

Insights

3 Ways to Close Gaps in Sickle Cell Disease Care: Recommendations from NICHQ Projects

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In the past several decades, clinicians, public health professionals, and those with lived experience have seen advancements in Sickle Cell Disease (SCD) treatments and research that have significantly improved outcomes and increased life expectancies for people living with SCD. For example, the FDA-approved medication [hydroxyurea](#) (HU) has been recommended as a [SCD standard of care](#) due to its ability to help people with SCD mitigate pain and the need for blood transfusions. Preventative measures, such as screening children and adolescents for risk of stroke and ensuring that all people who have SCD receive recommended vaccinations, have also been instrumental in reducing complications associated with SCD. And recently, development of gene therapies has presented possibilities of a new cure.

However, widespread quality care for this population is tempered due to gaps in services. Some gaps originate from an ongoing shortage of trained specialists who are willing to take care of people with SCD, while others are due to lack of provider knowledge about up-to-date guideline-based best practices when treating this population. Additionally, people who have SCD often

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experience episodes of extreme pain that are often most effectively treated with opioids. This has resulted in stigmatization of those living with SCD as a “drug seeking” population. Gaps in care also stem from a delay in SCD research progress, which has been caused, in part, by lack of funding. Notably, research for SCD, a condition that disproportionately affects Black and African American populations, continues to receive fewer research dollars when compared to Cystic Fibrosis, which affects fewer people in the U.S. and primarily white people. As stated in a recent [New Yorker article](#), “Sickle-cell disease traces the deep, long-standing inequities of American society. Defeating it will require confronting them.”

Sickle Cell Disease in the United States

Approximately 100,000 people in the U.S. live with SCD, a serious, debilitating, life-shortening, and often fatal condition, which disproportionately affects Black, African-American, and Hispanic-American populations, according to the Centers for Disease Control and Prevention. In the U.S., SCD is most common among African Americans. However, other racial and ethnic groups are affected, including Latinos and people of Middle Eastern, Indian, Asian, and Mediterranean backgrounds. An estimated 2,000 children are born each year in the U.S. with sickle cell disease. With advancements in treatment for SCD life expectancy for people living with the disease rose to 43 years in 2017, from 29 years in 1979 and 14 years in the mid-1970s.

NICHQ has been committed to improving care for patients with SCD for more than 10 years, including being the National Coordinating Center (NCC) for a recently funded Health Resources and Services Administration (HRSA) project, the Sickle Cell Disease Treatment Demonstration Regional Collaboratives Program (SCDTDRCP). The NCC worked with five regional coordinating centers (RCCs) with the goals of improving care delivery for people living with SCD, enhancing access to services, and expanding patient and provider education. During the project, collectively, the RCCs partnered with more than 100 health systems, clinics, and community-based organizations that served more than 25,000 people living with SCD.

For this project, the NCC engaged the RCCs to develop and create a comprehensive [Report to Congress](#), [Model Protocol](#), and [Compendium of Tools and Resources](#). The Report to Congress outlines the accomplishments of the project and includes recommendations for future SCD work. Many of the recommendations directly address the persistent gaps in SCD care and call out the considerations of health equity. Conversations on how to improve access to care should continue, and these three recommendations begin with some of the most pressing needs.

Recommendation 1:

Support the development and dissemination of standard models and/or curricula to improve knowledge related to SCD for all care team members.

Increasing and maintaining provider knowledge about SCD and up-to-date guideline-based standards of care is key to ensuring that people with the condition will receive quality care. Currently, training and education vary across provider types, with many general providers (e.g., primary care physicians, advance practice providers) receiving little or no initial or updated training about SCD. As a result, providers may not provide timely or best practice care despite the availability of evidence-based treatment, such as hydroxyurea and [transcranial dopplers](#).

Additionally, lack of understanding of the condition, its effects, such as pain episodes, and the need for timely and effective treatments means that stigma surrounding the disease persists. Because of the acute nature of SCD-related pain crises, people often need to be seen urgently in an emergency department. In these situations, while opioids are a highly effective treatment, they may be assumed to be "drug-seeking" when the reality is that they simply need pain relief.

Progress in care will require that all people who are part of the patient's care team increase their learning – not only physicians. Nurses, physician's assistants, social workers, and other healthcare professionals who interface with people living with SCD must be able to deliver quality, unbiased care.

Is there a cure for sickle cell disease?

Sickle cell disease is an inherited blood disorder caused by a mutation on the adult hemoglobin gene, which, despite its name, typically begins to present around the time of an infant's birth. In people who have SCD, their red blood cells "sickle" in shape. When this happens, they can stick together, blocking blood flow and oxygen from reaching all parts of the body. This causes pain, anemia, infections, and other health-related issues. Sickle cell disease is a life-long condition, but it can be largely managed to mitigate impacts on daily life. However, a wide-spread cure for sickle cell disease is a relatively new possibility. In 1984, bone marrow transplant was established as the first curative treatment for SCD. While success rates are high for those who have undergone the procedure, it is not a viable option for most. One challenge is finding suitable bone marrow donors. As well, prohibitive cost and the risk of death associated with bone marrow transplant are key reasons why this option remains rarely utilized.

More recently, there is hope of another potential cure for SCD, based on advances in gene therapy. Gene therapies take an individual's stem cells and program them to make fetal hemoglobin, which do not "sickle." The stem cells are then reintroduced to the body via intravenous infusion.

This type of gene therapy was first attempted in 2019 with promising results, but research on long-term outcomes will be needed to determine if this truly is a cure. As gene therapies become more widely available, cost is likely to remain a significant barrier. Current gene therapies for SCD cost around [\\$1 to \\$2 million dollars per patient](#). Many people living with SCD rely on Medicaid for health insurance, thus the expenses rule this out as an option for most people who need it.

While the idea of training providers nationwide is a daunting endeavor, telementoring has proven to be an effective strategy for increasing provider capacity to treat SCD patients. To aid provider education, the SCDTDRCP used the Project ECHO® (Extension for Community Health Outcomes) telementoring model. Developed at the University of New Mexico, this model employs an "all teach all learn" concept that connects providers with specialists, even though they may be geographically dispersed. Remote, interactive sessions allow specialists to provide both generalized information about best practices and individualized mentoring. This remote delivery of training may be especially helpful in reaching providers in rural and other underserved communities. During the SCDTDRCP, the total count of provider attendance at ECHO sessions exceeded 3,500, showing that when given an accessible opportunity, providers are able to

engage and learn from SCD experts.

Recommendation 2:

Engage and educate individuals living with SCD and their families about the use of therapies (i.e., HU, transfusion, other disease-modifying therapies) to improve quality of life

Provider education alone is not enough to ensure that SCD patients are receiving adequate care and treatment – patients and their families also need to understand recommended disease-management strategies and advocate for their health care. Even if providers are knowledgeable about SCD standards of care, the relay of this information to patients is not guaranteed. Last year, the New York Times published a story about two sisters living with SCD. Both experienced strokes that were likely preventable. A stroke can happen if sickled cells get stuck in a blood vessel and block blood flow to the brain, making it harder for the brain to get the oxygen it needs to function properly. A stroke is more common among people with sickle cell anemia, and about 10% of children with SCD will have a symptomatic stroke. Neither sister was aware of the recommendation that some children with SCD should undergo preventative screening with a Transcranial Doppler (TCD). A TCD is a type of ultrasound that is used to identify children who are at high risk of a stroke. For more than two decades, the National Institutes of Health has recommended that children with sickle cell anemia as well as those with additional disease characteristics get screened annually from 2 years old to 16 years old. Unfortunately, patients and caregivers still miss or do not receive information about this potentially lifesaving test. In 2017, less than 40% of children aged 2 to 16 with SCD received a transcranial doppler.

Incorporating shared decision-making between providers and patients/families is a norm and is important to ensure that information about important treatment and preventative care options is exchanged and understood. The practice of shared decision-making gives patients greater autonomy in making informed decisions about their care and has been encouraged by an expert panel from the National Heart, Lung and Blood Institute. The SCDTDRCP [created a repository](#) that holds both print and electronic materials to support the shared decision-making process. These materials can help providers present important information and guide the conversation and allow patients to take information home to process and think about questions.

Recommendation 3:

Support efforts to ensure that all people with SCD have consistent health insurance so they can access uninterrupted care and newly approved treatments.

While lack of provider and patient education about SCD care are significant barriers to treatment, if people with SCD are unable to seek care, lack of provider knowledge is a secondary problem. People with SCD need consistent, high-quality health care. Although the Affordable Care Act ensures that [people with SCD can no longer be denied insurance coverage](#) due to the condition, many of those living with the disease remain under or uninsured and a high number of people

with SCD are dependent on Medicaid. In 2017, there were [more than 40,000](#) Medicaid and CHIP beneficiaries living with SCD. [Racial disparities exist in insurance coverage](#) as well, a cause for alarm considering that SCD disproportionately affects Black and other minority populations. In 2019, Black individuals were [still more likely to be uninsured](#) than their white counterparts.

NICHQ continues to support quality improvement in SCD care with our participation in the [Hemoglobinopathies National Coordinating Center \(HNCC\)](#) project as a subcontractor to Abt Associates. The HNCC is bringing Treatment Demonstration Programs, Newborn Screening Follow Up Programs, and community health workers together to foster partnerships between clinicians and community-based organizations, which will strengthen the SCD system of care. The lessons learned from this work as well as from NICHQ's deep experience with prior SCD projects will be important as individuals and organizations work to improve the care and experiences of people living with sickle cell disease.

Although having Medicaid provides greater potential access to care than being uninsured, it's not always reliable. [From 2014-16, 40% of people with SCD who were insured by Medicaid in Georgia, where the prevalence of SCD is among the highest in the country, had at least one gap in their coverage. The average length of each gap was 11 months.](#) There are several administrative reasons why people may experience gaps in coverage. One factor is that individuals must renew their Medicaid coverage every year. While the process has been streamlined since the implementation of the ACA, there are still [barriers to renewal](#). For example, states may still request additional eligibility information via mail. If individuals don't respond in a specific timeframe or don't receive the notice, they risk being disenrolled. Another contributing factor to gaps in Medicaid coverage is frequency of eligibility checks. These checks look for changes in factors such as income that occur between the annual renewals that might make an individual ineligible to receive Medicaid coverage. People who work on an hourly or seasonal basis, and therefore may have fluctuating income, are vulnerable to losing coverage when eligibility checks occur frequently throughout the year.

Together, complications with the renewal process and frequent eligibility checks significantly contribute to gaps in coverage for Medicaid recipients in general and gaps in health coverage are particularly harmful to those living with SCD. Without coverage, SCD patients may be more likely to miss regularly scheduled appointments, which are essential for disease management and prevention of complications such as stroke. One way to ensure that people living with SCD have consistent health insurance is to advocate for blanket Medicaid coverage for this condition. If individuals were eligible for Medicaid solely based on having an SCD diagnosis, challenges related to eligibility checks and the renewal process would be eliminated. Since one's SCD diagnosis does not change over a lifespan, eligibility for Medicaid would be consistent and annual renewal could be automatic.

From Recommendations to Action

Improving outcomes for those living with SCD will require action at individual, institutional, and systemic levels to ensure meaningful change for this historically neglected and marginalized population. Sickle Cell Awareness Month is a prime opportunity to use available platforms to increase awareness and strive for improvement at all levels by elevating the voice of patients with lived experience and highlighting gaps in care that need to be addressed. In addition, it is

imperative that public health professionals, healthcare teams, and administrators are not only aware of the deficits in SCD care but actively seek connections to leverage existing programs and projects that address them and continue to develop and advocate for evidence-informed strategies and solutions for outstanding issues.