with SCD. SCDC data are able to enhance activities for the SCDTDP and SCDIC programs operating in Georgia and California.



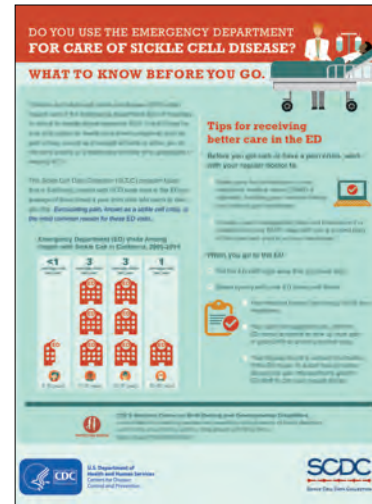
Patients and families

The SCDC program equips patients with SCD and their families with educational resources they can use to play a more active role in decisions about their care. These materials include

- [Videos](#) that capture stories about people living with SCD
- [Infographics](#)
- Blog articles
 - [“Sickle Cell Disease: Data Saves Lives”](#)
 - [“Sickle Cell Disease in the Emergency Department: Confronting Barriers to Care”](#)
- [Fact Sheets](#)
- [Quarterly webinars](#) presented by experts who share the latest about SCD
- [Social media content](#)

These products help empower patients to

- Self-advocate when engaging with healthcare professionals who may not be familiar with the complexities of SCD
- Stay up-to-date on the latest SCD research findings
- Connect with the larger SCD community.



FUTURE STEPS

Continue California and Georgia's work

The SCDC program's first priority is to continue work that has been occurring in California and Georgia. These states had the necessary infrastructure (resources) and institutional knowledge in place to participate in the program as soon as it began. One aspect of this infrastructure is the data sharing agreements that can take years to establish. If the work in California and Georgia is interrupted because of lack of funding or other reasons, these states would be required to restart the data sharing agreement process. Therefore, it is critical to maintain the work begun in California and Georgia and to continue to build a surveillance model that can be applied to other future states.

Expand the number of states participating in SCDC

It is vital that additional states engage in statewide, comprehensive surveillance of those living with SCD, as every state has a unique demographic makeup, distinct healthcare policies, medical and research centers, and access to care. As additional funding becomes available, the SCDC program seeks to create an 18 to 24 month telementoring-based training institute to improve knowledge and to provide access to California's database infrastructure. The training curriculum would include bimonthly (every two months) information-sharing sessions, monthly homework and milestones, and bi-annual (twice a year) in-person meetings that will prepare states to begin surveillance upon completion of training. Several states have expressed interest in participating in such a training institute and learning to apply a surveillance model that has proven successful.

Increase SCD knowledge

The SCDC program will continue to expand knowledge across the program's 5 priority areas by developing targeted, evidence-based educational materials and sharing them with the wide range of SCD stakeholders. SCDC communications activities will continue to be an essential priority, as the purpose of SCD surveillance is to share insights that will ultimately drive improvements in health care and treatment for people living with SCD.

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