



# Leveraging Data to Address Sickle Cell Disease

*Roundtable Briefing Paper*

June 2020

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# Executive summary

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Sickle cell disease (SCD) awareness and intervention is an increasing priority for government and the health sector. The federal government is addressing the need for SCD data collection to address the severity of the disease. This briefing paper serves as a resource for participants in the virtual *Roundtable on Leveraging Data to Address Sickle Cell Disease*, to be held on June 24, 2020 and June 25, 2020. The nonprofit Center for Open Data Enterprise (CODE) and the U.S. Department of Health and Human Services (HHS) Office of the Chief Technology Officer (CTO) will co-host the Roundtable, in partnership with the HHS Office of the Assistant Secretary of Health (OASH). The Roundtable will bring together medical experts, patients, and patient advocates to discuss the data needed to improve SCD diagnosis, treatment, and patient care.

This paper is divided into four sections, presenting an overview of the disease, the patient care journey, data collection efforts, and major themes in the data-driven approach to SCD. It explores the various dimensions of the disease from both the patient and provider perspective.

## Introduction

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Sickle cell disease (SCD) is the most common inherited blood disorder in the United States. There is a nationwide demand for population-level SCD surveillance data to address the needs of the sickle cell community. The federal government is taking steps to raise awareness and improve data collection for the disease. Based on these needs, the HHS Office of the Chief Technology Officer has partnered with the Center for Open Data Enterprise and &Partners to identify high-value datasets and potential interventions to improve care for patients with Sickle Cell Disease.

This project uses the Health+ methodology, now being utilized by HHS, which applies human-centered design, discovery, and prototyping to focus on specific, high-impact health needs. The Roundtable will be followed by a health-a-thon that applies insights from the Roundtable, and key datasets identified by its participants, to the management and treatment of SCD.

This document gives Roundtable participants a common framework for discussing SCD and relevant data issues. It draws on key insights from &Partners SCD Human Centered Design Report, and also develops data insights that build on those key findings. It presents research on available data, data gaps, and questions to be addressed by SCD-related data using a patient-centric perspective. The paper describes several themes for discussion at the Roundtable:

- Improving the continuity of care for SCD patients, with a focus on the transition from pediatric to adult care
  - Decreasing stigma, and the need to improve validation of SCD patients for the treatment of pain
  - Increasing awareness of SCD
- Improving SCD care in the emergency department (ED)
- Assessing treatment options and improving sustainable treatment
  - Improving SCD surveillance data
  - Improving treatment of SCD-related complications

# SICKLE CELL DISEASE OVERVIEW

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In recent years, the public health response to SCD has greatly increased awareness and understanding of the disease. There is a growing body of academic literature on the multiple dimensions of SCD symptoms, its prevalence, and treatment options. Public health officials now recognize that SCD is a major healthcare and societal problem that affects in the United States alone. Numerous offices within HHS have begun pilot programs and awareness initiatives for SCD, including the Centers for Disease Control and Prevention, the Office of Minority Health, and the National Institutes of Health. A number of new state programs have also emerged. This section outlines the types of SCD and SCD crisis episodes.

## Types of SCD

SCD is a group of inherited red blood cell disorders that affect hemoglobin, the protein in red blood cells that delivers oxygen to cells throughout the body. SCD is also known as Sickle Cell Anemia, due to the shortage of healthy red blood cells. SCD is most common among people of African ancestry (among whom 1 in 12 carry the gene), but also affects a small percentage of Hispanics. SCD can cause severe episodes of acute pain, and chronic pain, throughout an individual's life. Patients are often severely anemic and prone to infections as well.

In order for an individual to have the disease, he or she must inherit the hemoglobin S gene from at least one parent. Hemoglobin has two alpha chains and two beta chains, which are determined by the genes. Different genetic combinations produce four types of SCD.

- **Hemoglobin SS disease** is the most common and severe type of SCD. It occurs when a person inherits two copies of the hemoglobin S (Hb S) gene, one from each parent, and produces the type of hemoglobin known as Hb SS.<sup>1</sup> Approximately 20–25 million individuals world-wide live with HbSS, with an estimated 312,000 people born with the disease each year.<sup>2</sup> HbSS represents approximately 60% of SCD cases across the U.S.
- **Hemoglobin SC disease** is the second most common type of SCD, and occurs when an individual inherits the Hb C gene from one parent and the Hb S gene from the other. People with Hb SC have similar symptoms to those of HbSS, but the anemia is less severe. HbSC represents about 30% of SCD cases across states.<sup>3</sup>
- **Hemoglobin SB+ (beta) thalassemia** decreases the production of a component of hemoglobin called beta globin. Symptoms are not as severe as with other forms of SCD. Hb S Beta Thalassemia represents about 10% of SCD cases across states.

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<sup>1</sup> Healthline Editorial Team, "Sickle Cell Anemia." Healthline, March 29, 2017. Retrieved from <https://www.healthline.com/health/sickle-cell-anemia>.

<sup>2</sup> Saraf, Santosh L. et al. "Differences in the clinical and genotypic presentation of sickle cell disease around the world." Paediatric Respiratory Reviews, March 2014. Retrieved from <https://doi.org/10.1016/j.prrv.2013.11.003>.

<sup>3</sup> Hassel, Kathryn H. "Population Estimates of Sickle Cell Disease in the U.S." American Journal of Preventive Medicine, April 2010. Retrieved from <https://doi.org/10.1016/j.amepre.2009.12.022>.

- **Hemoglobin SB 0 (Beta-zero) thalassemia**, the fourth and rarest type of SCD, also affects the beta globin gene. HbSB thalassemia has similar symptoms to Hb SB+ anemia, but symptoms can be more severe.

The impact of SCD varies greatly between individuals. While some people with the disease live fairly normal lives, others can suffer from a variety of complications. SCD can cause organ and tissue damage over time, and people with SCD have shorter life expectancies than the general population.

Sickle cell trait (SCT) is the condition in which a person receives a sickle cell gene from one parent and a normal hemoglobin gene from the other.<sup>4</sup> People with SCT rarely produce symptoms or problems related to SCD and do not develop SCD as they grow older. However, they are carriers for SCD and may pass it on to their children.

### SCD Crisis & Pain

There are a few types of SCD crisis episodes, pain that can begin suddenly and last several hours to several days. Vaso-occlusive crises (VOC) occur when atypical hemoglobin molecules, called hemoglobin S, distort red blood cells into a sickle, or crescent, shape. The misshapen cells get stuck in blood vessels causing strokes, organ damage and episodes of agonizing pain as muscles are starved of oxygen. Acute chest syndrome is a condition where VOCs occur in the lungs as a result of infection. Aplastic crises are caused by a common childhood infection with parvovirus B19 and occur when bone marrow does not produce enough healthy red blood cells. Splenic sequestration is a crisis that occurs when sickled red blood cells build up within the spleen, enlarging it. Skull bone infarction as a result of SCD can cause bone pain crises as well.

SCD patients experience two types of pain: acute and chronic. Acute pain typically develops as a result of a VOC and should be treated as a medical emergency. Chronic pain is characterized by pain that lasts three to six months or longer. The cause of chronic pain is unclear, but may be an extension of recurrent painful episodes. This type of pain is often associated with neuropathic pain, which is caused by nerve damage, and occurs in 29% of adult SCD patients, and 40% of children and adolescent patients.<sup>5</sup> Children typically return to normal between crises, but teenagers and adults sometimes suffer chronic pain.

&Partners conducted interviews with a variety of patients, providers, and other relevant stakeholders to examine the needs and concerns of each group. From the interviews, they created a human centered design report to present their findings. One of the SCD patients interviewed in the Health+ methodology shared their crisis pain experience. “When my pain medicine at home is not working and I have done everything I could possibly do [...], you know it’s time to go. If you catch it early you can keep a lot of the long hospital stays to a medium, it won’t be so long, but if you wait around, it could be bad. [...] Once you start to flare, that means the oxygen is not flowing

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<sup>4</sup> Kark, John. “Sickle Cell Trait.” Howard University School of Medicine, December, 2000. Retrieved from [https://sickle.bwh.harvard.edu/sickle\\_trait.html](https://sickle.bwh.harvard.edu/sickle_trait.html).

<sup>5</sup> McGann, Patrick T., et al. “Current Management of Sickle Cell Anemia.” Cold Spring Harbor Perspectives in Medicine, August 2013. Retrieved from <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3721270/>.

to veins well and then you have to worry about your organs, not getting the proper blood flow."  
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## Treatment

While there are ample treatment options for pain management of SCD, many are underutilized by patients and physicians. Many patients are not on helpful therapies like hydroxyurea due to a lack of knowledge about the treatments, fear of side effects, fear of lack of proper optimization, and distrust of healthcare providers. Some of the treatment options are exceptionally costly, but a number of the options are covered by insurance.

- **Hydroxyurea therapy** – This therapy decreases the frequency of SCD crisis episodes by increasing levels of fetal hemoglobin (Hb F) in a patient's blood.<sup>6</sup> Hydroxyurea is a promising drug for SCD treatment. However, it does not affect patients in consistent, predictable ways and is not regularly prescribed.
- **Chronic blood transfusions** – This therapy is effective for stroke prevention, acute chest syndrome treatment, and management of SCD, but is required indefinitely for maximum benefit. Use of this method is increasing. According to previous studies, “Over a 10-year period, blood use in adults in a UK center increased from 1.7 to 3.86 units per patient per year, and rate of transfusion during acute admissions in children in the US increased from 14.2% to 28.8%”.<sup>7</sup>
- **Hematopoietic stem cell transplantation (HSCT)** – HSCT can establish donor-derived erythropoiesis, the production of new blood cells.<sup>8</sup> More importantly, it can stabilize or restore function in affected organs such as the central nervous system (CNS) and lungs and prevent further deterioration of function. HSCT is the only curative therapy for SCD and offers long-term survival rates, but its use is limited by the need for appropriate HLA matched donors.<sup>9</sup> HSCT with HLA matched sibling donors have shown high survival rates. Just 737 SCD patients received HSCT treatment between the years of 2013-2017.<sup>10</sup> This treatment is still in an experimental phase and is too novel to predict long-term outcomes.

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<sup>6</sup> Charache, Samuel, et al. “Effect of Hydroxyurea on the Frequency of Painful Crises in Sickle Cell Anemia.” *Journal of Medicine*, May 1995. Retrieved from <https://www.nejm.org/doi/pdf/10.1056/NEJM199505183322001>

<sup>7</sup> Howard, Jo. “Sickle cell disease: when and how to transfuse.” *The American Society of Hematology*, December 2, 2016. Retrieved from <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6142434/>.

<sup>8</sup> Walters M., Hardy K., Edwards S., Adamkiewicz T., Barkovich J., Bernaudin F., et al. “Pulmonary, gonadal, and central nervous system status after bone marrow transplantation for sickle cell disease.” *Biology of Blood Marrow Transplant*, February 2010. Retrieved from <https://www.sciencedirect.com/science/article/pii/S1083879109004613>

<sup>9</sup> Kassim, Adetola A., Sharma, Deva. “Hematopoietic stem cell transplantation for sickle cell disease: The changing landscape. *Hematology/Oncology and Stem Cell Therapy*, December 2017. Retrieved from <https://www.sciencedirect.com/science/article/pii/S1658387617300444>.

<sup>10</sup> “Transplant Activity Report Covering 2013-2017.” *Center for International Blood and Marrow Transplant Research*, December 12, 2018. Retrieved from <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6142434/>.

Another type of rarely used gene therapy is the process of genetically altering an SCD patient's hematopoietic stem cells to increase production of Hb F.<sup>11</sup> This form of gene therapy differs from HSCT in that it involves the direct modification/alteration of the patient's stem cells. Another method of gene therapy involves the use of CRISPR, a tool that allows scientists to snip out parts of genes and paste in new sections. The most advanced commercial approach is a new iteration of gene therapy by Bluebird Bio, but costs for the therapy are set at \$1.8 million per patient.

A few other treatment options are available for patients and covered by insurance, but aren't well known or widely used.

- **L-Glutamine**, an amino acid, is a pharmaceutical-grade product known to reduce pain crises in SCD patients. It can be used in combination with hydroxyurea and is non-toxic.
- **Voxelotor** and **Crizanlizumab** are two new drugs being made by specialty pharmacy companies and have been granted orphan status and FDA approval for expanded access. Such specialty pharmacies provide expensive specialty and outpatient drugs that treat rare and complex conditions like SCD, HIV, and autoimmune diseases.
  - **Crizanlizumab** is still in early stages of use, and is very expensive, but fully covered by insurance if the patient is eligible.
  - **Voxelotor** is also approved by insurance, and is suggested for those with more severe forms of anemia. Voxelator is still in orphan status with preliminary FDA approval for use. It is paid for insurance and available in pharmacies.

## *Pain Management*

When acute pain is caused by VOC, analgesic drugs should be started within 30 minutes of arrival to a hospital, or onset of crises. Non-steroidal anti-inflammatory drugs (NSAIDS) such as diclofenac and ibuprofen, and weaker tablet opioids are commonly used to treat moderate acute pain from home first. If pain worsens from manageable to moderate or severe, patients typically go to their ER to receive intravenous opioids, with moderate pain requiring weaker opioids like codeine, and severe pain requiring stronger opioids like morphine, levorphanol, or fentanyl. SCD patients often have poor vein access, so under-the-skin (subcutaneous), rather than intravenous (into veins) administration is recommended. To avoid repeated injections, a continuous infusion is recommended, known as patient-controlled analgesia (PCA), which may allow the patient to better control their pain.

High dose opioid-based regimens are the backbone of treatment for both acute and chronic SCD pain. However, due to the well-known side effects of chronic opioid use, alternative treatments for chronic pain are critically needed. Some patients who have undergone bone marrow transplantation to cure SCD continue to have chronic opioid requirements for at least six months post-transplant.

SCD patients often require large opioid doses due to increased opioid metabolism and clearance, as well as a high tolerance for opioids. Some SCD patients display opioid-induced *hyperalgesia*,

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<sup>11</sup> "Gene Therapy." Sickle Cell Disease News. Retrieved from <https://sicklecellanemianews.com/gene-therapy/>.

meaning hypersensitivity to pain. This chart below visualizes clinical pain expression over a patient’s lifespan, and incorporates the changes in fetal hemoglobin (HbF) and sickled hemoglobin (HbS) levels over time.<sup>12</sup> While HbF is primarily produced for oxygen transport in the fetus, young children may still have some levels of HbF, which can help protect them temporarily from the pain associated with SCD.

	Infancy (0–23 months)	Toddler (2–4 yrs)	Childhood (5–12 yrs)
<b>Clinical Pain Expression</b>	<ul style="list-style-type: none"> <li>• HbF elevated</li> <li>• Minimal pain</li> <li>• Acute intermittent pain and dactylitis (“hand-foot syndrome”)</li> <li>• Splenic sequestration</li> </ul>	<ul style="list-style-type: none"> <li>• HbF reaches nadir</li> <li>• Acute, intermittent pain events resulting in emergency department visits and hospitalizations</li> </ul>	<ul style="list-style-type: none"> <li>• Continued acute, intermittent pain events resulting in emergency department visits and hospitalizations</li> </ul>
<b>Potential Pathophysiology</b>	<ul style="list-style-type: none"> <li>• HbF protective against effects of HbS</li> <li>• Acute vascular occlusion and tissue ischemia by sickled cells</li> </ul>	<ul style="list-style-type: none"> <li>• Decrease in HbF accompanied by increased HbS</li> <li>• Acute vascular occlusion and tissue ischemia by sickled cells</li> <li>• Chronic inflammation</li> </ul>	<ul style="list-style-type: none"> <li>• Continued acute vascular occlusion and tissue ischemia by sickled cells</li> <li>• Chronic inflammation</li> </ul>

Time course of clinical pain expression over the lifespan of patients with sickle cell disease. Retrieved from <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5350013/table/T1/?report=objectonly>

## SCD Data: The Patient Journey

A patient’s care journey typically starts with a positive result from a newborn screening, and a patient’s first visit with an accompanying parent to a physician. Unfortunately, little is known about the standard treatment of care after a child screens positive for SCD and starts seeing a primary care provider (PCP). Each individual’s medical journey has unique challenges. In the case of children who failed to be screened, or were screened incorrectly, SCD patients generally present symptoms in the first year of life. Most children with SCD experience more severe medical consequences of SCD over time, even including strokes, that affect their lives differently. As children grow older, they are less able to participate in certain activities like sports and school events due to acute pain crises and their associated complications.

Pediatric care in the emergency department (ED) varies from adult ED care. Pediatric EDs are typically more trained in SCD care, and have a greater bandwidth to treat more patients.<sup>13</sup> These physicians tend to believe pediatric patients and their caregivers when they describe the young

<sup>12</sup> Brando, Amanda M., et al. “Sickle cell disease: a natural model of acute and chronic pain.” Journal of the International Association for the Study of Pain, April 1, 2020. Retrieved from <https://dx.doi.org/10.1097%2Fj.pain.0000000000000824>.

<sup>13</sup> &Partners, Office of the Chief Technology Officer. [CONFIDENTIAL] “Health+ Sickle Cell Disease HCD Report.”

patient's pain more than they may believe adult patients. Conversely, adult EDs are often overcrowded, busy, discriminatory, and lack SCD expertise. Many patients are seen as drug-seeking because physicians are unable to quantify the symptoms/pain of SCD patients.

Pediatric patients typically experience swifter care and are admitted faster than adults with SCD. This includes both patients who present symptoms in the first year of life and those who develop chronic pain and other severe symptoms between ages 6 and 10. While physicians will prescribe therapeutic treatments like hydroxyurea and analgesic treatment, they generally avoid prescribing opioids to younger patients as a means of pain management.

Chronic pain from SCD begins to develop around adolescence, often resulting in hospital stays that affect school attendance. By adulthood, patients may experience chronic daily pain affecting school and work, and more frequent acute pain crises exacerbated by chronic pain. Adult patients experience lengthier hospital stays, and experience more bias in care: They may be seen as drug-seekers by physicians who are not aware that they have SCD or who don't believe that they do. Adults with SCD experience longer ED waits, refusal of prescriptions, and a lack of trust overall from the health care system.

The [Sickle Cell Disease Foundation](#) of California (SCDFC), founded in 1957, provides a model for the kind of comprehensive care that would benefit patients with SCD, who often experience other health problems as well. The SCDFC is the first and oldest non-profit, social service, sickle cell disease organization in the United States. Their clinic aims to address the needs of individuals with sickle cell disease and their families by emphasizing educational and support programs and services that meet the physical, psychosocial, and economic needs of clients. Clinic patients see both a hematologist and PCP, with behavioral therapists also available. One of the clinic's hematologists stated, "Having the primary care provider sitting with me, we cover 90% of what the patient needs and it's very efficient."<sup>14</sup> This combination of expertise effectively addresses the complex health needs of patients with SCD.

## *Mapping the Care Journey*

As part of this Health+ project on SCD, the HHS office of the CTO and &Partners convened a workshop and a series of interviews to map out the "care journey" that SCD patients experience. The following sequences describe an SCD patient's typical care journey, transition to adult care, and ED journey, including the kinds of data that may be useful at each stage. Please consult the Sickle Cell Disease Human Centered Design Report for additional information and detail.

## TRANSITION FROM PEDIATRIC TO ADULT CARE

**Living with SCD as a Child** – Caregivers bear the burden of all care coordination, decisions, life interruptions, and more. The following events represent the primary dimensions of caregiving.

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<sup>14</sup> "MLK Jr Outpatient Center for Adults with SCD." Sickle Cell Disease Foundation. Retrieved from <http://www.scdfc.org/scd-clinic-at-mlk.html>.

1. Coordinating Primary Care – This phase involves coordination of finding and keeping a PCP for the patient. When coordinating care, caregivers often work with pediatricians who understand SCD well. They typically have just one provider, but may have a separate hematologist as well. The caregiver also makes all decisions in terms of healthcare, treatment, and administrative work. Pediatric care customarily seeks more curative treatment options.
2. Paying for Care – Paying for care involves all costs associated with SCD care. Caregivers bear the burden of finding health coverage and handling unexpected costs that may result from hospital visits or new prescriptions.
3. Going to the ED – This phase involves the events leading up to an ED visit and actions taken by the caregiver, the ED experience, and post-ED coordination. Pediatrics EDs are typically more knowledgeable about SCD, less busy, and able to provide proper attention to patients.
4. Hospitalizations – This phase highlights the typical process SCD patient caregivers experience when hospitalized. Hospitalizations cause pediatric SCD patients to miss school. Caregivers must handle the care coordination, which can result in disrupted family dynamics.
5. Going to School – Typical procedures and actions SCD caregivers must take when the disease impacts school life. As for school life for SCD patients, caregivers manage negotiations and protocol with the school, including missing classes.

**Living with SCD as a Young Adult** – Without caregivers as a buffer, they face many new and unfamiliar barriers, but also desire to be independent.

1. Coordinating Primary Care – When coordinating care as a young adult, it is difficult to find providers knowledgeable about SCD, and patients must also take on the burden of coordinating multiple specialists they did not require before. In addition to having to make healthcare decisions on their own, young adults must continue developing precautionary measures. There are also fewer curative treatment options available as patients get older.
2. Paying for Care – Young adults with SCD often lack knowledge on how to choose insurance, including what to look for, and when to enroll. They also struggle to self-finance at a young age.
3. Going to the ED – The adult ED is often busy, lacks SCD expertise, and is discriminatory. ED providers are often suspicious of young, potentially “drug-seeking” SCD patients.
4. Hospitalizations – Young adult SCD patients are primarily responsible for their medical decisions and coordinating work and school disruptions.
5. Work and School – Patients are responsible for communicating needs to their employers, teachers, and professors. Caregivers may still provide help to patients if it involves school.

One Health+ interviewee stated, "Transition age is when you start to encounter the discrimination a lot more, because you're not a cute little kid anymore that everybody has empathy for. You're an adult, you're grown and so they change, you receive different treatment."<sup>15</sup>

## THE ED JOURNEY

**Phase 1: Crisis Onset** – The crisis onset phase begins when a patient begins to feel symptoms at home but the symptoms do not improve. They then make the decision to head to the ER. Below are some major challenges that a patient might face during a Crisis Onset.

1. The SCD patient starts to feel symptoms, most of which are unpredictable.
2. Patient tries to manage crisis at home, but this can pose interruptions in their daily life. For example, the patient may be unable to complete basic tasks, and organ damage can sometimes occur due to prolonged crises.
3. Patient's crisis doesn't improve over time. This can occur due to the lack of preventative treatment options.
4. Preventive treatment.

<u>Data needs and opportunities</u>	<ul style="list-style-type: none"> <li>● Preventive care data</li> <li>● Pain data to identify type of crisis</li> <li>● SDOH data</li> <li>● Prevalence of opioid-prescribing pharmacies</li> <li>● Transportation data</li> </ul>
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**Phase 2: Preparing for the ED** – The preparation for the ED phase involves identifying the most suitable ED for a patient to attend. During this phase, the patient must make all the necessary arrangements in preparation for their absence.

1. Even after a patient identifies the most "familiar" ED to visit, patients face challenges in an ED's capabilities and awareness in treating SCD. For example, sometimes the best ED for a SCD patient is not always the geographically closest ED.
2. SCD patients must plan accordingly to be gone for an indefinite amount of time, in case of a hospitalization. Patients must consequently handle a number of unforeseen obstacles while in pain, including finding childcare and missing an indefinite number of days of work.
3. Once the patient decides they will go to the ED, they solicit advocates to assist them during their time of uncertainty. Advocates can help reach PCPs or hematologists at odd hours, and are available to accompany the patient and communicate if needed.

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<sup>15</sup> &Partners, Office of the Chief Technology Officer. [CONFIDENTIAL] "Health+ Sickle Cell Disease HCD Report."

4. The patient heads to the ED. Patients face challenges like not being able to drive themselves, difficulty taking public transportation, and lack of funds for alternative transportation services.

<u>Data needs and opportunities</u>	<ul style="list-style-type: none"> <li>• ED availability and location data</li> <li>• SCD clinic locations</li> <li>• Transportation data</li> </ul>
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**Phase 3: Arriving at the ED** – This phase begins when the SCD patient arrives at the ED and usually involves some patient uncertainty around the expected quality of care. The patient is assessed by ED physicians, and triaged accordingly.

1. Patients face a number of challenges when arriving at the ED. There is uncertainty about whether an ED provider will be understanding or considerate to the SCD patient's concerns, and whether the closest ED is one they are familiar with and has well-trained personnel.
2. After the patient is admitted to the ED they are triaged according to their medical needs. Patients face hurdles like incorrect triage conducted by nurses who are unfamiliar with SCD protocol, and a failure to prioritize treatment for SCD patients.
3. Once the patient is triaged they wait to see a provider. The lack of protocol for SCD results in long wait hours.

<u>Data needs and opportunities</u>	<ul style="list-style-type: none"> <li>• SCD Patient Registry to help confirm that patients have SCD and prioritize their treatment</li> <li>• SCD Surveillance data</li> <li>• Payer data</li> </ul>
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**Phase 4: Admission to the ED** – The ED admission phase begins after a nurse triages a patient according to their health needs. The patient then undergoes imaging, labs, and further tests to be analyzed by the physician.

1. After the SCD patient is admitted to the ED they speak with an ED provider. Patients face challenges with ED physicians because providers often lack knowledge on SCD, harbor suspicions about patients, perceive patients as drug-seeking, and may miss or ignore messages from patients' hematologists or PCPs.
2. Once the patient is evaluated the ED provider orders the appropriate labs, imaging, and fluids as needed. Because of the extensive lack of SCD knowledge, ED providers sometimes fail to order the correct labs and imaging, further extending the patient's pain.

3. The patient then waits for results after undergoing labs and imaging. SCD patients face challenges after tests because ED providers often wait until they receive the results to prescribe appropriate pain management.
4. Upon receiving a patient's test results, the provider decides on a course of treatment, whether that is to keep the patient in the ED (phase 5.1) or admit them as a patient to the hospital (phase 5.2). ED providers are also unaware of how SCD types affect test results differently, which affects their clinical decisions.

<u>Data needs and opportunities</u>	<ul style="list-style-type: none"> <li>● Quantifiable pain scale</li> <li>● Electronic Health Records Social determinants of health (SDOH) related to the patient</li> <li>● Data on EDs (wait times, length of stay, readmissions)</li> </ul>
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**Phase 5.1: Patient is stabilized in the ED** – If the ED provider decides the SCD patient can be stabilized in the ED, they will be released by the provider following prescribed treatment. Patients will then go home or look for a different ED or care setting if they still feel unwell.

1. If a provider deems the ED to be the best environment for a patient's treatment, the patient remains there until stabilized. The ED provider may not prescribe pain management until this point, when it could have been administered sooner to the patient's benefit. In addition, pain management prescribed may not be aggressive enough, due to increased tolerance for opioids over time. Providers are often apprehensive about prescribing pain medications due to fear of investigation for wrongful prescription of opioids. The patient may prefer to be admitted to the hospital if care is not sufficient in ED.
2. When an SCD patient is discharged, they may not be fully recovered and lack the next steps from providers and resources to follow up with treatment following discharge.
3. If the patient does not receive adequate pain medication, they either go home or look for another ED. These patients risk readmission due to lack of proper SCD care, potentially causing further health risks and financial burden.

<u>Data needs and opportunities</u>	<ul style="list-style-type: none"> <li>● Data on EDs (wait times, length of stay, readmissions)</li> <li>● SCD clinics</li> </ul>
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**Phase 5.2: Patient is hospitalized** – If the patient cannot be stabilized in the ED, the provider will hospitalize the patient who will then undergo specialty care. Patients risk further complications once hospitalized.

1. If the SCD patient fails to receive adequate care in the ED, they will be transferred to the appropriate specialty department. Transfer to another department also means dealing with more providers who lack SCD knowledge, and a potential lack of sufficient hospital beds.
2. After being placed in the appropriate department, the patient is handled by specialty care. This poses the risk of infection for the SCD patient due to extensive movement within the hospital.
3. When a patient is discharged, they again risk readmission, further health concerns, and greater financial burden. If a patient is readmitted, hospitals do not get paid by insurance for the first admission.

<u>Data needs and opportunities</u>	<ul style="list-style-type: none"> <li>• Data on EDs (wait times, number of beds, length of stay, readmissions)</li> <li>• SCD clinics</li> <li>• Readmission <u>data</u></li> </ul>
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## SICKLE CELL DISEASE DATA COLLECTION

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The upcoming Roundtable will address existing sources and availability of SCD data, as well as data needs for better understanding and treatment of the disease. Research for this briefing paper has identified a number of data collection efforts in the U.S., and potential applications of new forms of data.

### *National Data Collection Efforts*

As of now, there are few statewide SCD data collection efforts and none on a national scale. One major multi-state program is now collecting data on SCD on both an individual and population level. The [Sickle Cell Data Collection \(SCDC\) program](#) has worked with the Centers for Disease Control and Prevention (CDC) and others to collect information to monitor the long-term trends in diagnosis, treatment, and access to health care for people with SCD since 2010. The [CDC Foundation](#) is partnering with [CDC's Division of Blood Disorders](#), [California Rare Disease Surveillance Program](#), [Georgia Health Policy Center](#), [Pfizer Inc.](#), [Global Blood Therapeutics \(GBT\)](#), [Sanofi](#), and [Doris Duke Charitable Foundation \(DDCF\)](#) to support the development and implementation of the SCDC program. Pfizer, GBT, and DDCF provide funding support, while the CDC Foundation administers the grants. The CDC Foundation, an independent nonprofit created

by Congress to support the CDC's work, forges effective partnerships between the CDC and others to fight threats to health and safety.

The SCDC program aims to help inform policy and healthcare standards that improve and extend the lives of people with SCD. California and Georgia are the only participating states as of now, but there are plans to expand the program to additional states. This program specifically collects data to better understand: where people with SCD live, the transition from pediatric to adult care, the Hispanic patient population (who make up approximately 10% of SCD patients in the US), older patients (SCD patients are living longer, so there is a growing opportunity to study them as they age), and the use of healthcare services.

The purpose of this program is to serve as a surveillance and data collection framework for states, rather than to develop an SCD registry or data portal. The CDC has placed special emphasis on the program serving as an educational initiative for the ED, pediatric care, and large health care systems to better care for patients living with SCD. The SCDC program also aims to influence other federal organizations, like the Health Resources and Services Administration (HRSA) and National Institutes of Health (NIH), to connect and identify programs that overlap geographically. This will help to geographically identify SCD specialty care facilities and areas where they are lacking. The goal is to improve the process of connecting ED patients to outpatient specialty care centers, whether that means referral to PCPs or specialty SCD clinics. Currently, this program does not aggregate data or conduct data analysis.

The SCDC program collects data through newborn screening records, administrative datasets (i.e. hospital discharge, ED, and state Medicaid data), medical charts, and death records. The program currently provides data on the number of patients by age/sex, hospitalizations, ED admissions, and number of admission days for SCD patients in California and Georgia. The data is downloadable in XLSX format, a format used with Microsoft Excel.

In late 2019, the CDC announced plans to award nearly \$1.2 million to seven additional states, allowing them to take part in the data gathering program on sickle cell disease and how it affects daily life.<sup>16</sup> The program is designed not only to address the needs of SCD patients, but also to help develop therapies more suited to patients' needs and provide greater patient support. The program had nine participating states as of October 2019, but the CDC has so far only published archival data for [California](#) and [Georgia](#), which became the first state to participate in 2005.

A number of needs have been identified for the SCDC program, including the need for greater state participation. California and Georgia are the only states actively collecting data for the program, with seven additional states slated to become active in the foreseeable future. There is also a consistent lag in the timeliness of data, as surveillance efforts cannot keep up with the rate of SCD cases. There is also no state-wide, or national SCD patient registry in existence or in

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<sup>16</sup> Chapman, Mary. "CDC Expands SCD Data Collection Program to 7 More US States in Effort to Improve Services." Sickle Cell Disease News, October 2019. Retrieved from <https://sicklecellanemianews.com/2019/10/03/cdc-expands-data-collection-program-to-7-more-states-improve-scd-services/>.

development. There is a need and opportunity for additional different types of data sources, including data on SDOH and extensive population-level data.

The SCDC draws on the [California Sickle Cell Disease Longitudinal Data Collection Project](#).<sup>17</sup> For more than five years, the California program collected data on SCD in the state as part of the CDC and National Heart, Lung and Blood Institute’s Registry and Surveillance System in Hemoglobinopathies (RuSH) and CDC’s Public Health, Research, Epidemiology and Surveillance in Hemoglobinopathies (PHRESH) cooperative agreements. RuSH and PHRESH are no longer active, but data collected from the programs are utilized in state programs, including the SCDC program. The California project found strong evidence differentiating pediatric and adult clinical populations, the need for multiple data sources for complete surveillance, intermittent high health care utilization, specific needs of adult patients over age 40, and issues with the unreliability of death certificates.

## *Application of SDOH Data to Improve Care*

SDOH factors, “the conditions where people live, work, and play,” are a key component in identifying health risks and improving health care outcomes for individuals.<sup>18</sup> SDOH data can play a vital role in the advancement of SCD by identifying at-risk populations for differing health outcomes, and developing consequent community level interventions to aid the SCD community. By identifying populations with greater risk for adverse health outcomes, we can provide healthcare providers, researchers, and patients with the knowledge to better treat, manage, and allocate necessary resources to people living with SCD around the country.

Key types of SDOH data include:

- **Climate and Environmental Health** – e.g. air quality, water quality
- **Economic Stability Data** – e.g. employment, public assistance, household income
- **Neighborhood Data** – e.g. median home values, population data, transportation data
- **Medical Surveillance Data** – e.g. high blood pressure, obesity, alcohol abuse
- **Education Data** – e.g. high school education and above
- **Food Data** – e.g. food deserts, access to healthy options

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<sup>17</sup> “Longitudinal Data Collection for Sickle Cell Disease in California: History, Goals and Challenges.” CDC Foundation. Retrieved from

<https://www.cdc.gov/ncbddd/hemoglobinopathies/documents/donor-report-010416.pdf>

<sup>18</sup> “Social Determinants of Health | CDC,” April 9, 2019. <https://www.cdc.gov/socialdeterminants/index.htm>.

## AN EXAMPLE OF DATA COLLECTION: *Studying Patterns of ED Visits*

The CDC conducted a study using data from the SCDC program to analyze ED patterns of care in California, and to compare and contrast new outcomes with findings of existing studies.<sup>19</sup> The study looked specifically into patterns of ‘treat-and-release’ for SCD patients in the ED, instances where ED visits end in discharge from the ED, instead of admission into a care facility. The study used 2005-2014 ED and hospital discharge data from California’s SCDC program for their study, a cohort of approximately 4,600 SCD patients.<sup>20</sup> The researchers found that 88% of patients in the cohort had one or more treat-and-release visits, and the mean annual number of visits per person was 2.1. They identified a ‘highest utilization’ group in the cohort for ED visits. This group accounted for 45% of all patients, and was comprised of younger and older adults more so than pediatric patients. This benchmark study provides a high-level use case demonstrating the utility of a multisource state longitudinal data collection program. California’s SCDC program can now serve as an example framework for other states to model their own SCD programs after.

## MAJOR THEMES AND DATA NEEDS

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Despite the work of state programs, SCD-related data is still limited. The NIH has identified a need for a new population-based surveillance system to address the lack of information surrounding SCD and SCD patients. Such a system would aim to follow patients longitudinally, and should contain demographic, laboratory, clinical, treatment, and outcome information. This research overview of SCD and related data issues suggests several topics for discussion at the upcoming Roundtable. The Roundtable will address three key themes: Improving Continuity of Care for SCD Patients, Improving Care during ED visits, and Addressing Treatment Options for SCD Patients. Each of these themes can be analyzed through three key dimensions:

1. SDOH factors
2. Increasing awareness
3. Improving treatment

The following section summarizes data issues for these themes and dimensions and questions for discussion.

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<sup>19</sup> Centers for Disease Control and Prevention. “CDC’s Sickle Cell Data Collection Program Data Useful in Describing Patterns of Emergency Department Visits by Californians with Sickle Cell Disease (SCD).” Centers for Disease Control and Prevention. Retrieved from <https://www.cdc.gov/ncbddd/sicklecell/features/kf-sc-data-collection-californians.html>.

<sup>20</sup> Paulukonis, Susan T., et al. “Emergency department utilization by Californians with sickle cell disease, 2005-2014.” *Pediatric Blood Cancer*, December, 2016. Retrieved from <https://doi.org/10.1002/pbc.26390>.

## **Theme 1: Improving continuity of care for SCD patients, with a focus on the transition from pediatric to adult care**

Pediatric SCD patients are often treated with more swiftness and concern, particularly in the ED, than adult patients. A major challenge will be developing strategies for improving the consistency and continuity of care. This should include increased genetic screening for SCD at birth as a basis for continuous care, with appropriate safeguards for data privacy and security. Aspects of this topic include:

- **Dimension 1: SDOH data application**
  - **Application of SDOH data.** A pediatric SCD patient may reside in a community with sufficient resources and relatively good SDOH, but this may change when they transition to adult care, particularly for individuals going off to college or moving communities. Physicians could use SDOH data to inform care plans and proper treatment, especially in cases where a newly transitioned patient may be experiencing declines in health. How can both individual and population-level data on the SDOH inform patient care?
- **Dimension 2: Awareness**
  - **Increasing awareness of SCD.** Our understanding of SCD suffers from poor data collection efforts, lack of federal funding for data-driven studies, and disparate state-level initiatives. Health care is underutilized by people with SCD, many of whom are unaware they have the disease. How can we improve the care of patients with SCD and mutual trust between them and the healthcare system?
  - **Decreasing stigma, and the need to improve validation of SCD patients for treatment of pain.** SCD is most common among people of African ancestry, but also affects a small percentage of Hispanics. A significant number of patients also lack health insurance and have trouble affording care. These parameters create a perception that SCD patients exhibit drug-seeking behavior, and may unfairly stigmatize patients. What kinds of policies would support better data sharing across the full spectrum of care for SCD patients?
- **Dimension 3: Treatment**
  - **Improving treatment for adult SCD patients.** Pediatric SCD patients typically receive more swift care and receive greater understanding from providers, while adult patients face a harder time in the ED. Are there opportunities to improve the SCD surveillance data program to increase data gathering on treatment disparities?
  - **Improving pain validation for adult SCD patients.** Adult SCD patients may need to demonstrate that they have SCD before they receive adequate pain medication. What kinds of programs could give them the data they need to demonstrate their status quickly to healthcare providers?

- **Care management and information sharing.** As pediatric SCD patients transition into adult care, they often switch to new PCPs or new provider networks. Information may be lost during this transition. How can providers better share data to improve continuity in care, especially during the transition from pediatric to adult care? Are there existing data sharing models that would be effective?

Data needs and Sample Datasets:

- **Healthcare outcomes, such as coexisting or co-occurring conditions, surgeries, stroke, death.** For example, the [CMS Mapping Medical Disparities Tool](#) describes chronic conditions and their associated costs for Medicaid and Medicare recipients across the United States.
- **Electronic Health Records (EHRs).** The [Million Veteran Program](#), a precision medicine initiative piloted by the VA, aims to gather de-identified, research data to work on a range of genetic and inherited diseases such as Sickle Cell Disease.
- **Prevalence of chronic and acute pain data.** The [National Health Interview Survey \(NHIS\)](#) conducted by the CDC provides specific information about the prevalence and degree of pain that a person experiences during a six month period.
- **Quantifiable SCD pain scale.** A number of pain scale measures have been suggested from the NIH, including patient pain measures, and other techniques.<sup>21</sup> The use of new sensors and wearables also hold promise to measure pain may hold promise for understanding a patient’s level of pain.

## **Theme 2: Improving SCD care in the ED**

SCD patients often face a number of obstacles when admitted to the ED. The ED is often a patient’s last resort when seeking pain management, but some patients must use the ED because it’s their only option. ED physicians generally lack knowledge or experience working with SCD patients, and hold biased stigmas that prevent them from delivering the appropriate care patients need. The application of SDOH, clinical, and claims data, along with the development of education initiatives for providers can improve care for SCD patients in the ED.

- **Dimension 1: SDOH data application**
  - a. **Application of SDOH data.** SDOH data can be used by ED providers to better inform post-treatment care after an SCD patient is discharged. For example, if a patient resides in a low-income community with high levels of homelessness, they may be more likely to experience greater crisis episodes due to transient housing or other external factors. How can both individual and population-level data on the SDOH inform patient care?

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<sup>21</sup> Johnson, Lanny L., Andrew Pittsley, Ruth Becker, and Allison De Young. “A Novel Quantitative Pain Assessment Instrument That Provides Means of Comparing Patient’s Pain Magnitude With a Measurement of Their Pain Tolerance.” *Journal of Clinical Medicine Research* 7, no. 10 (October 2015): 781-90. <https://doi.org/10.14740/jocmr2277e>.

- **Dimension 2: Awareness**

- a. **Hospital, ED, and specialty care prevalence data.** Health care prevalence data can be used to better inform patients of their care options. For example, an SCD patient may not be familiar with care facilities in their communities, and may not know which ones will be adept at treating SCD. How can data on care prevalence aid SCD patients when going to an ED?
- b. **Educational initiatives for ED providers/nurses.** Most health care providers are not knowledgeable on the various dimensions of SCD. This causes biased care by physicians, prolonged stigma around SCD patients, and withholding of proper care like opioid prescription. How can patient pain validation be improved using SCD awareness education? What are some existing SCD education initiatives, and where are they taking place? How can the observed best practices be applied to other care teams?
- c. **Application of claims data.** ED providers are often reluctant to prescribe sufficient first-rate care due to the fear of wrongly prescribing treatments like opioids. Because there is currently no method to quantifiably measure SCD patient pain, and patient's possess stigmas, providers prefer to be safe than sorry when caring for patients. How can claims data, like patient pharmaceutical claims, provide more information for pain validation and improve data sharing in general?

- **Dimension 3: Treatment**

- a. **Improved efficiency of SCD patient care.** Adult SCD patients typically experience a tougher time in the ED than on average, due to their chronic pain, and the difficulty of validating such pain. How can the medical community treat pain management of SCD patients swiftly and efficiently?
- b. **Clinical data application.** SCD patients often have specific treatment plans recorded in their EHRs that may indicate levels of opioid tolerance, specific treatments, and history. How can the medical community make EHRs and patients' care history available to ED providers so they can better prescribe treatment options?
- c. **ED Treatment Sustainability.** What are sustainable vs non-sustainable treatments (e.g. blood transfusions) and their associated cost effectiveness? How is this data collected and communicated to providers?

Data Needs and Sample Datasets:

- **Community-level SDOH data.** For example, the [County Health Rankings](#) supported by Robert Wood Johnson Foundation provide information about a wide range of community factors such as high school graduation rates, children in poverty, violent crime, and severe housing problems.

- **Pharmaceutical claims data.** Health technology companies like [ZeOmega](#) often have access to comprehensive claims data that provide information about medical services provided and prescriptions.
- **Hospital/ED/specialty care prevalence.** For example, the [U.S. Department of Homeland Security](#) collects geocoded information about hospitals and medical providers across all 50 states.
- **Clinical data (EHRs).** [Epic](#), one of the leading EHR vendors with nearly 230 million records, proposed the Cosmos program in 2019, to make de-identified patient data available for research purposes.<sup>22</sup>

### **Theme 3: Assessing treatment options and improving sustainable treatment**

There is no standard treatment of care for SCD patients, and care is inconsistent and unaffordable for many people with SCD. Many SCD patients choose to utilize the ED rather than a PCP for a number of reasons, but primarily due to the quick, though unsustainable, prescription of drugs, particularly opioids. Many SCD patients rely heavily on Medicaid, or have no health insurance at all. While treatment by a PCP is preferable to treatment in the ED, PCPs are often unable to provide consistent care to SCD patients, as they are sometimes unwilling or unable to afford such care. Aspects of this topic include:

- **Dimension 1: SDOH Data Application**
  - **Application of SDOH data.** The efficacy of different treatments may depend on individual factors, including an individual's social environment, housing and food security, and other factors. Treatment plans may also need to address these social determinants to help patients. How can both individual and population-level data on the SDOH inform the assessment of treatment options? What community risk factors may cause a patient to improve treatment adherence?
- **Dimension 2: Awareness**
  - **Application of clinical trial data.** It has been difficult to establish standards of care for SCD patients, for many reasons. Although there are many clinical trials in progress, data from those trials is not consistently published. The application of clinical trial data can better inform providers of the availability and feasibility of treatments for different SCD patients. What can we learn about what does and does not work, for whom, and how do we communicate that information to SCD patients and providers? What is the availability of data on emerging treatments like transfusions and hydroxyurea?
  - **Improving SCD surveillance data.** National and state-level systems for assessing the prevalence and severity of SCD in the population need to be improved. This will

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<sup>22</sup> "Epic Unveils Patient Data Research Initiative, New Software." Accessed April 7, 2020. <https://www.beckershospitalreview.com/ehrs/epic-unveils-patient-data-research-initiative-new-software.html>.

include increased coordination between the CDC and the states. It will also require assurances of data security and privacy to protect patients' data. What is the available population-level SCD data, and which states collect it? Who is collecting the data?

- **Dimension 3: Treatment**

- **Cost-effectiveness and time efficiency.** ED treatment and hospitalizations are extremely costly, and people may lose their income if they are home sick from work for too long. Which treatment options (e.g. opioids for pain management, blood transfusions, hydroxyurea) are the most cost-effective and time-efficient? Which are sustainable and which are unsustainable? What kinds of funding and institutional structures are currently enabling or impeding additional research on cutting-edge treatments?
- **Pain management.** It is extremely difficult for health care providers to measure an SCD patient's level of pain. This results in inadequate and delayed management of pain by physicians, and sometimes additional health complications. How can PCPs and ED physicians better assess pain to prescribe pain medications for patients with SCD?
- **Improving treatment of SCD-related complications** (e.g. infections, strokes). SCD often exacerbates medical problems or creates new ones, on top of existing SCD symptoms. An emphasis on addressing supplementary care is essential. How can SCD clinics and hospitals take a more holistic approach to SCD patient care, and what are the potential benefits?

Data Needs and Sample Datasets.

1. **Community-level SDOH data.** In addition to the County Health Rankings, other determinants of health like the [500 Cities Project](#) may have additional data measures on health such as smoking rates, binge drinking, and other health measures.
2. **Uptake of recommended treatments.** Some hospitals are beginning to gather data on the treatments administered for SCD such as transfusions, hydroxyurea, and new experimental treatments including transcranial doppler screening,<sup>23</sup> penicillin, and immunizations.
3. **Use of health care services: location of care and use of outpatient, ED, and hospital services.** For example, public health departments in high-risk counties or high-prevalence SCD patient areas have data available on uptake of local services or hospital use.

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<sup>23</sup> Naffaa, Lena N, et al. "Transcranial Doppler Screening in Sickle Cell Disease: The implications of using peak systolic criteria." *World Journal of Radiology*, February 28, 2015. Retrieved from <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4326734/>

## Policy Needs

Policy and operational changes are needed to improve SCD data availability and collection. Challenges include insufficient government funding, a dearth of data sharing and collection efforts, and a historical lack of focus on SCD by the healthcare and policy making communities. To address these hurdles, policymakers should address the following needs.

- **Increased federal, state, and local funding for SCD surveillance data.** States and local authorities need surveillance data to allocate funding for SCD interventions, research, and added medical support. The CDC is in the process of allocating funding to SCD programs, and recently awarded \$1.2 million to seven additional states to participate in the SCDC program, and are slated to become active in the near future.<sup>24</sup>
- **Increased funding for state SCD data collection programs.** In order to increase national data collection efforts on SCD, states will require considerable funding to establish such efforts. In December of 2019, California’s legislature committed to allocating \$15 million in funding to enhance SCD care within the state.<sup>25</sup> California and Georgia are at the forefront of SCD state data collection efforts.
- **Creation of a national registry for SCD patients.** There are currently no national or state SCD patient registries, nor any existing efforts. A national, or even state registry of SCD patients would aid a multitude of stakeholders including researchers and health care providers, and would improve government funding allocation.
- **Uptake of SCD training courses by care providers** (nurses, ED physicians, PCPs). Most health care providers lack extensive knowledge on proper standards of care for SCD. The US Department of Health and Human Services, Office of Minority Health recently created the Sickle Cell Disease Training and Mentoring Program (STAMP). STAMP is a pilot-program designed to train PCPs on the basics of SCD care.<sup>26</sup>
- **Classification of SCD as a disability.** As of now, SCD patients are eligible for social security disability benefits, but only if the disease prevents them from working. Patients must be considered permanently disabled, meaning unable to work for at least a year, for them to qualify for disability benefits.<sup>27</sup> Disability insurance, social security, and other

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<sup>24</sup> Chapman, Mary. “CDC Expands SCD Data Collection Program to 7 More US States in Effort to Improve Services.” Sickle Cell Disease News, October 2019. Retrieved from <https://sicklecellanemianews.com/2019/10/03/cdc-expands-data-collection-program-to-7-more-states-improve-scd-services/>.

<sup>25</sup> Chapman, Mary. “California Earmarks \$15M for Expanding Care of Adult SCD Patients.” Sickle Cell Disease News, December 17, 2019. Retrieved from <https://sicklecellanemianews.com/2019/12/17/california-funding-enhanced-care-adult-sickle-cell-patients/>.

<sup>26</sup> “The Sickle Cell Disease (SCD) Training and Mentoring Program (STAMP): A Pilot Program for Primary Care Providers.” HHS Office of Minority Health, 2019. Retrieved from [https://www.minorityhealth.hhs.gov/sicklecell/documents/STAMP\\_FAQs.pdf](https://www.minorityhealth.hhs.gov/sicklecell/documents/STAMP_FAQs.pdf).

<sup>27</sup> “Social Security Benefits for Sickle Cell Anemia.” Disability Benefits Help. Retrieved from <https://www.disability-benefits-help.org/disabling-conditions/sickle-cell-disease-and-social-security-disability>.

benefits have the potential to improve SCD patients' quality of living, but a reduction of the one-year wait period should be considered.

## *Data Gaps*

By and large, current SCD research relies on **clinical and administrative data**. Clinical data is often collected through clinical cohorts, clinical sites, and state-led newborn screening programs. Conversely, administrative data, used for record keeping purposes rather than research, is collected through electronic health records (EHR), hospital discharge, ED, and state Medicaid records. Although both of these means of data collection provide some insight on the SCD patient experience, they fail to provide a comprehensive analysis of the disease's prevalence and patient experience. The application of claims data would also be beneficial to SCD. For example, pharmacy claims data, including drug dosage, fill dates, and financial information can be valuable in addressing the opioid-seeking stigma SCD patients often face. Data limitations include the following:

- **Clinical data only covers patients who seek care at SCD clinics.** However, many patients do not have access, are unable to afford, or are not aware of the existence of such clinics, and may simply use hospital EDs instead. Consequently, clinical data excludes a large number of people living with SCD.
- **Administrative data sometimes includes inaccurate reporting of SCD and related issues due to the miscoding of patients.** Some patients who have other blood disorders may inaccurately be given an SCD medical code, while others who do have SCD may not be diagnosed with the disease. Overall, miscoding results in an overestimation of SCD prevalence.
- **There is a significant knowledge gap following newborn SCD screening,** to subsequent pediatric care after results are shared with parents and pediatricians. All states have universal screening for SCD as of 2006 as part of state administered newborn screening programs.

## **CONCLUSION & NEXT STEPS**

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This document is the background for a Roundtable that will bring diverse stakeholders together to address SCD and support patients by leveraging data. The structure of the Roundtable will focus on prioritizing key challenges faced by SCD patients in their continuity of care, identifying challenges faced by SCD patients and their providers in the ED, and prioritizing challenges in creating better SCD treatments. Drawing on this research on the disease, treatment approaches, data needs, and the patient/provider experience, participants in the Roundtable will explore challenges and solutions to managing SCD through interactive breakout sessions. In these sessions, they will identify high-priority data needs to address key challenges, and craft potential interventions, policy challenges, and possible solutions to help improve the ecosystem of SCD data. Following the Roundtable, CODE will prepare a public report of findings and recommendations based on the participants' work.

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## **About CODE**

CODE is an independent nonprofit organization based in Washington, D.C. whose mission is to maximize the value of open government data for the public good. CODE believes that open government data is a powerful tool for economic growth, social benefit, and scientific research. Since 2015 CODE has worked with the White House, numerous U.S. federal agencies, and several national governments and NGOs around the world to help them improve how they collect, publish, and apply data to better meet the needs of their data users. For more information, please visit [OpenDataEnterprise.org](https://OpenDataEnterprise.org).