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SICKLE CELL DISEASE IN UTAH

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Drug Regimen Review Center

Monet Luloh, PharmD, Clinical Pharmacist
Lauren Heath, PharmD, MS, BCACP, Clinical Pharmacist
Valerie Gonzales, PharmD, Clinical Pharmacist
Kristin Knippenberg, MFA, Administrator and Editor
Joanne LaFleur, PharmD, MSPH, Director and Associate Professor

University of Utah College of Pharmacy, Drug Regimen Review Center

CONTENTS

LIST OF TABLES

ABBREVIATIONS

AAP	American Academy of Pediatrics
ACP	American College of Physicians
AAFP	American Academy of Family Physicians
APHON	Association of Pediatric Hematology/Oncology Nurses
ASH	American Society of Hematology
ASPHO	American Society of Pediatric Hematology Oncology
CBO	Community-based organization
CDC	Centers for Disease Control and Prevention
CMS	Center for Medicare and Medicaid Services
CRCT	Chronic red cell transfusion
DUA	Data use agreement
DUR	Drug Utilization Review
ECHO	Extension for Community Healthcare Outcomes
ED	Emergency department
HbS	Hemoglobin S; Sickle hemoglobin
HIPAA	Health Insurance Portability and Accountability Act
HRSA	Health Resources and Services Administration
NASCC	National Alliance of Sickle Cell Centers
NBS	Newborn screening
NHLBI	National Heart, Lung, and Blood Institute
NIH	National Institute of Health
PCH	Primary Children's Hospital
SCAPN	Sickle Cell Adult Providers Network
SCD	Sickle cell disease
SCDAA	Sickle Cell Disease Association of America
SCDC	Sickle Cell Data Collection
SCT	Sickle cell trait
SHHC	Sugar House Health Center
US	United States
UU	University of Utah
VOC	Vaso-occlusive crisis

1.0 INTRODUCTION

Sickle cell disease (SCD) is a rare, lifelong, autosomal recessive, hematological disorder that is estimated to affect approximately 100,000 individuals in the United States (US),[1,2] predominantly of African American/Black (1 in 365 births) or Hispanic (1 in 16,300 births) heritage.[3] In the US, it is the most common inherited hemoglobinopathy.[4] SCD is caused by a single amnio acid point mutation in the beta globin gene (*HBB*); this mutation leads to the formation of hemoglobin S (HbS), an abnormal hemoglobin variant.[2,5-9] Polymerization of deoxygenated HbS induces the sickle-shaped morphology of erythrocytes,[2] leading to premature hemolysis, vaso-occlusion, and eventual multi-organ damage.[5] Subsequently, individuals with SCD often experience acute episodes of pain (commonly called vaso-occlusive crises [VOCs] or sickle cell pain crises), attributed to 95% of SCD-related hospitalizations.[7,10] SCD primarily manifests with multi-organ system complications, requiring specialized and well-coordinated medical care across the patient's lifespan.[1,2] The estimated annual SCD economic burden is $3 billion, with more than half of the expenses attributed to inpatient care.[11]

At the national level,[12,13] as well as specifically in Utah, SCD is gaining recognition as a pressing health concern that demands urgent attention and strategic policy interventions due to unmet patient needs, potentially ascribed to health care inequities.[2] For example, despite SCD having a higher prevalence in the US compared to other genetic conditions such as cystic fibrosis (approximately 35,000 individuals)[14] or hemophilia (approximately up to 33,000 males)[15], individuals with SCD experience less access to specialty care,[3] potentially due to the limited number of specialized centers, especially for adults.[16] Moreover, the dearth of SCD familiarity among non-specialized providers may cause substandard care due to unawareness of evidence-based guideline recommendations.[2]

In recognition of the health care disparities experienced by the SCD population, and in an effort to improve the overall health of residents with SCD in Utah, House Bill 487, supported by Rep. Sandra Hollins, Black Physicians of Utah, and the American Red Cross of Utah,[17] was signed into law in early 2023.[18] The goal of this law is to develop recommendations for improving all aspects of SCD management, including the screening, surveillance monitoring, diagnosis, and treatment within the state of Utah.[18]

The objective of this report is to identify gaps in SCD care for patients in Utah based on comparison with existing SCD programs in other states, and to develop recommendations on improving the access of care to help inform healthcare policymaking decisions. See **Appendix A** for a list of additional useful resources, including best practice guides for the treatment of SCD.

2.0 METHODS

To gain insight into the clinical practice of managing SCD within Utah, we conducted interviews with local hematologists, pharmacists, and other relevant personnel (eg, program manager, health equity

specialist) affiliated with one of the following major healthcare organizations known to treat SCD*:

- Hematology Clinic at University of Utah (UU) Sugar House Health Center (SHHC; Salt Lake City, UT)[19]
    - We conducted two interviews with healthcare professionals affiliated with this clinic: the first interview was conducted one-on-one with a pharmacist, and the second was with a hematologist in a group setting over Zoom
- The Utah Center for Bleeding and Clotting Disorders at Intermountain Primary Children's Hospital (PCH; Salt Lake City, UT)[20]
    - We held one interview with a hematologist, a program manager/physical therapist, and a social worker/health equity specialist in a group setting over Zoom

Permission was obtained from the interviewees to disclose the information discussed in the interviews. For specific details regarding the interviews, please refer to **Appendix B**.

Because it is often difficult to discern hematologists that specialize in treating SCD, we focused on interviewing physicians at institutions that publicly declared (based on online platforms) expertise in managing SCD. However, it is possible that some patients with SCD may be treated in non-specialized centers or in private practices by providers who may be specialized in hematology. Furthermore, not all pediatric patients are referred to the UU SHHC for adult care due to payer restrictions. Therefore, conclusions formulated by the interview process may not be reflective of all patients with SCD or practice settings across Utah. Nonetheless, information obtained from these organizations may garner attention on areas of improvement for SCD care.

To supplement the information obtained in the interview process about potential barriers to care, we performed a literature search in Ovid Medline using the keywords "sickle-cell" and "barrier*" in the title field. Notably, this report focuses on barriers that are not explicitly related to access of pharmacologic treatment; potential barriers to receiving newer pharmaceutical agents for SCD were addressed in a Drug Utilization Review (DUR) report completed in July 2023, available here: https://medicaid.utah.gov/pharmacy/drug-utilization-review-board/

3.0 SICKLE CELL DISEASE (SCD) OVERVIEW

SCD is characterized by multi-system acute and chronic complications (eg, stroke, acute chest syndrome, infection, chronic anemia, pain episodes, organ damage).[2,21] Although severe acute pain episodes (or vaso-occlusive crises [VOCs]) are a highly prevalent symptom,[10] clinical manifestations tend to vary between affected individuals.[22] Nonetheless, individuals with SCD often have a poorer quality of life and reduced life expectancy (estimated average is 54 years) compared to individuals without the disease.[2,23]

* The Hematology Clinic at University of Utah (UU) Sugar House Health Center (SHHC), and the Utah Center for Bleeding and Clotting Disorders at Intermountain Primary Children's Hospital (PCH) are National Alliance of Sickle Cell Centers (NASCC)-recognized centers, which are required to meet certain criteria regarding personnel, protocols, and treatments; for more details, please visit this website at: https://sicklecellcenters.org/center_criteria

Carriers of SCD (referred to as sickle cell trait [SCT]) are often asymptomatic but may pass the abnormal gene to their offspring.[2,24,25] In rare instances, SCD carriers may experience SCD-related symptoms (eg, VOCs), especially under stressing conditions (eg, scuba diving, mountain climbing, dehydration).[25]

4.0 INCIDENCE OF SCD

The US population with SCD is estimated to be about 100,000 individuals, and approximately 1−3 million Americans are carriers (ie, SCT).[2] SCD and SCT prevalence tends to be the highest among African American/Black people,[3] with 1 in 13 having SCT, and 1 in 365 having SCD.[3] However, the exact SCD prevalence in the US remains to be determined because no data registry encompassing all 50 states currently exists.[3,26,27]

Newborn screening (NBS) is universally conducted across the US in all 50 states, including Utah,[28] to identify potential carriers or those with SCD.[2] Although there are limitations of NBS, such as the inability to capture individuals born outside of the US or born prior to adoption of universal screening, it does enable early disease detection and prompt referral to care, including appropriate immunizations and guideline-recommended treatment.[2] Furthermore, NBS can aid in determining the state SCD prevalence.[29]

The last precise estimated measurement of SCD prevalence in Utah is based on data from the mid-to-late 2000s. According to data from the National Newborn Screening Information System from 2005−2007 and the 2008 US Census, individuals living with SCD tend to be concentrated in the southern and eastern US,[2] with the highest density in Florida and New York (>8,000)*.[30] Based on this data, Utah had an estimated SCD prevalence of 82†, likely due to the lower number of minority ethnic populations (ie, African Americans, Hispanic) living in the state.[30] Although more recent SCD prevalence data is available, it does not provide a precise estimated number, but rather a broad range. Based on 2017 data from the Center for Medicare and Medicaid Services (CMS), Florida and New York had the greatest number of SCD cases among Medicaid recipients (≥3,577), whereas Utah had <597 cases (the lowest threshold limit).[31] Regarding the number of *carriers* within Utah, NBS data from 2010 showed that of 51,486 screened newborns, 126 tested positive for SCT; the estimated SCT incidence was 2.4 cases per 1,000 newborns screened.[32]

Similar to historical trends, Utah has a predominantly White population (90.0%), and lower Black/African American (1.6%) and Hispanic (15.1%) populations, according to 2022 US Census Bureau data.[33] Although a Utah SCD data registry currently does not exist, a rough estimate of state SCD prevalence can be made using the US Centers for Disease Control and Prevention (CDC) reported prevalence rates in African Americans and Hispanics, and the 2022 US Census Bureau data for Utah.[33] Considering that SCD affects 1 in 365 African Americans and 1 in 16,300 Hispanics,[3] we can expect that about 148 African Americans and 31 Hispanics have SCD in Utah. Nonetheless, Utah may have a lower SCD population than this rough estimate given that patients may move out of Utah (or avoid Utah) to mitigate the increased risk of VOCs or splenic infarcts at higher elevations (ranging from >3,600−6,500 feet above sea level; ie, altitude-induced hypoxemia).[34-37]

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† Estimate was based on the total birth cohort-prevalence, adjusted for premature mortality due to sickle cell anemia

The program manager for Utah NBS confirmed that the positive screenings for SCD over the past several years are extremely low (<5 patients per year), especially compared to other states (personal communication [phone call] with Kim Hart, MS LCGC, Newborn Screening Program Manager, August 10, 2023). Notably, the numbers are likely to fluctuate with changes in the population demographics, and are currently increasing at a very modest rate.

4.1 Sickle Cell Data Collection (SCDC) program

The CDC manages the Sickle Cell Data Collection (SCDC) program, which gathers state-level, multi-source health information (ie, NBS data, health care utilization data, demographics) on individuals with SCD, to analyze the demography and geography of the patient population to ascertain the long-term treatment, diagnostic, and accessibility patterns of affected individuals within the US.[26,38] The goal of the program is to improve the health care, quality of life, overall health, and life expectancy of patients suffering from SCD,[26] as well as support state-level policy change.[2,39]

The SCDC funds programs for individual states to collect and analyze data on their residents with SCD, as well as statewide, multidisciplinary partnerships that help guide the focus, content, and information dissemination of the project.[40,41] SCDC states have the resources and infrastructure required for program participation.[42] Some of the most onerous elements are the data sharing agreements, which can take several years to establish. Notably, if initiatives in a participating state are disrupted due to insufficient funding or other reasons, the state would be obligated to start its data sharing agreement process anew.[26,42]

Currently, there are 11 participating states in the SCDC program (California, Colorado, Minnesota, Wisconsin, Michigan, Indiana, Tennessee, Virginia, North Carolina, Alabama, and Georgia),[43] representing an estimated 36% of the American population with SCD.[40] Although it is ideal to expand the program into other states (eg, Utah), the primary focus of the SCDC program is to maintain its efforts in currently participating states.[26,42] For non-participating states, the CDC, in collaboration with partnered states,[44,45] has provided a guiding framework for implementing a comprehensive statewide SCD surveillance system.[46]

4.1.1 Developing a statewide SCD surveillance system

Utah does not currently have a SCD surveillance system. The CDC, in collaboration with other participating SCDC states, developed a Capacity Building toolkit to serve as a framework for implementing a successful statewide SCD surveillance system[‡].[45] Although state-specific programs will differ with respect to data sources, resources, and methods, among other factors, the underlying foundational aspects are similar, prioritized into three-phases of development.[45] **Table 1** provides an overview of the essential components of an SCD surveillance system, according to the CDC.

[‡] The CDC also provides additional resources for data linkage, data policy, and data use agreements (DUAs), available at: https://www.cdc.gov/ncbddd/hemoglobinopathies/building-surveillance-capacity.html

Table 1. Framework for Establishing a SCD Surveillance System, According to the US Centers for Disease Control and Prevention[46]

Phase Milestones
Phase 1: Develop multi-stakeholder collaborations

• **Form a multidisciplinary team:**
  ○ Organize a multidisciplinary team (eg, healthcare providers, community organizations, public health officials, patients with SCD)
  ○ Multidisciplinary team goals:
  ▪ Develop insights into the requisites and objectives for the data system, emphasizing the potential use of the data to improve the quality of life for persons with SCD
  ▪ Identify the data repositories holding essential information for SCD surveillance
  ▪ Inform and involve collaborators who can facilitate data accessibility
• **Engage stakeholders:**
  ○ A variety of stakeholder involvement to improve policy and health care practices aids in the early identification of requisites and focal points for the SCD surveillance system, thereby ensuring the value and applicability of the information.
• **Data sharing needs:**
  ○ Most of the data included in the surveillance system is protected by HIPAA to protect patient privacy and confidentiality of shared data; this also safeguards against the potential misuse of data to discriminate against marginalized patients.[2]
• **Initiating data use agreements (DUAs)[a]:**
  ○ It is best to set up DUAs at an early stage, as these agreements may necessitate a significant amount of time for execution. Specifically, DUAs that entail the exchange of personally identifiable information could require extra time to guarantee adherence to HIPAA regulations.
  ○ It may be helpful to include multidisciplinary team members who possess expertise in legal dimensions of DUAs, IRB procedures, state and federal legislations, and other integral components of the initiative.

Phase 2: Build data infrastructure

• **Data transfer:**
  ○ The process of transferring (electronically sending information from one site to another) and acquiring data from sources necessitates meticulous preparation to mitigate any potential delays.
  ○ Elaborating data transfer methodology within the DUA is imperative to secure IRB endorsement
  ○ Each data source will entail specific data transfer procedures
• **Establish a data linkage[b] algorithm:**
  ○ The multidisciplinary team will need to develop a data linkage plan, based on identified variables (eg, geographic location, service dates, social security number, date of birth).
  ▪ The plan may include describing data sources that are used (eg, Medicaid data, NBS data, hospital/emergency department discharge data), the identified variables that are required to link datasets, linkage process order, selecting data linkage software (eg, SAS) or tool(s) (eg, Link+), and "the creation and contents of the SCD surveillance system file."[46]
  ▪ It is important to consider related expenses (eg, personnel hours, data source accessibility, software) when creating a data linkage plan.
• **Prepare data for linkage:**
  ○ The preparation of data for linkage is a labor-intensive endeavor encompassing the standardization of data formats, data cleansing (process of evaluating data quality, and if necessary, rectifying or eliminating inaccurate or incomplete entries), and data de-duplication.
• **Develop a data analysis plan:**
  ○ An effective data analysis plan derives insights from the program's goals; goals should be reflective of the program's objective and address the purpose of the data and analytic results.
  ○ Some states with established SCD surveillance programs have used the information to evaluate the following among their residents living with SCD:
  ▪ Changes in accessibility to care over time
  ▪ The relationship of differing treatment modalities, insurance status, or geographic location on overall health
  ▪ The association between demographic variables (eg, socioeconomic status, ethnicity) and access to care, treatment, or supportive services

Phase 3: Share data findings
• This is a pivotal phase within the SCD surveillance process because it involves disseminating findings from compiled data to engaged stakeholders and the community. • Careful consideration should be given as to how data findings can be presented in a meaningful manner to key stakeholders, the general populace, and fellow state health agencies. • Establishing a plan for sharing findings should be developed early during the planning phase, and the plan should be shared with stakeholders for their insight and support, thereby bolstering the ability to sustain the surveillance system.

[a] DUAs are as a legally binding contract between a primary entity (the organization instituting the surveillance system) and an external party (eg, health payers, healthcare institutions, state public health agency). This agreement is invoked upon a solicitation that "includes the use of personal identifiable data that is covered by legal authority."[46]

[b] Data linkage is used to gather information from multiple sources on a singular individual or entity to create a comprehensive dataset.

Abbreviations: DUA, data use agreement; HIPAA, Health Insurance Portability and Accountability Act; IRB, Institutional Review Board; NBS, newborn screening; SCD, sickle cell disease; US, United States

5.0 INTERVIEW FINDINGS

This section summarizes information ascertained from interviews with healthcare professionals at the following two multidisciplinary treatment centers in Salt Lake City, Utah:

- Hematology Clinic at University of Utah (UU) Sugar House Health Center (SHHC)[19]: this clinic manages **adult** patients with SCD
- The Utah Center for Bleeding and Clotting Disorders at Intermountain Primary Children's Hospital (PCH)[20]: this clinic manages **pediatric** patients with SCD

Currently, the two aforementioned institutions are the only Utah centers that are recognized by the National Alliance of Sickle Cell Centers (NASCC).[47] NASCC identifies centers that provide high-quality comprehensive care for SCD, with the goal of unifying centers across the US to increase resources and infrastructure to support the SCD patient population.[48]

As part of routine NBS, all newborns at Intermountain PCH are screened for SCD 24 to 48 hours after birth using a blood sample obtained via a heel stick.[49] If a patient is identified as having positive sickle cell status, the caregiver is notified, and hematologists follow up with the patient's primary care provider or the caregiver directly to schedule appropriate consultation with pediatric hematology. There have been around 3 positive screens for SCD in the past year, and overall, approximately 45 pediatric patients with SCD are currently being treated at Intermountain PCH.

In the adult setting, there are about 20 patients with SCD currently being treated at the UU SHHC Hematology Clinic. Although there may be a couple of explanations for the disparate patient number between these two clinics, it does not undermine the importance of maintaining continuity of care during the transition period from pediatric to adult medical care. Dr. Ming Lim, a hematologist with the UU, noted that not all patients that transition from Intermountain PCH are able to be seen at the SHHC for adult care due to payer-driven restrictions; for example, the UU is not preferred in-network for several insurances (eg, Select Health, Cigna, Aetna), whereas this is less of a concern at Intermountain PCH. Consequently, patients may be lost to follow-up (by UU SHHC) as they transition from pediatric to adult care. This is a critical area of concern because, as expressed by Dr. Sasidhar "Sashi" Goteti, mortality rates among patients with SCD have historically been the highest in the transition age group due to fragmented care. Hematology clinic personnel at Intermountain PCH estimate that approximately 5 to 10 patients in the past year have been referred to other organizations within the state (eg, Utah Cancer Specialists), and very few, if any, have transitioned to a sickle cell treatment center out of state.

Interviewees also expressed concerns about coordination of care, particularly between the inpatient and outpatient settings. Unlike other blood disorders (eg, hemophilia), patients with SCD are often hospitalized due to VOCs (severe acute pain episodes). Currently, Intermountain PCH and UU SHHC do not have a dedicated infusion center for *severe* sickle cell pain, which is often uncontrolled with oral analgesics; therefore, patients are sent to the emergency department (ED) for pain management, which is a time-intensive process. Because the health care system is not integrated allowing for an exchange of information across services, it is difficult for hematologists to provide comprehensive care for patients in these scenarios. Additionally, because SCD affects multiple organ systems, patients require access to not

only hematologists, but also other providers (eg, pulmonologists, neurologists, nephrologists), making coordination of care even more challenging.

According to the interviewed hematologists, their organizations, and most hospitals across the country, use standardized treatment protocols for SCD based on evidence-based practice guidelines published by the National Heart, Lung, and Blood Institute (NHLBI) and/or American Society of Hematology (ASH). However, the use of certain disease-modifying pharmacologic agents is often dependent on the patient's insurance, and prior medical history[§].

Currently in Utah, resources and support for patients with SCD and their families are very limited, and therefore, any disruption may profoundly impact the patient's ability to access care (eg, insurance changes). To our knowledge, there are very few centers in Utah that provide care for patients with SCD, potentially causing patients to seek care at non-specialized centers; these centers may not have the resources (eg, staffing) and/or clinical expertise required to manage patients with SCD, potentially leading to symptom exacerbation and overall decline in the patient's health.

6.0 POTENTIAL BARRIERS TO CARE

Patients with SCD tend to struggle with multilevel barriers when seeking access to high-quality SCD-specific health care.[27] Barriers to accessing care are likely to negatively impact patient perceptions and result in reduced health-related quality of life.[50] Barriers to care may include insurance coverage gaps, inadequate access to specialists, stigmatization, transportation issues, lack of provider expertise, or negative provider or patient attitudes.[27,51] The following subsections discuss the potential barriers to care according to interviews and published literature.

6.1 Identified barriers to care based on conducted interviews

Based on conducted interviews, several potential barriers to care for patients with SCD in Utah were noted as follows, with additional details discussed below:

- No formal SCD pediatric-to-adult transition of care program at institutional- or state-levels
- No SCD-specific acute pain management facility, contributing to coordination of care issues
- Limited resources, and few specialized SCD centers for adult care

6.1.1 Lack of pediatric-to-adult transition of care program

Historically, SCD was considered a childhood disease because patients generally did not survive into adulthood due to fatal infections, prompting an effort to improve the infrastructure for pediatric care.[2] Starting in the early 2000s, mortality among young children (<4 years of age) with SCD significantly decreased due to vaccination against invasive pneumococcal disease.[3] Coupled with advances in treatment, robust pediatric care, and NBS, more patients with SCD are living into adulthood,[4,52] but there is a lack of adequate resources for adult patients resulting in health care disparities.[2]

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§ For information on the potential barriers to receiving newer disease-modifying agents, please refer to the Drug Utilization Review (DUR) report completed in July 2023, available at: https://medicaid.utah.gov/pharmacy/drug-utilization-review-board/

According to published literature, the morbidity and mortality rates escalate for patients in the transition age group (18 to 30 year olds),[52,53] resulting in higher utilization of acute care services, and poorer health outcomes.[2,51,54] Some of these factors may be attributed to poor self-management skills, lack of provider trust, minimal adult specialized providers, poor care coordination, or loss of insurance.[55] According to a recently published retrospective cohort study, over half of pediatric patients with SCD failed to transition to an adult SCD clinic between 1995 and 2001, with some patients becoming lost to follow-up (eg, not receiving pediatric care for ≥2 years) before 15 years of age.[52] Therefore, some SCD centers in other states (eg, Alabama, Tennessee, Philadelphia) provide programs dedicated to helping pediatric patients transition to adult services to maximize continuality of care, and to help overcome potential transition-related barriers.[2,55-58] Maryland, as part of the Sickle Cell Disease Follow-up Program, also provides information on care transitioning to patients starting in early adolescence.[59] Transitioning programs help prepare and educate the patient, as well as the family, with the hope of integrating the pediatric and adult health care systems to ensure care remains uninterrupted.[56] **Section 7.1** provides details on essential components of a pediatric-to-adult transition program.

6.1.2 No SCD-specific acute pain management facility

Adults with SCD often experience pain, either acute (duration of <1 month),[60] chronic (duration of ≥6 months),[61] or a mixture of both.[2,62,63] For patients with SCD, pain is a predictor of quality of life and mortality.[2] The pathophysiologic mechanisms of SCD pain are multi-factorial (eg, inflammation, nervous system sensitization, hypoxia-reperfusion injury), and likely differ between acute and chronic pain.[64]

Acute pain management in an ED setting presents several potential barriers to care including restricted access to opioids as a result of the current opioid epidemic, treatment delays, lack of care coordination with the patient's SCD provider, and racial discrimination as a result of provider biases.[64] For example, studies have shown that African Americans are more likely to receive substandard pain management (eg, less prescribed medication) in comparison to White patients due to prejudiced beliefs and negative provider attitudes, including the perception that African American patients with SCD have drug-seeking behaviors.[2,64] Individuals with SCD may experience pain exacerbation due to emotional distress arising from doubts about their claims, prolonged delays in accessing pain relief, insufficient medication, and stigmatization.[2] Therefore, some centers in the US with comprehensive SCD programs have dedicated infusion centers to allow for prompt pain management treatment without requiring inpatient hospital admission,[58] or have SCD-specific pain treatment programs,[65] thereby potentially decreasing costs for the patient and the overall health care system.[64] The Georgia Comprehensive Sickle Cell Center at Grady Memorial Hospital was the first in the world to establish a 24-hour acute pain center for severe pain crisis management in adults with SCD.[2,66] Currently, hematology clinics at Intermountain PCH and UU SHHC refer patients directly to the ED if they are experiencing severe VOCs due to the lack of a SCD-specific hospital-based acute care facility.

For acute pain (VOC) management, guidelines published by NHLBI and ASH prefer that pediatric and adult patients seek treatment at dedicated SCD-specific, hospital-based acute care facilities (eg, day hospitals, infusion centers, observation unit) before receiving treatment at the ED[**].[64,67] This delivery care model allows patients to receive care in a facility that has the appropriate resources and expertise to manage severe SCD-related acute pain episodes,[60] and can potentially enable continuity of care with the patient's SCD provider.[64] By implementing such approach, patient-centered outcomes are improved including pain control, time to starting analgesic delivery, length of stay, hospitalization rates, cost, requirement for ED care after discharge, and most likely health equity.[2,64] ASH (2020) specifically recommends that the facility be hospital-based rather than free-standing or off-site to have the capability of providing immediate access to higher level of care if needed, and communication between both practice settings should be bidirectional for optimal implementation.[64] Resource requirements for implementing a SCD-specific, hospital-based acute care facility may largely depend on the number of patients who are likely to utilize it for pain management.[64] Some infusion centers and day hospitals across the country are shared facilities, treating patients with several hematological conditions, including SCD.[2] Having an SCD-specific acute care facility can potential save costs by reducing episodic, ED (re)admissions.[64]

6.1.3 Limited resources and specialized SCD care centers for adults

Currently, there are limited resources available for patients with SCD in Utah, and few centers that have adequate expertise in treating SCD, especially in adults. This issue may be attributed in part to the scarcity of funding dedicated to garnering resources and public awareness about SCD and SCT, and because the number of patients with the condition in a given region is relatively small.

Historically, SCD has been underfunded compared to other rare hereditary conditions (eg, cystic fibrosis).[2] For example, from 2008 to 2016, National Institute of Health (NIH) funding for cystic fibrosis research was roughly four times greater than research funding for SCD (as measured by average yearly funding per affected individual). Because the US prevalence of cystic fibrosis is lower than SCD and the condition predominantly affects White people, some individuals attributed the unequal funding appropriation for SCD to systematic racism. Additionally, African Americans, the population with the highest prevalence of SCD, tend to be socioeconomically disadvantaged, intensifying the economic disease burden. Adults with SCD may require additional support because they have accumulated years of injury, more end-organ damage,[2] and may be hesitant to use health care services as a result of racial discrimination.[51] Because SCD was previously regarded as a condition primarily affecting children, there is a lack of training among healthcare providers in adult care, compounded by a dearth of nonmalignant hematology providers.[4] Consequently, many primary care providers are treating patients with SCD, and they may be unfamiliar with SCD practice guidelines, potentially resulting in suboptimal care.[4]

[**] The 2020 American Society of Hematology (ASH) guideline on management of acute and chronic pain in patients with SCD, graded the recommendation for seeking SCD-specific, hospital-based acute care over emergency department (ED) care for the treatment of acute pain episodes as *conditional*, based on low certainty of evidence; therefore, it is a suggested recommendation that should take into account patient values/preferences and risks

Currently, there are 4 NASCC-recognized adult SCD treatment centers in the western US: 1 in Utah, and 3 in California; 6 NASCC-recognized pediatric SCD treatment centers in the western US, and of these, 1 is in Utah; and 20 "whole life" treatment centers nationwide.[47] In comparison, there are more than 130 comprehensive treatment centers across the US for hemophilia and cystic fibrosis, both are rare hereditary conditions with a lower prevalence[††] than SCD.[4,14,15] Nonetheless, this indirect comparison may be limited in perspective regarding concentration trends of affected patients and clinic size/capacity unaccounted for when making such comparison.

Conditions such as hemophilia may be used as guides to improve access to high-quality care for patients with SCD,[2] but the clinical nuances of SCD should be taken into account. Overall, public awareness of SCD may be increasing since September was designated by Congress as National Sickle Cell Awareness Month in 2020,[68] but additional efforts should be made to promote education among existing healthcare providers in the community.

6.2 Identified potential barriers to care based on published literature

There is extensive published literature on the topic of barriers to care for patients with SCD; studies vary based on whose perceptions are evaluated (eg, patient, provider, caregiver), and the setting (eg, ED). For barriers to care explicitly related to access of pharmacologic treatment, please refer to the Drug Utilization Review (DUR) report completed in July 2023, available at: https://medicaid.utah.gov/pharmacy/drug-utilization-review-board/

Barriers to care identified from interviews were generally consistent with the barriers identified in the published literature, albeit published literature provided additional insight on patient perceptions regarding SCD care. To help overcome the potential barriers discussed below and to improve the quality of care for patients with SCD, care should be comprehensive, coordinated, and accessible. In addition providers and the community should have an increased awareness of SCD,[69] and address overarching biases.[2]

6.2.1 Patient-reported potential barriers to care

Based on a 2022 survey study evaluating the perspectives of patients with SCD across three comprehensive centers, several multilevel patient-reported barriers to care were identified.[27] Approximately one-third of survey respondents (n=67) reported experiencing at least one insurance challenge (eg, high co-pays, coverage restrictions for providers, services, or medications, extended approval times), with the most frequently reported being insufficient coverage for medications coupled with high copayments. Other identified socio-environmental and organizational level barriers were limited transportation, poor care coordination, administrative challenges (eg, trouble obtaining timely appointments, long clinic wait times, inconvenient operational hours, difficulty contacting providers), and limited sickle cell specialty services (eg, pain management, laboratory).[27] Many of these administrative barriers have also been reported by parents of children with SCD.[50]

[††] In the US, sickle cell disease (SCD) affects approximately 100,000 persons compared to approximately 35,000 with cystic fibrosis and up to 33,000 with hemophilia.[2,14,15]

The following bullets highlight some considerations of identified socio-environmental and organizational level barriers[27]:

- Interviewees noted difficulties in locating healthcare providers or clinics that would accept their insurance. On occasion, gaps in coverage of medications and difficulties with obtaining appropriate pain medication resulted in ED visits and/or necessitated hospitalization.
- Transportation barriers (eg, lack of a personal vehicle, living an extended distance from a care center, relying on others for transportation) potentially contributed to patients missing appointments.
- Respondents conveyed difficulties with securing appointments at day hospitals or infusion centers to manage pain, frequently attributed to limited space and shared utilization of services with oncology patients.
- Respondents noted challenges in locating an adult healthcare provider with expertise in SCD, especially upon transitioning from pediatric care or in cases where the existing provider relocated. Additionally, they highlighted a scarcity of available providers, including specialists, in rural areas.
- Some respondents experienced a dismission from a hematology/oncology practice because the provider either chose to no longer provide care to patients with SCD or felt unequipped to provide suitable care for this patient group.

Regarding provider level barriers to care, 56% of survey respondents reported experiencing at least one barrier pertaining to provider knowledge and attitudes.[27] Difficulties in finding a provider knowledgeable in treating SCD was reported in almost half (45.4%) of survey respondents. Patient-provider relationship barriers included poor communication, a lack of provider empathy, no provider appreciation of the patient's understanding of their own condition, and mistrust. Almost half (48.1%) of surveyed respondents reported being accused of drug-seeking behavior, and half of surveyed respondents reported that providers did not believe their claims of severe pain, highlighting feelings of stigmatization.[51] Respondents also reported encountering disparate care relative to patients without SCD, further contributing to patient perceptions that non-SCD patient populations receive better disease management.[27]

About one-third of survey respondents reported challenges with having minimal to no social, family, or caregiver support; of these, 44.6% reported needing assistance with daily activities/chores, especially after an ED visit or hospitalization.[27] Respondents also described having difficulties with locating community support groups.[27] Competing life demands (eg, conflicts with work/school) was also an identified barrier when remembering or finding availability for appointments,[27] which could be particularly impactful for children who require screening for potential SCD-related complications.[70]

Disease-specific barriers, which pertain to the influence SCD itself has on proactive healthcare-seeking behaviors, were the most prevalent type of barrier reported by survey participants (82.3%); this barrier involved feeling distressing symptoms and emotions, including pain, worry, anger, fatigue, and depression.[27] Experiencing any of these symptoms and emotions may contribute to an overall sense of unwellness, resulting in absence at scheduled appointments. Other individual-level barriers to care included lack of self-management knowledge or understanding of required SCD care.[27]

6.2.2 Provider-reported potential barriers to care

According to a 2022 review, providers' attitudes towards patients with SCD have recently started to shift to be more positive, encouraging equitable health care.[51] Nonetheless, negative provider attitudes may still exist as a potential barrier to care,[71] especially in the ED for managing acute pain,[51] potentially, in part, due to unfamiliarity with SCD management.[2] Cited studies found that a considerable number of surveyed ED providers were unaware of the National Heart, Lung, and Blood Institute (NHLBI) guideline recommendations for VOC management.[72] Approximately half of surveyed ED providers used specific dosing protocols for SCD-related pain.[51,69] One cited study revealed that despite unawareness of treatment guidelines, nearly all (98.1%) ED providers expressed confidence in their ability to care for patients with SCD.[72] Other identified provider-perceived barriers to care in the ED setting were the opioid epidemic, patient behavior, overcrowding, addiction concern,[51,69] and implicit bias.[72] Providers, including those managing pediatric patients, are often fearful of prescribing opioids as pain management for SCD due to the development of tolerance or dependence, leading to patient-provider distrust and suboptimal care.[51,71] Additionally, providers were often unaware of patients' perceptions regarding stigmatization for requiring opioids.[51]

Studies in this review also noted that providers' comfort in managing patients with SCD tended to coincide with their experience in treating patients with this condition; however, providers who are at ease with treating SCD patients may not necessarily possess the most suitable skill set, including adequate knowledge and training.[51] A survey of US healthcare providers in non-emergency care found that hematologists and SCD specialists were significantly more comfortable prescribing opioids than non-specialized providers, and overall most (77%) felt comfortable managing acute pain.[73] Another study found that primary care providers are often uncomfortable and lacked the necessary knowledge, including awareness of recent evidence-based guidelines, to manage patients with SCD, but would be willing to use guidelines if they were accessible.[51] In contrast, another study found that physicians often did not follow guideline recommendations because of preconceived perceptions that the patient would not adhere to the recommendations; discussion as to the reasons behind the providers' skepticism about the patient's ability to adhere to recommendations was not addressed.[51]

6.2.3 Red cell transfusion barriers

A guideline-recommended treatment for patients with SCD, particularly high-risk children with sickle cell anemia, is chronic red cell transfusion (CRCT).[74] CRCT is used for primary and secondary stroke prevention, and may be used to prevent other SCD-related complications (eg, acute chest syndrome).[74] The frequency of chronic (or regular) blood transfusions may be monthly,[75] but is often based on the patient's hemoglobin level and symptoms,[76] with the goal of preventing SCD-related complications by maintaining a HbS percentage below a particular threshold.[77] Because the antigen profile of erythrocytes can vary based on race (eg, Black people tend to lack C, K, and E antigens), it is ideal for donors to be the same race or ethnicity to the recipient to prevent the development of alloantibodies.[75,78,79] Unfortunately, potential barriers exist that impact the number of blood donors with African lineage to fulfill the transfusion demands for patients with SCD, who are predominantly African American.[80]

Based on a qualitative study conducted in Canada, in collaboration with the Sickle Cell Disease Association of Canada and Sickle Cell Foundation of Alberta, multilevel barriers to donating blood identified by adults (aged 19−35 years) of African heritage include, but are not limited to, the following[80]:

- **Systemic racism and a lack of trust in the health care system**: Issues of systemic racism and mistrust often stemmed from personal experiences with discriminatory practices within the health care system. Additionally, exploitation, awareness of mistreatment, and prejudice in areas unrelated to blood donation shaped the perspectives and expectations of participants, their families, and their communities concerning blood donation.
- **Sociocultural beliefs and views on SCD:** Influenced by sociocultural perspectives, perceived stigma related to SCD was recognized as a potential barrier to donating blood. For example, some participants noted the absence of candid conversations about SCD within their families and communities, or a sense of "secrecy" prompting participants to regard SCD as a topic that should remain unaddressed.
- **Limited knowledge about the importance of blood compatibility for people with SCD:** Although all participants were familiar with SCD and most participants were acquainted with someone affected by it, most participants were unaware that blood donation from individuals of African descent played a pivotal role in enhancing the probability of achieving a compatible blood-type match and improving outcomes for recipients with SCD.
- **Deferral criteria:** The most common reason for deferral of blood donation was due to low hemoglobin levels. Notably, reference ranges for hemoglobin, among other hematological markers, have shown to be comparatively lower for African Americans relative to White people. This potentially substantiates the perspective held by some participants that hemoglobin level criteria disproportionately hinders the inclusion of individuals within the Black community, suggesting a need for race-specific deferral criteria across blood collection services.

Other unique caregiver-reported barriers to receiving CRCT included obtaining and maintaining venous access, concern of alloantibodies, and infusion-related reactions; provider-reported barriers included concern for iron overload, patient adherence to chelation, and challenges in convincing the family that CRCT is needed.[74]

7.0 CARE MODELS

The 2020 National Academies' consensus report, *Addressing Sickle Cell Disease: A Strategic Plan and Blueprint for Action,* identified six key components of an ideal healthcare model (see **Table 2**) based on reviewing various care models including the patient-centered medical home, comprehensive primary care plus, and the hemophilia care model, as well as reviewing established cystic fibrosis center models.[2] The following key components in **Table 2** can help formulate a healthcare model that provides high-quality, comprehensive SCD care.

Table 2. Key Components of an Ideal Healthcare Model[a]

Key Component	Description
Team-based care	• A multidisciplinary team, led by an SCD specialist, that includes patient and family engagement to facilitate shared decision making • Alternative appointment types are offered (eg, telemedicine visits) to overcome geographic barriers • Pre-visit planning
Patient-centered care	• Care is aligned with patient's goals, preferences/values, and needs • Care incorporates shared decision-making practices, and considers cultural appropriateness • Includes physical and physiological care by using a multidisciplinary team-based approach • Patients are empowered to self-manage their own disease state • To ensure evidence-based care is provided, clinical decision support tools should be used by healthcare providers • Support community outreach efforts
Patient access to care	• Patients have access to outpatient services, medical records, monitoring and assessments, and continual care
Case management	• Risk evaluation and mitigation via care management • Individualized care plan and patient-centric care management
Care coordination and transitions	• Transitions of care • Newborn screening
Quality improvement initiatives	• Indicators are developed to cue which patients need evaluation • Transparent methods for measuring and documenting clinical outcomes • Quantitative and qualitative evaluation • Benchmarking • Ongoing enhancement for data-driven quality improvement

[a] *These key components of an ideal care model were reported in "Addressing Sickle Cell Disease: A Strategic Plan and Blueprint for Action", published in 2020 by the National Academies of Sciences, Engineering, and Medicine*

With an increasing number of patients with SCD reaching adulthood (>95% of pediatric patients),[4,52] there is a necessity to restructure the approach of disease management towards chronic care; this framework emphasizes pain crisis (VOC) prevention, and appropriate management of SCD-related complications.[53,81] Establishing an integrated health care delivery system by using models for adult SCD

care that emphasize health equity is integral to enhancing health care outcomes for this patient population.[53] In a 2020 publication by Kanter et al, surveyed adult SCD treatment centers were categorized based on their models of care dictated by the center size, patient population, structural organization (within a department or division, stand-alone), services, and clinical staff.[4] Four models of SCD care were outlined: 1) classic comprehensive; 2) embedded care; 3) specialized medical home; and 4) hub and spoke.[4] A brief description of each type of care model is provided below[4]:

1. **Classic comprehensive care model**: Centers with this type of model provide organized, team-oriented SCD management through dedicated space and clinical staff. This type of model is commonly used in centers that are located in urban areas within extensive hospital networks or academic institutions, and tend to cater to the needs of a sizeable SCD patient population.
2. **Embedded care model**: SCD centers using the embedded care model are "embedded" or assimilated into a larger, financially viable care program (eg, comprehensive cancer center), allowing for resources to be shared across programs. This type of care model is often used for centers within urban areas that provide care to a smaller SCD patient population, or lack sufficient designated resources.
3. **Specialized medical home care model**: A unique attribute of this type of model is that primary care is integrated within the sickle cell care framework, thereby allowing the primary care physician to be the "designated care coordinator". This enables coordinated care between primary and specialty care, and offers flexibility to patients by addressing multiple services within a single center. The specialized medical home care model is often used at centers located in urban and suburban areas.
4. **Hub and spoke care model**: Unlike the aforementioned care models, the hub and spoke care model uses a networked system of care. The model includes a well-established, comprehensive central center ("hub"), augmented by secondary, distant centers ("spokes") that provide a narrower range of services; centers with any of the aforementioned care models have the potential to function as a "hub". The design enables the network to be highly scalable to allow for additional facilities as needed. Therefore, this type of model is ideal for rural areas which usually have limited access to care.
    a. Project Extension for Community Healthcare Outcomes (ECHO) uses this type of model to educate providers in the community (act as "spokes") by using telementoring offered by specialist providers (act as "hub") to provide high-quality care.[2]

Table 3 provides an overview of the four adult SCD care models and summarizes key differences.

Table 3. Adult Sickle Cell Disease Comprehensive Care Models[a] [4]

	Classic Comprehensive	Embedded Care	Specialized Medical Home	Hub and Spoke
Clinical space	• Dedicated clinical space • Typically includes a day hospital or infusion center to offer transfusions for acute pain management of VOCs (pain crises) • Centers that do not have a dedicated infusion center have designated locations for acute treatment, and specific care plans	• May share resources by becoming "embedded" within a larger, more financially viable care program (eg, cancer center) • Clinical space is shared between programs	• Dedicated clinical space • May include a day hospital or infusion center ○ Must have defined plans/protocols for acute care management, including pain	• Organizational design is a healthcare network: a central comprehensive center serves as the "hub", and is augmented by secondary, distant centers ("spokes") that provide a narrower range of services • "Hub" may use classic comprehensive, embedded care, or specialized medical home care models • Clinical space at "spokes" tend to be limited, and may need to share infusion space with other programs
Staffing	• Focus on team-based care, with dedicated clinical staff • Care is led by an SCD specialist, supported by APPs (eg, physician assistants, advanced nurse practitioners) • Often have plans/protocols with PCPs to ensure coordinated care	• Care is led by an SCD specialist • Supporting APPs may be shared with the primary program to reduce costs and improve efficiency, given that coordinated, team-based care is provided to patients with SCD • Should have plans/protocols with PCPs and other services to ensure coordinated care	• Focus on team-based care, with dedicated clinical staff • Care is led by an SCD specialist, but care is delivered simultaneously and collaboratively with the PCP (designated care coordinator) • "Patients who prefer to maintain a PCP relationship outside of the medical home can still receive SCD care at the specialty center."[4] (page 3809)	• Care is provided by a network of clinical staff • Central "hub" is led by an SCD specialist, with immediate access to subspeciality services • Distant centers "spokes" may be led by APPs with specialized training in SCD, or PCPs
Environment	• Urban locations within academic centers or larger hospital systems • Provide care to a relatively large population	• Urban locations, but do not have the dedicated space/staffing, or care for a lower number of patients with SCD	• May be considered for urban and suburban areas • Unlikely to be financially feasible in rural areas	• Able to provide care to multiple geographic areas, including rural regions
Access to services	Must have prompt availability to radiology, apheresis, laboratory services, a blood bank, and other essential specialties required for the management of SCD			• "Spokes" must have the capability to offer both transfusion and infusion services within their immediate premises • "Spokes" should be equipped with telehealth functionalities to communicate with the central "hub", and have plans/protocols for collaborative management, including of acute pain, and emergent situations

Example(s)	• Sickle Cell Center for Adults at John Hopkins Medicine (Baltimore, Maryland)
	  ○ Provides care for patients with SCD who live in the metropolitan areas of Baltimore and Washington, DC
	  ○ Offers genetic counseling, routine outpatient visits, pain management, transfusions, education, referral to other specialties/social services
	  ○ Full-time hematologists, APPs
	  ○ Integrated with John Hopkins Medicine healthcare system, including the emergency department
	  ○ Dedicated day hospital and infusion center
		• University of Connecticut Health Center (Farmington, Connecticut)
		  ○ SCD center is "embedded" within the cancer center
		  ○ Helped reduce hospitalization rates, including for acute pain management, and duration of inpatient admission, and improved the yearly number of preventative outpatient visits
		  ○ Improved access to SCD-specific care, as indicated by an increase in hydroxyurea use for eligible patients
		  ○ Transitioned into a classic comprehensive care model with dedicated space and clinical staff after receiving hospital resources
			• The Lifespan Comprehensive Sickle Cell Center at the Medical University of South Carolina (Charleston, South Carolina)
			  ○ From 2014 to 2018, provided adult specialty and primary care
			  ○ For ease of coordinating communication and scheduling appointments, 55% of patients received primary care at the specialized center
			  ○ This model was shown to decrease emergency department visits by 65% and hospitalizations by approximately 46%
			• The Adult Comprehensive Sickle Cell Center at the Ohio State University (Columbus, Ohio)
			  ○ Partnered with internal medicine providers who visit patients at home to allow for longer duration visits
			  ○ Improved immunization rates, cancer screenings, and reduced hospital (re)admissions, and length
				• South Carolina has a hub and spoke state network: the "hub" is the Lifespan Comprehensive Sickle Cell Center at Medical University of South Carolina (Charleston, South Carolina), with several "spokes" throughout the state, including Beaufort Memorial Hospital
				  ○ A full-time APP, who previously trained at the central "hub", runs the Beaufort Memorial Hospital SCD clinic
				  ○ Care is provided under the supervision of the central "hub" SCD specialist
				  ▪ Sees patients remotely via telemedicine, and in-person on a quarterly basis

[a] The care models do not address the capability to conduct clinical research and the need for connections with SCD community-based organizations (CBOs). While it is anticipated that both recently established and well-established SCD centers would engage in clinical research and collaborate with local CBOs, it is not considered essential.[4]

Abbreviations: APP, advanced practice provider; DC, District of Columbia; PCP, primary care physician; VOCs, vaso-occlusive crises; SCD, sickle cell disease

Regardless of the care model used at an institutional level, a multidisciplinary team approach, led by an SCD specialist, is essential to provide high-quality care to patients with SCD.[2,4] Essential staff of the team include an SCD specialist, social workers, patient coordinator/navigator, and advanced practice providers.[4] As part of comprehensive SCD care, patients should have access to acute and chronic pain management, specialist services, and transfusions (including apheresis). Optimal elements[‡‡], specifically for an adult comprehensive SCD center, were lead nurse/clinic manager, behavioral health staff, and access to contraception. Additional optimal elements included having an established pediatric-to-adult transition of care plan, and a dedicated infusion center or day hospital to reduce ED visits and provide prompt SCD-specific care. Adjunct elements, which are not required but are preferred, included a physical/occupational therapist, a pharmacist, a primary care provider (in an embedded model), an SCD educator, access to dental care, and dedicated clinical space and staff.[4]

Innovative care models may also incorporate community-based organizations (CBOs; eg, state-level chapters of Sickle Cell Disease Association of America [SCDAA]), as evident by the CDC-designed model for managing chronic diseases.[2,82] This model uses all of the following strategies[2,82]:

- Epidemiology and surveillance
- Environmental approaches that foster wellness and encourage health-promoting behaviors
- "Health system interventions to improve the use of clinical and preventive services"[2] (page 229)
- Community resources connected to clinical services

The 2020 National Academies' consensus report, *Addressing Sickle Cell Disease: A Strategic Plan and Blueprint for Action,* encourages that CBO integration should be explored further to improve care and services for individuals with SCD, acting as intermediaries between the patient population and health care system.[2] Authors note that in order to transition effectively from local-level impact to system-level change, it is imperative to reorganize SCD CBOs and patient advocacy groups; this involves clarifying their capabilities and integrating their role within the health care system.[2] Refer to **Appendix C** for additional details on CBOs, including the number of organizations by state.

7.1 Pediatric-to-adult care transition model

In 2011, the American Academy of Pediatrics (AAP), in collaboration with the American College of Physicians (ACP) and the American Academy of Family Physicians (AAFP), developed an expert opinion and consensus statement providing recommendations to effectively transition adolescents from a child/family-oriented model of care to an adult/patient-oriented model of care.[2,83] Since then, Six Core Elements that define the foundation of a structured transition process have been created by the Got Transition/Center for Health Care Transition Improvement, a national resource center, to ensure an optimal transition for pediatric patients to adult care[2,84,85]; this pre-transition approach is recommended in the updated AAP, ACP, AAFP clinical report (2018),[86,87] and has undergone quality improvement evaluation in numerous practice settings.[2,55,85] Although the Six Core Elements of Health Care Transition remain consistent across settings, the approach can be tailored to various practice settings (eg, family

‡‡ Optimal elements were not considered necessary for an adult comprehensive center by surveyed respondents, but may be valuable resources

medicine, internal medicine),[88] and subspecialties.[55] A statement specific to patients with SCD, issued by the Association of Pediatric Hematology/Oncology Nurses (APHON) and the American Society of Pediatric Hematology Oncology (ASPHO), recommends that all patients with SCD receive preparation for adult transition from pediatric care.[89] Recommendations from this position statement regarding early transition discussions, collaborative involvement in developing a transition plan, conducting transition assessments, and care coordination between pediatric and adult providers during the transfer are in alignment with the Six Core Elements of Health Care Transition. The APHON/ASPHO statement recommends transition assessments annually which may include addressing SCD complications and SCD inheritance, and to consider a phased transfer approach to prevent patients from transitioning out of both primary and specialty care simultaneously.[89] Importantly, although the transition process is initiated at the pediatric health care facility, the adult practice should also play a proactive role in the patient transitioning process.[53] **Table 4** provides an overview of the Six Core Elements for transitioning an adolescent to an adult healthcare provider.

Got Transition provides additional resources, including implementation guidance using a quality improvement process,[90] customizable sample tools,[85] and payment reimbursement options.[91] The American Society of Hematology (ASH) has created a SCD-specific pediatric-to-adult transition toolkit that includes patient assessment readiness forms and clinical summary forms.[92]

Table 4. Six Core Elements of Healthcare Transition for Youth Transitioning to Adult Care[85] [a]

	Core Element	Timeline based on youth age (years)[88]	Process milestones
1	**Transition and care policy/guide**	12 to 14	• Collaboratively create a transition and care policy/guide with involvement from adolescents and their parents/caregivers, outlining the transition approach, the adult-centered care approach regarding confidentiality and informed consent, and appropriate transitioning age to an adult provider. • Provide comprehensive education to all personnel regarding the transition approach, and educate about patient, parent, and pediatric and adult health care team roles during the transition process, taking into consideration cultural values/preferences. • Display the transition and care policy/guide in a readily accessible location within the practice area. • Initiate discussions and distribution with adolescents starting between ages **12 and 14**, and parents/caregivers, and routinely revisit it as an integral aspect of continual health care.
2	**Tracking and monitoring**	14 to 18	• Formulate criteria and procedures for identifying transition-aged adolescents • Construct a method for tracking the utilization of the Six Core Elements, and if feasible, integrating it with the electronic medical record.
3	**Transition readiness**	14 to 18	• Perform routine transition readiness evaluations, starting between ages **14 to 16**, to identify and engage adolescents and their parents/caregivers in conversations about self-care needs and strategies for accessing health care services. • Offer education and resources on essential proficiencies identified during the transition readiness evaluation
4	**Transition planning**	14 to 18	• Formulate and consistently update the care plan, incorporating results from the readiness evaluation, the goals and prioritized steps of the adolescent, a succinct medical overview and emergency care plan, and if necessary, a condition fact sheet and legal documents. • Equip both the adolescent and parent/caregiver with the necessary preparation for an adult approach to health care, including changes in legal decision-making, privacy and consent, self-advocacy, and information access. • Assess the need for decision-making support for the adolescent and direct them to legal resources as required. • Collaborate with the adolescent and parent/caregiver to determine the most suitable timing for the transfer from pediatric to adult care. For patients with both primary and specialized care, the ideal timing of transfer for each setting should be discussed. • The adolescent should receive assistance in identifying a provider within the adult health care system, and resources for insurance, self-care management, and local community support services should be provided. • For the disclosure of medical information, consent from the adolescent/parent/caregiver should be obtained. • Take into consideration cultural preferences throughout the process of transition planning
5	**Transfer of care**	18 to 21	• Assemble the comprehensive transfer dossier, including the final transition readiness evaluation, care plan with transition goals and prioritized steps, a medical synopsis and emergency care plan, and if necessary, condition fact sheet, legal documents, and supplemental clinical records. • Verify the date of the first appointment at the adult practice • Prepare a letter with the transfer dossier, and send it to the healthcare provider at the adult practice; confirm the receipt of the transfer dossier. • Correspond with the designated healthcare provider at the adult practice regarding the pending transfer of care. • Confirm the continued responsibility of the pediatric healthcare provider until the young adult is seen by the healthcare provider at the adult practice. • Facilitate the transfer of the young adult when their medical condition attains a state of maximal stability
6	**Transition completion**	18 to 23	• To confirm initial appointment attendance at the adult practice, the young adult and parent/caregiver should be contacted 3–6 months following the final pediatric visit. • The young adult and their parent/caregiver may be solicited for anonymous feedback concerning their transition experience • Establish communication with the adult healthcare practice to confirm the successful transfer completion, and offer consultation assistance, if required. • Foster ongoing and collaborative associations with adult primary and specialized healthcare providers

[a] *The Six Core Elements (version 3.0) reported in this table were released in 2020 by Got Transition, national resource center dedicated to health care transition*

8.0 ACTIONS TO ADDRESS HEALTH DISPARITIES AND RACISM

The CDC is dedicated to promoting diversity, equity, and inclusion within the health care system, as emphasized by a presentation delivered by Dr. Rochelle P. Walensky, the previous Director of the CDC (2021 to 2023),[93] during the ASH's Grassroots Network Lunch.[94] Dr. Walensky proposed four actions that healthcare providers/systems may consider to mitigate health care disparities and oppose racism at the structural, institutional, and interpersonal level[94]:

- **Recognize** occurrences of structural or interpersonal racism within the health care environment and decide to implement measures aimed at addressing and mitigating such instances
- **Educate** healthcare practitioners about the distinctive needs of patients with hematological disorders, including the potential impact racism has on patient outcomes
- **Advocate** for patients' welfare and needs, and provide information and tools to access necessary health care services
- **Listen** to patients to understand their goals/aspirations and needs, while offering support throughout their course of treatment

Table 5 provides specific examples for each of the aforementioned actions to address health disparities and racism.

Table 5. Four Proposed Actions to Address Health Disparities and Racism in the Health Care System[94 a]

Action	Examples
Recognize	• Use appropriate language and educate others to use the same approach by refraining from using derogatory terms such as "sickler" when referring to patients with SCD. • Practice mindfulness and engage in introspection while caring for patients with SCD, acknowledging that people have inherent biases. • Confront instances of interpersonal racism by openly addressing the subject of race, fostering an environment where healthcare professionals can feely converse about matters of race and racism (safe spaces), and report incidents as they arise. • Reflect on the manifestation of racism within research settings, evaluating the influence of race and racism on the allocation of funding for disease-related research.
Educate	• Implement mandatory training on racial implicit bias for all healthcare practitioners in a nurturing environment. • To increase knowledge and proficiency in evidence-based SCD guidelines, training programs should be encouraged. • Conduct research and surveillance to acquire insights into disease risk factors within populations that have historically been underrepresented in clinical trials.

Advocate	• Offer patients psychosocial support (eg, patient navigators, social workers, psychologists)
	• Establish structured reporting mechanisms within hospitals to systematically record and address instances of racist conduct.
	  ○ May be similar to systems that are used for quality improvement or safety incidents
	• Antiracism task forces should include patients with SCD, or their advocates
	• Implement specific SCD pain management protocols to expedite opioid administration and improve health outcomes.
	• Facilitate a mechanism through which patients with SCD can safely express concerns about racial bias or inequitable treatment.
	• As appropriate, provide patients with information on clinical trials during their course of treatment.
	  ○ The underrepresentation of diverse populations in clinical trials results in health disparities within medical research, and ultimately, health care practice

Listen	• Develop collaborations with patients; providers should acknowledge their patient's ability to educate them on the influence of race and racism related to their personal experiences within the health care system. • All patients should be treated with courtesy and dignity. • All patients should have their needs addressed by ensuring the diagnosis is accurate. • Providers should attentively listen to patients in an effort to understand the experiences of marginalized and minority populations, and recognize that disease risk factors may differ based on race. • Providers should demonstrate sensitivity to cultural nuances and actively participate in partnerships with communities of color.

[a] *The four actions reported in this table (recognize, educate, advocate, and listen) were proposed by Dr. Walensky, the previous Director of the CDC, on December 11, 2021 at the ASH's Grassroots Network Lunch.*[88]

Abbreviations: ASH, American Society of Hematology; CDC, Centers for Disease Control and Prevention; SCD, sickle cell disease

9.0 RECOMMENDATIONS FOR CONSIDERATION

Utah currently lacks any established nonprofit or governmental SCD program,[95] aside from universal newborn screening (NBS) for sickle cell status.[28,49] Although specific recommendations for consideration related to funding are not proposed, it is ideal for policymakers to collaborate with state level partners (eg, advocacy groups, professional associations, community-based organizations [CBOs]) and/or secure grants to support SCD programs.[96] To help improve care to patients with SCD in Utah, we propose several recommendations for consideration based on the current gaps in the management of SCD, and as applicable, provide examples of an approach to that recommendation from other states:

1. *May consider developing a Sickle Cell Task Force consisting of, but not limited to, the following: hematologists, person or caregiver of a person with SCD, members of CBOs with experience in managing patients with SCD, and a healthcare institution representative.*
    a. In 2019, Texas passed legislative action allowing the establishment of a task force based on House Bill 3405.[97-99]
        i. The task force includes 7 members appointed by the Executive Commissioner of the Health and Human Services Commission[99]: 1 healthcare institution representative, 2 hematologists, 2 persons or relatives of a person with SCD or SCT, and 2 members of a SCD-experienced CBO
    b. As mandated by Act No. 117 (Senate Bill 57), Louisiana Legislature created the Louisiana Sickle Cell Commission in 2013 to improve the health of individuals with SCD in their state through 4 workgroups: Patient Navigation, Medical Servies, Education and Advocacy, and Data and Surveillance.[100]
        i. The Louisiana Sickle Cell Commission includes 11 members; of these, 8 members across various organizations are appointed by the governor, with required confirmation by the Senate.[100]
2. *May consider setting up a statewide sickle cell registry for disease surveillance*
    a. Although NBS for SCD and SCT is conducted on all infants born in Utah,[28] there is currently no statewide sickle cell surveillance registry (based on the participating states of the Sickle Cell Data Collection [SCDC] program).[43] Identifying and monitoring patients with SCD throughout their lifespan could help to address additional barriers to care, especially during the transitioning period from pediatric to adult care in which patients with SCD are at a high risk of morbidity and mortality.[2,51-54] Additionally, long-term monitoring of sickle cell carriers could provide genetic insights.[2]
    b. The registry should have a defined purpose and allowable uses, and ensure that health information is protected according to federal and state regulations (eg, Health Insurance Portability and Accountability Act [HIPAA]).[44,46]
        i. Discussion may be needed to determine how the collected data will be used (eg, inform policy changes, identify patients who are unable to access routine care, serve as a resource for ED providers to verify a patient's diagnosis).[44]
    c. If feasible, may consider applying to the SCDC program to become a participating state.[41]
3. *May consider raising awareness of SCD and SCT in the state by developing a statewide public campaign to engage the community, healthcare providers, and other relevant entities*

a. May consider launching the awareness campaign annually each September to align with the national-designed Sickle Cell Awareness Month,[68] that may include an SCD event at the state Capitol.[96]
b. The campaign may include disseminating educational resources (via email or social media), and sharing patient and/or provider experiences. Additionally, it may involve engaging the public in supporting patients with SCD through blood donations.[99]
    i. May share educational brochures at doctor's offices, hospitals, treatment centers, and faith-based institutes; consider translating the brochures into other languages.[96]
c. The Sickle Cell Statewide Family Support Initiative implemented in Ohio, under the Sickle Cell Services Program, allocates funding to support efforts that provide statewide education, increased awareness, and community engagement towards patients or families of those with sickle cell hemoglobinopathies, and professionals dedicated to their care.[101]
d. South Carolina has a Sickle Cell Program that increases public awareness of SCD by educational initiatives and aiding those in need.[102] Aside from providing educational resources, the program offers support for financial assistance of outpatient medical services, equipment, supplies, and prescription medications to patients with SCD, and provides care coordination as needed.[102]

4. *May consider implementing healthcare provider (including students) educational sessions on best medical practices for patients with SCD, which may be especially valuable for healthcare professionals who care for adults in the emergency department (ED) or primary care*[96]
    a. May consider creating an educational SCD webinar targeted for healthcare professionals, or local/regional educational symposiums for healthcare providers.[96]
        i. Educational sessions can encourage institutions to adopt a feasible healthcare model for patients with SCD, based on the institution location, size of the patient population, and available resources (eg, clinical staffing, services).
    b. Encourage local medical associations to promote educational talks (using a "Ted Talk" model) on SCD, making it available to watch online or on a podcast channel.[96]
    c. May consider identifying potential grants for bolstering physician residency and nursing programs to incorporate practical training for SCD from a dedicated center.[96]
        i. As part of the Sickle Cell Initiative in Ohio, grant funds are provided to enhance professional education on SCD, among other hemoglobinopathies.[101]
5. *May consider ensuring that infants with SCD identified by NBS have appropriate follow up care coordination, and are offered appropriate resources upon diagnosis (eg, genetic counseling)*
    a. Maryland Department of Health has a SCD follow-up program, enacted by legislation, designed to support patients with SCD (18 years of age and younger) by providing information on SCD, preparing for daycare/school, and transitioning into adult care, among other resources.[59,103] The information appears to be tailored to specific ages, and is given starting at the time of a confirmed diagnosis.[103]
    b. Under the Sickle Cell Initiative in Ohio, patients with abnormal hemoglobin testing results (newborn or non-newborn) are tracked and followed-up.[101] Positive cases are offered disease education or counseling, and are directed to specialized providers and/or resources.[101]
    c. The Louisiana Genetic Diseases Program is set up to ensure that genetic evaluation/counseling and referral to specialized SCD centers, if needed, are performed in a timely manner.[104]

6. *May consider increasing awareness of more inclusive criteria at blood banks to increase participation by donors of African heritage*
    a. If it is not already in use by blood collection services, race-specific deferral criteria for hemoglobin thresholds can be implemented to encourage blood donations by the Black community. Currently, the American Red Cross performs a hemoglobin screening test prior to each blood and platelet donation.[105] Donors may be deferred for having hemoglobin values below the normal range.[105] The normal hemoglobin range for persons of African ancestry is lower by 0.7 g/dL than for White people, suggesting that different thresholds for excluding people from blood donation due to anemia should be used for people who self-identify as Black.[106]
    b. According to 2022 US Census Bureau data, 1.6% of the population in Utah is African American or Black people.[33]
        i. Due to the potential for blood antigens to differ by race, blood donation from individuals of African descent often increases the probability of achieving a compatible blood-type match for recipients with SCD,[75,78-80] who tend to be predominantly African American/Black people.[3]
7. *Upon understanding the prevalence of SCD in Utah and identifying the required resources to support a statewide approach to implementing the infrastructure needed for SCD programs and services, may consider developing a strategic state plan that outlines the projected milestones over the next several years.*

10.0 SUMMARY

Sickle cell disease (SCD) is a rare, chronic, autosomal recessive, hematological disorder that predominantly affects individuals of African American/Black (1 in 365 births) or Hispanic (1 in 16,300 births) heritage.[2,3] SCD causes multi-organ complications (eg, stroke, infections, chronic anemia),[2,21] with the most prevalent being severe acute pain episodes, also known as vaso-occlusive crises (VOCs) or sickle cell pain crises.[10] Because SCD-related complications reduce quality of life and life expectancy,[2,23] patients with SCD require specialized and well-coordinated medical care across their lifespan to ensure positive outcomes.[1,2]

However, comprehensive care for SCD has been inadequate,[2] even compared to other, more rare conditions (eg, hemophilia, cystic fibrosis).[3] Much of this inadequacy can be partially attributed to health care inequities.[2] Thus, strategic policy interventions are warranted to address the unmet needs of the SCD patient population.[2]

Certain barriers to adequate health care repeatedly emerge in care systems intended to serve SCD patient populations in the US[§§]. Through interviews conducted with Utah SCD specialists, we determined that the main barriers in Utah include the following:

- No formal SCD pediatric-to-adult transition of care program at institutional- or state-levels

[§§] For information on the potential barriers to receiving newer disease-modifying agents, please refer to the Drug Utilization Review (DUR) report completed in July 2023, available at: https://medicaid.utah.gov/pharmacy/drug-utilization-review-board/

- No SCD-specific acute pain management facility (eg, day hospital or infusion center)
- Limited resources and few specialized SCD centers for adult care

Additional, more generalized barriers to adequate SCD care, as identified in published literature, include inadequate insurance coverage, disease or racial stigma, transportation issues, lack of provider expertise or knowledge in treating SCD, or negative provider or patient attitudes.[27,51] Some of these barriers impact not only patients with SCD, but also SCD treatments: adults of African heritage, who are most likely to have a compatible blood-type match with SCD recipients, experience barriers to donating blood that could be used for chronic red cell transfusion (CRCT), a guideline-recommended treatment for stroke prevention in patients with SCD.[74,80]

In an effort to improve care to patients with SCD in Utah and inform policymaking decisions, several recommendations for consideration are proposed based on identified gaps in SCD care and established SCD programs in other states. In fulfillment of Utah House Bill 487's provisions to "review and develop recommendations for improving the surveillance, screening, diagnosis, and treatment of sickle cell disease among residents of the state,"[18] important first steps include:

- Developing a Sickle Cell Task Force to determine surveillance, screening, diagnosis, and treatment priorities
- Setting up a statewide SCD registry
- Raising patient, provider, and public awareness of SCD in Utah

Despite the historically small SCD population living in Utah, demographics continue to change.[107,108] Between 2010 and 2021, the Hispanic population increased by 36.9% (360,487 in 2010 to 493,636 in 2021), and the Black population increased by approximately 50.0% (26,455 in 2010 to 39,687 in 2021).[107] If demographic trends projected in a 2019 report are accurate, the Hispanic and Black populations will continue to increase for the foreseeable future.[108] Therefore, SCD care infrastructure will become increasingly necessary.

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APPENDIX A: ADDITIONAL RESOURCES

Resource directory:

The Centers for Disease Control and Prevention (CDC) provides a national resource directory for patients on their website, which lists sickle cell centers/providers, and if available, other sickle cell organizations (ie, associations, nonprofits, foundations, support groups). Notably, this directory was published in June 2011, and therefore, the information is most likely outdated. For example, Intermountain Primary Children's Hospital (PCH) is the only listed sickle cell center for the state of Utah; to our knowledge, there are currently at least two centers, including this one, within the state that treat sickle cell disease (SCD). The directory is available at the following website: https://stacks.cdc.gov/view/cdc/11903

Provider network:

The Sickle Cell Adult Providers Network (SCAPN) is a national organization that serves as an interactive network for multidisciplinary healthcare providers who treat adults with SCD. A primarily goal of this organization is to expand engagement, support, and education to all healthcare providers, regardless of if they currently treat adults with SCD. More information on SCAPN can be found at: https://www.scapn.org/about-us

Organizational resources for emergency care:

The Emergency Department Sickle Cell Care Coalition provides additional information and resources to help improve outcomes regarding emergency care for patients with SCD; additional details can be found at their website: https://www.acep.org/by-medical-focus/hematology/sickle-cell

The American College of Emergency Physicians provides an in-depth guide for managing patients with SCD, available at: https://poctools.acep.org/POCTool/04082647-6422-473b-83ee-3a5b6291a415

State best practices guides for treating patients with sickle cell disease (SCD):

Although the American Society of Hematology (ASH) and the National Heart, Lung, and Blood Institute (NHLBI) have guidelines addressing acute and chronic pain management in SCD,[64,67] the Commonwealth of Pennsylvania has also published best practice guidelines for pain management in patients with SCD, available here: https://www.health.pa.gov/topics/Documents/Opioids/SCD%20Guidelines.pdf

Louisiana has a best practice guide, created by Dr. Renee Gardner (a pediatric hematologist) and the Louisiana Sickle Cell Commission, for treating patients with SCD, published in 2017; the guide can be found at: https://ldh.la.gov/assets/oph/Center-PHCH/Center-PH/genetic/LAStandardsforCareSickleCellFinal2017.pdf

APPENDIX B: INTERVIEW DETAILS

We conducted 2 formal interviews with local hematologists, pharmacists, and other relevant personnel (eg, program manager, health equity specialist) affiliated with the Hematology Clinic at University of Utah (UU) Sugar House Health Center (SHHC),[19] or the Utah Center for Bleeding and Clotting Disorders at Intermountain Primary Children's Hospital (PCH).[20] Permission was obtained from the interviewees to disclose the information outlined below from the interviews.

One-on-one in-person interview: This interview was conducted with Jeffrey Gilreath, a clinical hematology/oncology pharmacist at the UU SHHC on June 21, 2023. The duration of the interview was approximately one hour. Pertinent notes from the interview included the following:

- UU SHHC is currently caring for approximately 15 to 20 adults with sickle cell disease (SCD)
- A potential reason for a lower SCD prevalence in Utah is that higher altitude and poor air quality both likely contribute to states of hypoxia, triggering sickle cell pain crisis and higher risk of hospitalizations. Patients with frequent exacerbations have higher mortality rates; patients may also leave the state seeking more suitable environments, depending on available resources.
- Dr. Ming Lim is one of 3 providers who treat SCD at this clinic.
- In comparison to hemophilia, in which this clinic treats approximately 600 patients, there are no formal transition systems/procedures set in place for patients transitioning from pediatric to adult care. Therefore, patients are likely to become lost to follow-up.
    - Hemophilia treatment is supported by a Health Resources and Services Administration (HRSA)-funded 340b program, which enables the clinic to provide pharmacy, nursing, and social support. Patients with hemophilia are provided disease education, assistance with navigating insurance barriers to acquiring expensive treatments, and outreach in the event of missed visits.
- There are often coordination of care issues. Patients may often experience sickle cell pain crisis leading to inpatient hospitalization, and often there is a lack of care transition support from the inpatient to the outpatient setting. Often times, communication with patients occurs electronically and/or via telephone between appointments, without any in-person visits.
    - Similarly, there are no established mechanisms for transitioning care to adult clinical settings when patients age out of pediatric clinical care settings.
- There is a lack of resources for patients with SCD (eg, access to disease education, genetic counseling, pharmacy resources, social worker support); inadequate amounts of these resources can impact outcomes. Patients are often required to advocate for their own health due to understaffing, including following up on any medication adverse effects, and scheduled appointments. However, patients tend to miss appointments due to being hospitalized for sickle cell pain crisis, feeling unwell, or dealing with family/life struggles, and therefore, care is often delayed.
    - Because of a lack of resources for appropriate care, patients tend to decompensate gradually over time.
- Although not unique to SCD, there are challenges navigating reimbursement (eg, insurance), and almost all the patients seen at this clinic experience financial difficulties affording necessary medication(s).

Zoom interview: The second interview was conducted remotely via Zoom on July 12, 2023. The meeting attendees included the following individuals affiliated with the Utah Center for Bleeding and Clotting Disorders at Intermountain PCH:

- Dr. Sasidhar "Sashi" Goteti, MD, MCR, FAAP, pediatric hematologist
- Heidi Lane, PT, DPT, PCS, program manager and physical therapist
- Leanne Rohrbach-Stange, MSW, CSW, health equity specialist and social worker

In addition to the aforementioned persons, Dr. Ming Lim, MBBCH, MS, an adult hematologist with the UU SHHC, also attended the meeting. The duration of the interview was approximately one hour. Questions were primarily directed towards Dr. Goteti and Dr. Lim, but other meeting participants were welcome to share their own insights and experiences. The following highlights relevant details discussed during the interview:

- Every infant born in Utah is required to undergo newborn screening for certain diseases, which includes SCD. The caregiver of the infant is notified if a positive status is detected, and pediatric hematology contacts either the caregiver directly or the primary care physician to schedule consultation at PCH.
    - There is no mechanism for ensuring appropriate follow-up with patients who have SCD and move to Utah from another state.
- In the last year, there have been 3 positive screens for SCD at PCH. Currently, there are approximately 45 patients with SCD being treated at PCH.
- The UU SHHC is currently treating approximately 20 adults with SCD, cared for by 5 different physicians.
- Not all patients that transition from Intermountain PCH are able to be seen at the UU SHHC for adult care due to payer-driven restrictions; for example, the UU is not preferred in-network for several insurances (eg, Select Health, Cigna, Aetna), whereas this is less of a concern at PCH. Consequently, patients may be lost to follow-up (by UU SHHC) as they transition from pediatric to adult care.
    - Approximately 5 to 10 patients in the past year have been referred to other organizations within the state, and very few, if any, have transitioned to a sickle cell treatment center out of state.
- There is a concern of coordination of care between inpatient and outpatient settings; patients with SCD are often hospitalized due to severe pain crisis, unlike other hematological conditions (eg, hemophilia). Concern regarding fragmented care expands to transitioning from pediatric-to-adult care because mortality rates among patients with SCD have historically been the highest in the transition age group.
    - In addition, because SCD affects multiple organ systems, patients require access to not only hematologists, but also other providers (eg, pulmonologists, neurologists, nephrologists), making coordination of care even more challenging.
- Because there are limited resources available for patients with SCD, it can be particularly detrimental for the patient if a disruption in services occurs, and it may be challenging to appropriately optimize existing resources at an institutional level.
- The best disease state model for SCD is hemophilia; SCD and hemophilia are both funded federally by HRSA. However, funding for SCD is in its infancy relative to hemophilia, which has been in place

since the 1970s. Moreover, unlike the hemophilia grant, the SCD grant does *not* allow grantees to participate in the federal drug pricing program; generated income from the discounted drug price allows sustainability of the hemophilia program by acting as an additional source of income.
    - Despite federal funding for SCD, institutions are often burdened with supporting most of their available resources.
- Having a collaborative network between institutions is highly valuable to share best practices and ongoing education for SCD, and to encourage provider engagement.
- PCH does outreach once a year to ensure care for patients in rural areas. When clinically appropriate, virtual visits are used to help accommodate patients. However, patients living in rural areas of the state often need to travel long distances to access care at PCH or UU SHHC.
    - PCH is the only pediatric hematology clinic in Utah that is able to care for patients with SCD.
- There are barriers to care that may be attributed to racism and socio-economic status which may be unique to SCD.
- Having a healthcare model that uses multidisciplinary team-based care is ideal to coordinate primary and specialty care for patients with SCD.
- Currently, Intermountain PCH and UU SHHC do not have a dedicated infusion center for *severe* sickle cell pain, which is often uncontrolled with oral analgesics; therefore, patients are sent to the emergency department (ED) for pain management, which is a time-intensive process, and often results in feelings of frustration for the patient.

APPENDIX C: STATE-LEVEL SICKLE CELL ADVOCACY GROUPS AND/OR COMMUNITY-BASED ORGANIZATIONS

One potential method for improving care to patients with sickle cell disease (SCD) is by creating state-level patient groups or community-based organizations (CBOs). According to information on public online platforms, there are currently no sickle cell nonprofits, foundations, or associations in Utah. **Table B1** provides the number of sickle cell nonprofits, foundations, or associations in other states as of October 2019, organized from highest to lowest.[2] Notably, the geographic location for some organizations does not necessarily restrict support to only residents of that particular state.[109] There are also national organizations for SCD, including Sickle Cell Disease Association of America (SCDAA) and the Sickle Cell Disease Coalition (by American Society of Hematology [ASH]).[2]

Table B1. Number of State-level Sickle Cell Nonprofits, Foundations, and Associations[2] [a]

State	Number of organizations	State	Number of organizations
Florida	15	Georgia	2
California	14	Illinois	2
Alabama	8	Kentucky	2
North Carolina	7	Massachusetts	2
Ohio	6	Mississippi	2
Louisiana	5	**New Mexico**	2
Maryland	5	Washington	2
Pennsylvania	5	**Arizona**	1
Colorado	4	Indiana	1
Connecticut	4	Kansas	1
Missouri	4	Michigan	1
South Carolina	4	Minnesota	1
Texas	4	**Nevada**	1
Virginia	4	New Jersey	1
New York	3	Oklahoma	1
Arkansas	2	**Oregon**	1
Delaware	2	Tennessee	1

Bolded states *are those that are located in the western United States (US); unlisted states were not identified to have any sickle cell organizations (ie, nonprofits, foundations, or associations).[2]*

[a] This information was provided in "Addressing Sickle Cell Disease: A Strategic Plan and Blueprint for Action", published in 2020 by the National Academies of Sciences, Engineering, and Medicine; please refer to this publication for the names of the organizations.[2]