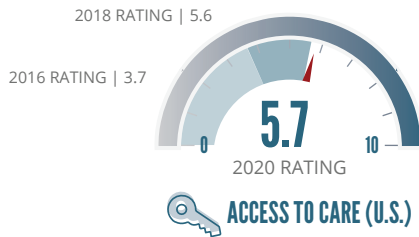


# STATE OF SICKLE CELL DISEASE | 2020 REPORT CARD

Sickle cell disease (SCD) — an inherited disorder that causes a person's red blood cells to become deformed and get stuck in veins, blocking oxygen flow throughout the body — can cause many complications, including severe pain, stroke, organ failure, and even death. There is no widely available cure for SCD, and care is inconsistent in the United States and even worse in large parts of the world. To highlight the urgent need for change, in 2016 and 2018 SCD stakeholder groups issued report cards on the state of the disease based on surveys of individuals with SCD, family members, caregivers, health care providers, researchers, advocates, industry representatives, and global health leaders. In 2020, SCD stakeholder groups conducted a follow-up survey to measure progress. While this 2020 report card suggests we are making progress in most areas, the scores also indicate that we have much to do to improve the state of care for those living with SCD.



## POSITIVE CHANGE

Greater awareness of SCD among health care providers and the recent development and dissemination of treatment information and guidelines are important steps forward. More treatment options (beyond pain relief alone), including the recently FDA-approved therapies, expand options for care. Passage of and funding for the Sickle Cell Disease and Other Heritable Blood Disorders Research, Surveillance, Prevention, and Treatment Act grant programs will improve public health efforts to address the needs of people living with SCD.

## WHERE WE STILL NEED TO GO...

In the United States, barriers to receiving quality, comprehensive, outpatient preventive and primary care for SCD remain, resulting in health care disparities and inequities. Steps are being taken to further identify and address access to quality care, especially for adults with SCD, who often face challenges accessing appropriate and coordinated care. Barriers to access can include lack of insurance, transportation needs, and provider inexperience and lack of knowledge about SCD. The opioid crisis has affected the ability of people to fill their pain relief prescriptions, and COVID-19 has disrupted care and increased challenges and mortality. Mistrust among patients and bias among providers continue to affect access to and quality of care.



## POSITIVE CHANGE

A number of recent efforts are underway across the country to train health care providers in SCD, and there has been more national activity focused on the need for professional education (e.g., SCD Training Module Course, Community Health Worker Training, SCD Training and Mentoring Program for Primary Care Providers, Emergency Department Sickle Cell Care Coalition Education Programs). New evidence-based guidelines for treatment published in the past year have been valuable to ensuring the medical community can better treat SCD. These efforts are in their early stages but hold much promise for expanding the pool of expertise in SCD treatment and care.

## WHERE WE STILL NEED TO GO...

Greater efforts are needed to increase uptake of professional education resources among providers across multiple specialties. Easily accessible point-of-care information for primary care providers can supplement training programs. Recruiting providers to specialize in SCD care (e.g., through fellowships) will expand the base of knowledgeable professionals, which is critically needed for adults living with SCD. Unfortunately, SCD care in the emergency setting remains a significant concern and requires expanded educational and training efforts for these providers.



## POSITIVE CHANGE

Several new efforts — a consortium, a coalition, and an alliance — have launched to demonstrate and scale-up newborn screening programs, therapeutic interventions, and provision of hydroxyurea in Sub-Saharan Africa where SCD is endemic. U.S. federal agencies, the European Union, global health leaders, foundations, and industry have expanded their efforts to address the global burden of SCD in partnership with local governments. These and other efforts have helped build local infrastructure for research and treatment.

## WHERE WE STILL NEED TO GO...

Despite progress, newborn screening and early intervention falls far too short in many parts of the world, particularly Sub-Saharan Africa and India. With limited support available for SCD care and services, the overwhelming financial burden of SCD across the globe often falls solely on the patient or family, who are likely already struggling to meet their basic needs. As a result, morbidity and mortality remain high and SCD remains a major cause of death for infants and children in low-resource countries. These regions need government strategies and financial support for newborn screening and early therapeutic intervention as a public health program, similar to childhood immunization initiatives. They also need more primary health care providers trained in SCD screening, treatment, and support services. SCD public awareness campaigns focused on health-seeking behavior and run by patient advocacy groups in partnership with health officials will promote diagnosis and treatment and improve health outcomes. Expanded awareness of SCD across the globe is needed to decrease stigma and discrimination.



## POSITIVE CHANGE

In the past two years, two new drugs have been approved for SCD in the U.S., and gene therapy trials show promise. Industry and federal investments in research have led to more clinical trials and critical infrastructure such as the Cure Sickle Cell Initiative led by the National Heart, Lung, and Blood Institute (NHLBI). The ASH Research Collaborative SCD Clinical Trials Network and Data Hub have been established and promise to accelerate research by centralizing data and facilitating clinical trial operations for SCD. Importantly, the SCD community is increasingly seeking out and asking questions about clinical research participation.

## WHERE WE STILL NEED TO GO...







While expanded infrastructure to facilitate SCD research has been developed since the 2018 report card, it will take time to see the benefits of these new programs. New therapeutics that offer more than minimal benefit over existing options and address the many different types of SCD symptoms are needed. Despite efforts to raise awareness about SCD research and new therapies, there is still a lack of clinical trial participation, which can be overcome by expanded education to the SCD community. While more clinical trials are being conducted, those living in more remote communities far from urban medical centers are often unable to participate in research.

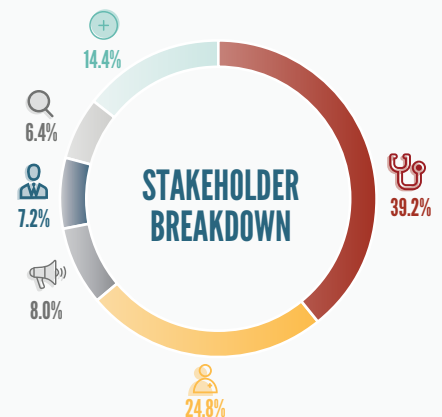
# STATE OF SICKLE CELL DISEASE | 2020 REPORT CARD

The organizations that endorse the **State of Sickle Cell Disease: 2020 Report Card** include:



## 2020 State of Sickle Cell Disease Survey Respondent Breakdown

-  HEALTHCARE PROVIDER : 39.2%
-  INDIVIDUAL WITH SCD AND/OR PARENT/SIBLING/RELATIVE/CAREGIVER OF INDIVIDUAL WITH SCD: 24.8%
-  SCD ADVOCATE AND/OR REPRESENTATIVE FROM COMMUNITY ORGANIZATION : 8.0%
-  INDUSTRY REPRESENTATIVE : 7.2%
-  RESEARCHER: 6.4%
-  OTHER (THOSE WHO IDENTIFIED WITH MULTIPLE GROUPS): 14.4%



For a more detailed look at the state of SCD, visit [scdcoalition.org/report](https://scdcoalition.org/report)