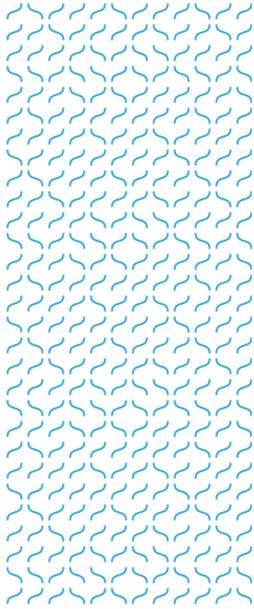


US Public Policy

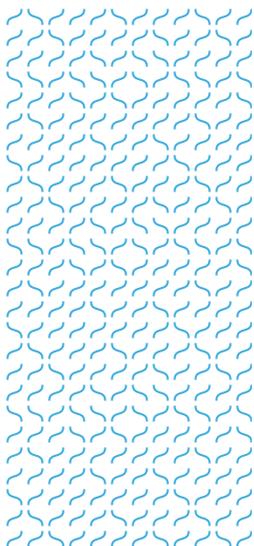


Sickle Cell Disease Policy Principles

September 2018



Sickle cell disease is a rare genetic blood disorder with about 100,000 patients impacted in the US, with its highest prevalence in African Americans and Hispanics.¹



At a Glance

Sickle cell disease (SCD) is a rare genetic blood disorder with about 100,000 patients impacted in the US, with its highest prevalence in African Americans and Hispanics.¹

The disease impacts:

1 in 365

African Americans²

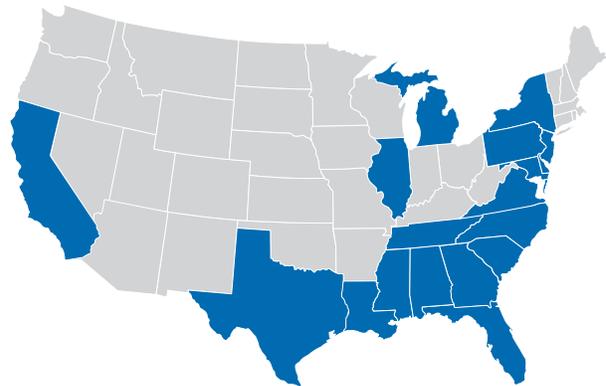
1 in 16,300

Hispanics²

SCD is prevalent in highly populated states and the south



SCD patients in the United States live in 18 states³



Improving access to treatment and care for sickle cell disease should be a public health priority.

The life expectancy for SCD patients is **approximately 30 years less** than for people without it.⁴ SCD affects every organ and body system and the most common complications are stroke, infection, eye disease, and pain episodes or crises.⁵ SCD patients often have both chronic pain and acute pain crises, called vaso-occlusive crises (VOC), which occur without warning and can last any length of time. VOC is a hallmark of SCD, causing severe pain and life-threatening complications including long-term damage to organs, hospitalization, decreased quality of life, and early death.⁶ SCD is a chronic and debilitating genetic blood disease for which there are few treatments and curative options.⁷

Overview

Vaso-occlusive crises (VOC) is commonly referred to as sickle cell-related pain crises. VOC can lead to multi-organ failure and increased mortality. Acute VOC is a common painful complication of the disease. SCD patients often have both chronic pain and acute pain crises, which can last any length of time.

Since 1998, the FDA has approved only two medications to treat the disease symptoms⁶. Opioids are recommended for pain management, although their usage, lack of disease awareness, and the current opioid crisis may lead to misperceptions of patients as “drug seekers” when they look to receive care in emergency departments for VOC.⁸

Cost of Care

Approximately 68% of children and 40% of adults with sickle cell disease have Medicaid coverage.^{9,10} While Medicare covers approximately 37% of adults with sickle cell disease, 21% of adults may be dually eligible for Medicaid.^{10,11}

SCD patients are considered some of the most expensive “super-utilizers” in Medicaid.¹² Estimated emergency room costs are \$356 million for approximately 232,000 emergency department (ED) visits.¹³ Inpatient care costs are estimated at \$488 million accounting for about 80% of overall treatment cost.¹⁴

Total medical costs for patients with SCD exceed \$1.1 billion annually, driven by high emergency department (ED) visits and inpatient stays.¹⁴



Access to Care

Most SCD patients are diagnosed at birth at pediatric sickle cell centers. In the United States, there are a disproportionate number of adult versus pediatric centers specializing in SCD.¹⁵

States with highest disproportion of adult versus pediatric care in SCD¹²

STATE	PREVALENCE	ADULT CENTERS	PEDIATRIC CENTERS	CENTERS TREATING BOTH	ADULTS PER CENTER	PEDIATRICS PER CENTER
NJ	3229	1	12	1	969	99
TX	6765	2	12	2	1015	193
CA	6474	5	18	1	647	136

Greater Research Funding Needed

The National Institutes of Health (NIH) and foundational research support for the disease is chronically underfunded. The National Institutes of Health recently announced the launch of the Cure Sickle Cell Initiative to accelerate the study of curative gene therapies for sickle cell disease (SCD). The US Food and Drug Administration (FDA) has identified SCD as a priority for additional research.¹ Only **two treatments** have been FDA approved since 1998.⁶ In contrast, hemophilia has an estimated population of about 20,000 in the United States and yet more available treatments than SCD.^{4,16}

The American Society of Hematology, a leading physician organization for blood disorders, recognizes there are not enough knowledgeable, experienced doctors, including specialists, to care for patients with SCD, especially adult SCD patients.¹¹

Such disparities have led to poor patient outcomes and life expectancy 30 years less than people without the disease.¹³

Establishing systemic mechanisms to improve the treatment of sickle cell disease and the prevention of sickle cell pain crises should be a public health and public policy priority.

Policy Recommendations

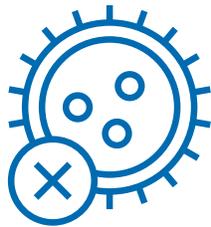
For patients with sickle cell disease, access to quality primary and speciality care is critical. Quality sickle cell care includes early screening and diagnosis, surveillance, and the availability of effective treatments to address pain crises or other disease symptoms. Education and increased awareness of factors that exacerbate VOC and assist in improving long-term health outcomes is essential for patients, caregivers, families, and providers.

Five Key Areas for Sickle Cell Policy Development



Policymakers should facilitate SCD patients' access to care, especially in Medicaid

- Develop novel reimbursement policies that improve patient access to quality primary and speciality care, including management services for acute pain episodes
- Encourage appropriate coverage and reimbursement of medicines and services that are determined necessary by a health care provider
- Ensure that innovative therapies utilizing novel mechanisms, to positively impact pain or other disease symptoms, are readily available



Address disparities in care to positively impact health outcomes and create sustainable healthy communities

- Support and enact legislative and regulatory initiatives to better address the high unmet needs of this patient population
- Ensure the existence of and compliance with non-discrimination policies that allow affordable access to health insurance as well as needed therapies and services for SCD patients
- Increase programs that will allow state Departments of Health, the CDC, and other stakeholders to gather data on screenings, health outcomes, complications, and treatments related to SCD
- Support the use of patient support programs and engage community-based organizations to ensure access and adherence to prescribed therapies



Ensure the ability of providers and patients to utilize care delivery models that employ quality measures and innovations to deliver improved patient outcomes

- Support efforts that improve access to high quality, coordinated, and comprehensive healthcare services through the development of innovative care models
- Create programs that allow pediatric sickle cell providers to continue providing care to young adults until an appropriate transition for lifetime care can occur
- Support medical facilities for pediatric and adult patients to receive specialized, coordinated, lifetime care for SCD
- Enable the creation and adoption of quality measures to guide care delivery and improve patient-relevant outcomes such as frequency and time to VOCs, hospital stay and readmission rates, patient experience, quality of life, and mortality
- Recognize the value created by new therapies and ensure patient access in order to delay or prevent the long-term damage from SCD and positively impact short- and long-term spend throughout the system



Recognize the need for increased education and awareness among stakeholders, including patients, caregivers, providers, policymakers, payers, and communities

- Support the creation and reauthorization of sickle cell disease awareness and treatment programs that provide funding, education and other services
- Educate healthcare professionals, patients, and their caregivers about importance of quality and continuous care, particularly when transitioning from pediatric to adult care
- Support education and training requirements for physicians, nurses and other healthcare professionals to improve understanding of the disease and mitigate possible misperceptions regarding pain crisis, particularly in the wake of the opioid crisis
- Support the educational efforts of states' Departments of Health by providing funding for sustainable community engagement and outreach
- Support the creation of SCD patient registries that will facilitate the collection of robust data nationwide related to the incidences and nature of SCD and treatment, allow for the education of primary care, specialty, and emergency physicians and other healthcare practitioners, guide more informed policy discussions, and increase public awareness of the disease



Develop and implement changes to enable patients and providers to access innovative therapies

- Develop innovative policies dedicated to expanding, coordinating, and implementing transition services for adolescents with SCD transitioning from pediatric to adult-focused healthcare
- Ensure timely and novel reimbursement policies for new therapies to ensure access for Medicaid, Medicare, and dual eligible patients
- Create pilot programs that allow manufacturers, payers, and providers to collaborate on enhancing the quality of care delivered, access to care innovations, and improving patient outcomes
- Support access to stem cell transplants and innovative therapies to treat SCD

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