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Forma Sickle Cell Disease Trend Report: The State of Transition

U.S. Perspectives On Transition from Pediatric to Adult Care

2021: First Edition

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An informational report provided by Forma Therapeutics, Watertown, MA
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Forma Sickle Cell Disease Trend Report: The State of Transition
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On November 30, 2021, Forma Therapeutics issued a press release announcing the appointment of Dr. Osunkwo to the company's executive leadership team as the company's inaugural chief patient officer and senior vice president. In this role, Dr. Osunkwo will be responsible for realizing Forma's vision to transform the lives of patients by improving access and care through partnerships with global patient and community stakeholders. She will join Forma in the first quarter of 2022.

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About Forma Therapeutics

Forma Therapeutics is a clinical-stage biopharmaceutical company focused on the research, development and commercialization of novel therapeutics to transform the lives of patients with rare hematologic diseases and cancers. Forma's R&D engine combines deep biology insight, chemistry expertise and clinical development capabilities to create drug candidates with differentiated mechanisms of action focused on indications with high unmet need. Forma's work has generated a broad proprietary portfolio of programs with the potential to provide profound patient benefit. Forma's investigational medicine, etavopivat, is in a Phase 2/3 trial for SCD, The Hibiscus Study (NCT04624659). For more information, please visit www.FormaTherapeutics.com or follow on Twitter @FORMAInc and LinkedIn. Forma Therapeutics is located in Watertown, MA.

About formabridge

Together with the community, we will work to **support a safe transition** from pediatric to adult care and **strive to change the future of SCD.**

www.formabridge.com is a resource hub with educational and actionable information to help the community navigate transition. There is practical information on how to enroll and re-enroll in Medicaid, Disability, as well as other federal and state support programs. The site also includes educational and inspirational videos to engage and support patients during the transition process. Additionally, Forma is committed to supporting SCD transition in collaboration with the community through its corporate giving program. (www.FormaTherapeutics.com/corporate-giving/)

Report Disclaimer

This report is non-promotional in nature. While the development and publication are sponsored by Forma Therapeutics' *formabridge* program (formabridge.com), content of this report was prepared by consultancies Guidehouse and Anderson DDB with guidance from the report's Editorial Board. Members of the Editorial Board were compensated by Forma Therapeutics for their participation in this report. Sources of information include secondary research of published literature and primary research with patients, caregivers, and clinical professionals who were blinded to the report's sponsor. Statements and opinions contained within this report do not necessarily reflect those of Forma Therapeutics, but rather synthesize common themes gathered from the broader SCD community. No pharmaceutical brands were mentioned as part of this research, except to qualify participating respondents based on approved SCD therapies. Quotations may have been edited for clarity.

Introduction

As improvements in sickle cell disease (SCD) treatment and care have allowed more children to survive into adulthood, SCD has increasingly become a lifelong multi-system chronic and progressive disorder[1]. With this improvement in child mortality rates, transition from pediatric to adult care has emerged as a significant challenge for many patients during which health inequities can be amplified. Moreover, while pediatric mortality has decreased over the years, adult mortality rates have not[2]. Indeed, there is a tripling of mortality rates during the critical period of transition from pediatrics to adult care[3].

During this transition, patients with SCD tend to have more severe symptoms, higher healthcare utilization, and reduced access to lifesaving treatments than were previously available in pediatrics[4]. Data have shown that upon reaching adulthood, **over 59% of individuals with SCD have accumulated end organ damage** involving at least one organ (such as, stroke, chronic kidney disease, avascular necrosis, end-stage renal disease, pulmonary hypertension)[2]. At this pivotal point in their lives, many young adults are experiencing a significant care gap with interruptions to continuity of care[5]. Despite their substantial medical needs, there is a dramatic shortfall in the services and support available to young adults compared with the care they received as pediatric patients. Furthermore, the psychosocial needs unique to adolescents and emerging adults go unrecognized and unattended. While most children with SCD are cared for by specialists (e.g., pediatric hematologists), many adults transition to primary care providers due to the lack of a comprehensive nationwide network of adult SCD providers[6]. Patients with SCD experience increased medical, social, and personal challenges during their transition from pediatric to adult care, while many also confront the burdens of systemic racism, bias, and the stigma associated with SCD[5]. As Dr. Ifeyinwa Osunkwo, a lifespan hematologist and SCD expert at Atrium Health explains, "*all transitions are hard, but this is made 100 times worse when you are also dealing with a lifelong chronic, painful, and debilitating illness and are facing social determinants and societal stigma and neglect as is the case with SCD.*"

> *People care when you are a child but as an adult, they look at you as if you are lying or faking it.* ***No one cares when you are an adult****. It goes from being extremely cared for to no help at all.*
>
> **– Patient with SCD**

"*Transition is an important step in the life of someone who lives with SCD, and should be approached strategically,"* says SCD community advocate André Marcel Harris. *"Transition can be a virtually smooth and uneventful process if adequate preparation, planning, and education takes place.*" Thus, a comprehensive understanding of the challenges faced by young adults with SCD as they transition will help enable improvements in care, support programs and services, and policies to relieve patient burden of disease.

This report provides a general overview of SCD care for young adults, as well as providing focused perspectives on the challenges with transitioning from pediatric to adult care and potential solutions. It highlights the perspectives of individuals living with SCD or their caregivers (collectively termed "patients" in this report) and healthcare professionals to provide insights specific to their experience and challenges in an effort to facilitate discussion about opportunities to better support transition in SCD. "*Recognizing and spotlighting these challenges is the first step to identifying potential solutions and mitigation strategies — it takes all of us working together, communicating about the good, bad, and ugly of transition to make this work,*" says Dr. Osunkwo.

Summary of Key Insights

1 **Patients with SCD place a higher value on overall wellness and quality of life when considering treatment goals, while physicians focus more on clinical improvements**

Surveyed patients and physicians shared four of their top five treatment goals. There is still a mismatch in treatment priorities illuminating a key disconnect around the differing perceptions of the burdens associated with SCD and the need to receive lifelong medical care in a health system that is mired with discrimination and inequity.

2 **Social determinants of health and lack of support prevent adequate SCD management throughout transition**

62% of surveyed patients found their overall experience either somewhat or very challenging, with inadequate housing, transportation, information, and insurance listed as a few of the challenges patients face in transition that often impede treatment adherence and proper care.

3 **Discrimination and stigma impede prompt and ongoing access to appropriate care, especially in urgent situations**

78% of surveyed patients report discrimination and stigma prevent them from receiving the care they need, with patients reporting particularly negative experiences in emergency departments.

4 **Transition discussions begin late for many patients with SCD, preventing them from being adequately prepared when transition to adult care occurs**

Surveyed physicians and patients indicated transition discussions typically did not start until age 17, well beyond the Got Transition® recommended age of 12[7]. This means patients are ill-prepared for abrupt changes in insurance, health system culture, and access.

5 **Available guidelines and protocols are underutilized in the management of SCD care and in transition**

Only 38% of physicians report their institution utilizes either a standard guideline or toolkit for SCD management and transition (e.g., ASH Transition Readiness Toolkit or NHLBI SCD Guidelines). Lack of awareness and challenges with integration of guidelines into clinical practice are likely hurdles.

6 **Establishing trust and rapport with an adult physician while still in pediatric care is pivotal to transition success in SCD**

Meeting with an adult physician while still in pediatric care can be an important step in building a patient-physician relationship prior to actively transitioning. A majority of surveyed patients ranked this as their top factor for transition success.

7 **Community-based organizations are key to providing resources and facilitating access to care, particularly during transition**

When established and available, community-based organizations (CBOs) are seen by both patients and physicians as a helpful way to fill the access and education gaps many patients face during the transition period.

Methodology

To gather patient and healthcare provider perspectives, quantitative market research instruments were developed and deployed from 9/7/2021 to 9/24/2021 via a web-based survey platform. Recruitment was conducted via managed and focused e-mail distribution lists. All research participants were required to meet pre-qualification criteria for integrity of responses, and consented to having their responses used in this report. Honoraria were paid to market research participants who completed the survey. Research was conducted in a double-blinded manner in which participants were unaware of the identity of the sponsor and the sponsor remains unaware of the identity of the participants.

The surveys were specifically designed to understand challenges experienced by people living with SCD, particularly regarding young adults transitioning from pediatric care to adult care. Questions captured stakeholder perspectives related to ongoing management, treatment goals, use of transition plans, experience with healthcare services during transition, and desirable support services and resources, among other key topics. Survey programming logic directed participants to relevant and appropriate questions based on their individual characteristics. Respondents' beliefs, behaviors, or experience with specific pharmaceutical products were not the subject of this research.

Patient/Caregiver Demographics

The sample included 100 participants, comprising 82 patients living with SCD and 18 caregivers. To qualify, patients had to be 18–35 years of age, diagnosed with SCD, seeing a physician to manage SCD, and have undergone transition from pediatric care to adult care. Similarly, caregivers qualified if they were caring for a patient between the ages of 17–30 who met the same criteria. Age cutoffs differed for patients and caregivers to both capture perspectives of those under 18, and ensure relevance of responses across the transition time frame.

Respondents lived across the U.S. with 32 states represented. Across the sample, 48% of respondents lived in an urban setting, 42% suburban, and 10% rural. Patients represented a mix of insurance types.

PATIENT SNAPSHOT

- 100 participants surveyed
- The average patient age was 28 years old with respondents ranging from age 18–35
- 55% of respondents currently obtain treatment at a comprehensive SCD center
- Distribution of SCD genotypes:
  - » 61% sickle cell anemia (HbSS and HbS-β0)
  - » 17% sickle-hemoglobin C disease (HbSC)
  - » 21% other sickle cell genotypes, including hemoglobin S beta plus thalassemia (HbS β+), HbSD, HbSE or HbSO
  - » 1% unsure of specific genotype

Physician Demographics

The sample included 150 physicians (Figure 1), including 50 specialists practicing at a CSCC* and 100 specialists practicing at a non-CSCC. Qualified provider respondents had to be Board Certified or Eligible, have over 2 years of clinical practice, and spend over 25% of time in direct patient care. Respondents had to actively manage at least 10 SCD patients per year. Physicians practicing in a CSCC had to actively manage at least 50 SCD patients per year.

Respondents included physicians practicing across 29 U.S. states. Physicians practiced in a mix of 66% urban, 32% suburban, and 2% rural settings. Physicians cared for patients with SCD who obtained medical treatment paid by a mix of insurance types.

* For the purposes of this report, a comprehensive sickle cell center (CSCC) is defined as an institution with a dedicated sickle cell disease clinic that includes specialist providers and focused resources.

PHYSICIAN SNAPSHOT

- 150 physicians surveyed (all respondents were MDs)
- Respondents averaged 16 years in practice post residency, ranging from 2–42 years
- CSCC respondents treated on average 203 patients with SCD annually (ranged 50–1,900), while non-CSCC respondents averaged 132 patients with SCD annually (ranged 10–1,200)
- Respondents spent on average 89% of their professional time in direct patient care
- 51% of respondents practiced in the community and 49% practiced in an academic center
- 60% of respondents managed both pediatric and adult patients with SCD

Data Analysis and Reporting

Data were collected, blinded, aggregated, analyzed, and reported by Guidehouse. Subgroup analyses were conducted when sample sizes permitted and revealed notable differences and trends between distinct groups. The Editorial Board reviewed the report's analysis and provided interpretation and commentary.

A literature review was also conducted to supplement the primary research findings in this report and to further explore research topics. References are provided at the end of this report.

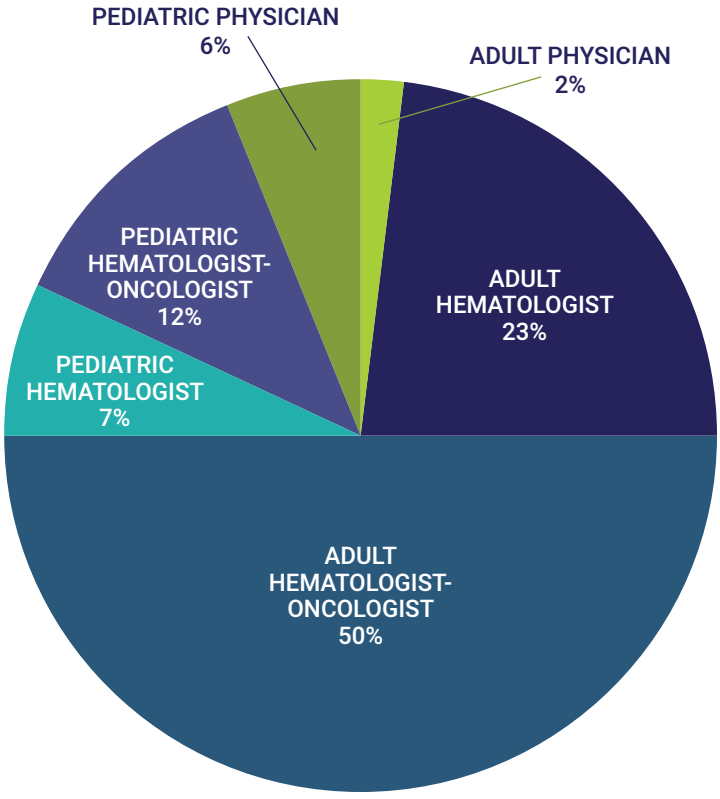


FIGURE 1.
PHYSICIAN DISTRIBUTION BY SPECIALTY
(N=150)

Treatment Goals & Challenges for Young Adults with SCD

SCD is a multifaceted disease burdening patients with physical, emotional and, socio-economic challenges that result in diminished quality of life. Patients state their disease has negatively affected their education, prevented them from holding stable jobs, robbed them of a sense of control of their days, and even impacted their ability to parent.

> *If you get into a crisis you will miss school, and in college they do not provide tutors like they do for sickle cell patients in grade school, so it makes it much harder in college to complete classes.*
>
> **– Patient with SCD**

> *[I am not] able to fully be a parent to my daughter. I'm always absent due to being hospitalized all the time. I miss plenty of first moments with her because I'm sick and not around.*
>
> **– Patient with SCD**

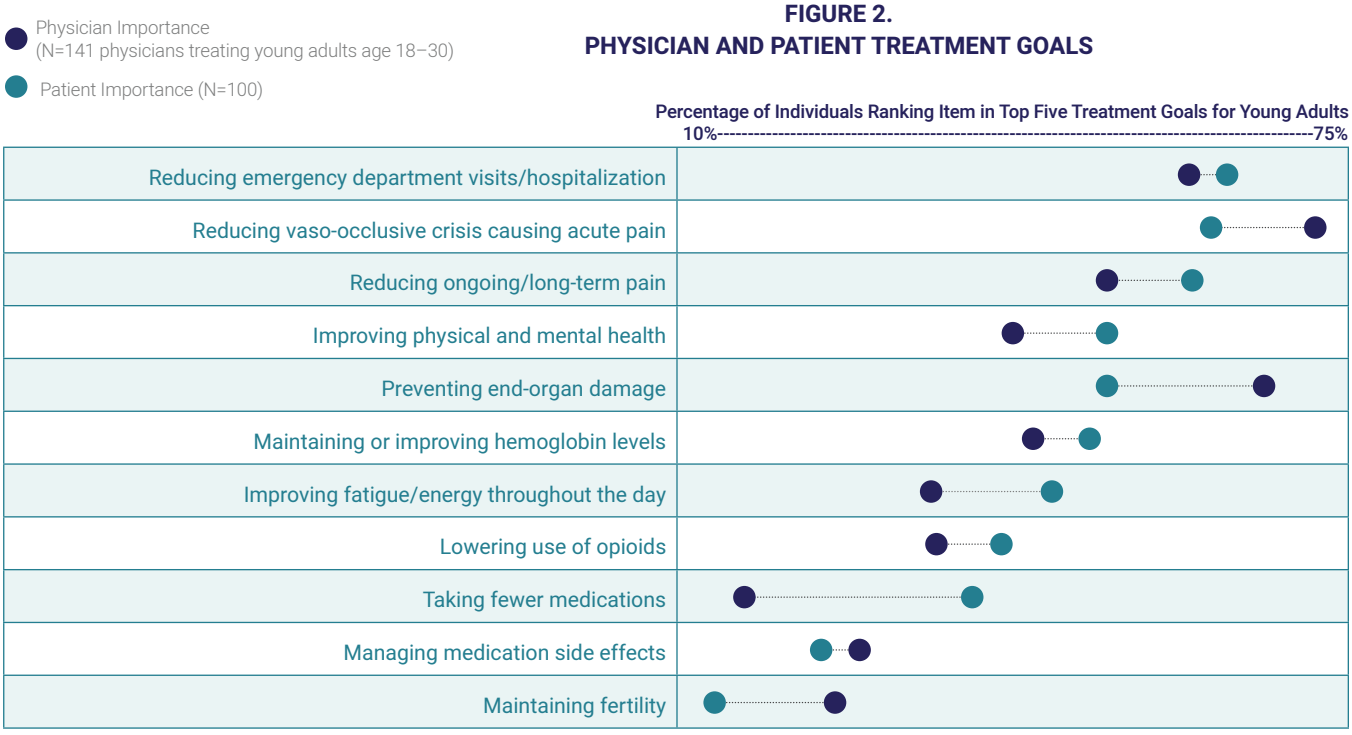
In addition to these challenges, patients need to navigate multiple treatment providers, sometimes with variable treatment goals, while often feeling as though they receive little support from the medical community and society in general. This highlights the importance of recognizing the goals and challenges a patient has, especially during transition, so that optimal support can be provided.

Key Treatment Goals: Patient and Physician Perspective

Identification and alignment of patient and physician treatment goals is a pivotal aspect of optimizing SCD care. **65% of surveyed physicians agree the shift in treatment goals (discussed below) between pediatric and adult practitioners contributes to patient anxiety and uncertainty**.

There is concordance of many treatment goals between young adult patients and their physicians. Amongst both

FIGURE 2.
PHYSICIAN AND PATIENT TREATMENT GOALS



50% OR MORE PHYSICIANS AND PATIENTS agree the following are within their **TOP FIVE** treatment goals:

- Reducing emergency department visits/hospitalization
- Reducing vaso-occlusive crises
- Reducing chronic pain
- Preventing end organ damage

physicians and patients, 50% or more agree that **reducing emergency department visits/hospitalization, vaso-occlusive crisis, chronic pain, and preventing end organ damage are within their top five treatment goals** (Figure 2).

Physicians, however, prioritized more clinically focused treatment goals when compared with patients such as reducing vaso-occlusive crises (75% vs 60%, respectively) and preventing end organ damage (67% vs 50%, respectively). These goals presented in Figure 2 were also relatively consistent across physicians at comprehensive sickle cell centers (CSCCs) and non-CSCCs. The only exception was the ranking of preventing end organ damage (74% at CSCC, 58% at non-CSCC). "*CSCCs are more aware of the increased risk of early end organ damage in SCD,*" explains Dr. Osunkwo.

In contrast, "*patient goals are more holistic and quality of life focused,*" explains clinical psychologist Dr. Lori Crosby; "*at times, there may be differing perspectives with patients desiring improved quality of life and physicians focused on clinical improvements (e.g., prevention of end organ damage).*" Patients generally focused on treatment goals related to overall wellbeing. Surveyed patients' top priorities were reduced hospital visits, reduced pain, and improved physical and mental health. This misalignment between physicians and patients can be most clearly seen in their desire to reduce the medication burden, a top five treatment goal for 34% of surveyed patients compared with only 16% of physicians. This is the largest disconnect between patients and physicians. "*From the patient and caregiver perspective, it's completely reasonable to want to take fewer medications!*" explains Dr. Titilope Fasipe, co-director of the Sickle Cell & Thalassemia Program at Texas Children's Cancer & Hematology Center. "*Medications are at times necessary and beneficial, but they are also a reminder that something is wrong with you, which may affect quality of life, especially for a younger adult population. As for the physician view of this category, the lower ranking items are a reflection of how the medical community may not always appreciate the related burdens associated with receiving care.*"

Patient Satisfaction with Emergency Care

Patients were **most unsatisfied with emergency department physicians among their care team** (of the 88% of surveyed patients who reported ED physician involvement in their care, just 52% indicated satisfaction). These findings are similar to previously reported figures on the relative dissatisfaction by patients with SCD with care received in emergency departments[8]. As Dr. Crosby reasons, "*ED physicians may only see these patients one time, they may not know them, and they have limited time to work with them.*"

In addition to being unfamiliar with the patients with SCD that may present at the ED, many emergency physicians are also unfamiliar with treatment guidelines for SCD. In one survey of emergency department providers, only 32% were aware of National Heart, Lung, and Blood Institute (NHLBI) SCD guidelines[9]. Another survey showed that despite 54% of emergency department physicians feeling high comfort in treating vaso-occlusive crises (VOCs), fewer than 10% knew the recommended time from triage to first dose of treatment medication[10].

"*The ED is probably the most notorious place a patient with SCD will experience subpar care, racism, discrimination, and bias,*" explains Harris. "*Our pain is undervalued and undermined; we are seen as drug seekers, and mostly it feels like the provider seems to want us to get out ASAP so that they can attend to 'more important' patients.*" According to Dr. Nirmish Shah, director of the Sickle Cell Transition Program at Duke University, "*discrimination and stigma in adult emergency departments is made worse by the lack of understanding of SCD in many ED systems and also the progressive nature of SCD causing more complications/VOCs as patients become older.*"

There are, however, groups that have emerged and are actively working to alleviate issues. Supported by the American College of Emergency Physicians, the Emergency Department Sickle Cell Care Coalition (EDSC3) aims to provide a national forum dedicated to improving the emergency care of patients with SCD and offers a range of tools to emergency departments. Dr. Shah is also involved with efforts to develop useful SCD order sets (for use in common Electronic Medical Record platforms) that can be integrated into emergency department workflows around the country.

Challenges of Managing SCD in Young Adults

Patients and physicians identified many of the same key challenges with managing SCD in young adulthood, reporting that **suboptimal transition support, lack of psychological/mental health support, and limited information availability or understanding are in the top six challenges for both parties** (Figure 3).

FIGURE 3.
IDENTIFIED CHALLENGES RANKED BY PATIENTS AND PHYSICIANS

SCD Challenges Ranking by Patients (N=100)

1. Excessive time in hospitals (e.g., ED visits)
2. Infusion/transfusions centers are hard to access, making ED visits the next best option
3. Lack of support in transition from pediatric to adult care
4. Lack of psychological and mental health support
5. Limited information available to help understand management of SCD
6. High medication costs
7. Lack of coordination between different healthcare providers

Note: Patient ranking based on number of respondents who responded to items as very challenging (top2box)

SCD Challenges Ranking by Physicians (N=150)

1. Poor patient adherence
2. Suboptimal transition support
3. Lack of psychological and mental health support
4. Inadequate ED discharge plans
5. Suboptimal use of preventative care
6. Poor patient comprehension and retention of information discussed during office visits
7. Tracking patients across health systems is difficult

Challenges 6 and 7 were ranked equally.

Note: Physician ranking based on estimates from treating physicians on young adult (YA) patient percentage dealing with identified challenges

Interestingly, the greatest challenge surveyed physicians noted is poor patient adherence, stating that it prohibits adequate disease management for 38% of their patients. This sentiment was often alluded to by physicians when discussing challenges treating SCD patients. Despite a desire to adhere to therapy, Harris explains, "*social determinants of health prevent some patients from adhering, with barriers to care including inadequate housing, transportation, and insurance.*" Furthermore, Dr. Fasipe highlights that "*the top challenges that patients face are made more difficult by the fact that these challenges are all likely to be interpreted as poor patient adherence by providers*" (Figure 3). To better understand and support their patients, further education of providers is needed, Harris suggests, and "*providers need to work better with social workers and interdisciplinary teams that can help support the patient's social needs.*" There is additional difficulty in that not every provider has access to social workers for their patients.

TOP PATIENT CHALLENGES
reported by both patients and physicians include:
- suboptimal transition support
- lack of psychological/mental health support
- limited information availability/retention

Regarding transition support, patients were often vocal about their lack of support during this time, explicitly verbalizing the following:

> ***You have to do it yourself****—even if you asked the doctor or the nurse for help, they will only put in 1% of the work, but you'll have put in all the work and it's stressful when you have no help at all.*
>
> **– Patient with SCD**

> *...In the early 2000s there was no such thing as transition care or plan.* ***We were just thrown to the wolves****, and I almost lost my life because of it.*
>
> **– Patient with SCD**

According to physicians, suboptimal transition support is estimated to prevent adequate care for 36% of patients. However, pediatric and adult practitioners differ in their estimations of patients challenged by suboptimal transition support. Pediatric physicians estimate suboptimal transition support prevents adequate care in more than half of the young adult patient population (53%) while adult physicians estimate it prevents adequate care in only 31% of young adult patients. Additionally, these estimations differ by physicians at CSCCs vs non-CSCCs (44% vs 32%, respectively).

Considering the lack of psychological and psychosocial support, one patient expressed the deep emotional impact of SCD, stating the following:

> *I sometimes feel like a burden because of the fact that I have sickle cell, and that my family has had to witness these moments which, honestly, I hate myself for. I hate that I am the only person in my family who is like this, and I feel defective.*
>
> **– Patient with SCD**

"*Psychological challenges are a significant issue during transition,*" explains Dr. Shah. A range of social and psychosocial factors are present[11], many of which may not be fully understood by physicians. "*Physicians have not been extensively trained on the impact of the disease itself, let alone all the psychosocial dynamics that are involved, unless there has been a dedicated adult program established with a behavioral health team,*" explains SCD program manager and Virginia Commonwealth University social worker Shirley Johnson.

When looking holistically across all identified challenges, physician and patient challenges ranked similarly across CSCC and non-CSCCs. However, patients treated at non-CSCCs found all surveyed items to be more challenging than patients treated at CSCCs.

62% of patients characterized their overall transition experience as **"moderately challenging"** or **"very challenging"**.

Transition in Sickle Cell Disease

Transition from pediatric to adult care is a critical step that presents specific challenges for young adults living with SCD. Although there has been an increased focus on improving the transition experience for SCD patients in recent years[12], significant hardship persists. In this survey, **62% of patients characterized their overall transition experience as "moderately challenging" or "very challenging"**. As Johnson explains, "*Transition needs to continue to focus on the psychosocial issues facing this population, education on the disease as they become adults, and the resources in the adult medical community needed to continue to support this population… If resources, provider training, and support of the pediatric community can be expanded, more positive outcomes are possible.*"

Timing of Transition from Pediatric to Adult Care

As patients with SCD approach adulthood, conversations are initiated prior to transition to discuss the process of transferring to adult providers and clinics. In this survey, **both patients and providers report these conversations begin on average around age 17**. This timing is problematic, explains Dr. Fasipe. "*Age 17 is too late to begin education on transition. Transition readiness conversations need to start around age 12 and gradually build with age. Ultimately, what often occurs with sickle cell disease is that transition readiness does not usually correlate with transition/transfer date." "Generally, the age transition occurs will vary depending on the availability of an adult SCD provider to transfer to*," explains Dr. Shah. "*Programs without an adult provider will typically hold on to patients longer.*" Surveyed physicians estimate an approximate 5-month gap between last pediatric and first adult visit, with some patients taking up to 3 years to fully establish care in the adult setting. Furthermore, physicians estimate that as many as **23% of their patients become "lost during transition"** and may not be seen by an adult hematologist or primary care provider for over two years. However, some experts, including Dr. Osunkwo, believe the true number of patients failing to transition successfully is higher, and there is definite room for improvement across the board. Fortunately, improvements are possible at the institutional level with appropriate dedicated resources and staffing: "*in our program we went from a 50% transition rate to 92% successful transition to adult care when resources were added to the adult program*," says Johnson.

TRANSITION CONVERSATIONS BEGIN AROUND AGE 17 on average, well beyond the Got Transition® recommended **AGE OF 12**.

Adherence to Transition Guidelines and Quality Metrics

In recent years, development and adoption of transition guidelines are becoming an emerging focus for SCD care[2]. When transitioning patients to adult care, surveyed physicians overwhelmingly agree (73%) there is a lack of guidelines and protocols implemented at their practices and institutions. Just 38% of surveyed physicians report incorporation of either a standard guideline or toolkit for SCD management and transition (e.g., ASH Transition Readiness Toolkit or NHLBI SCD Guidelines). "*Although tools are available, there is lack of awareness, and the tools that are available can be too broad or general*" explains Dr. Shah. Additionally, "*the underutilization of guidelines may be related to the challenge of integrating these guidelines into practice*," explains Dr. Crosby, with Harris adding, "*ideally there should be a rubric that is implemented for every patient in every CSCC.*"

Of particular note is the disparity in responses between physicians practicing in CSCCs and physicians practicing in other settings (non-CSCC). **48% of physicians in CSCCs report their institution has transition guidelines. Additionally, CSCCs are twice as likely to track quality metrics than non-CSCCs** (Figure 4).

73% of physicians agree there is a **LACK OF TRANSITION GUIDELINES AND PROTOCOLS** at their institution or practice to aid their approach.

Incorporating transition care quality metrics into institution policies can also help increase quality of care. "*It's important to have transition guidelines/policies, as well as metrics, although, as seen here, most programs are still working on having even basic metrics for SCD*," explains Dr. Shah. In this survey, just one third of physicians report their institution has quality metrics for SCD management. Use of guidelines and tracking quality metrics is generally higher in many other disease areas, such as oncology and cardiovascular health, where NCCN guidelines are widely used[13] and Centers for Medicare and Medicaid Services quality metrics such as the hospital inpatient quality reporting program are applied, impacting reimbursement rates[14]. When SCD quality metrics are tracked, they tend to include number/length of hospital and/or ED admissions, number of transfusions, number of VOCs per year, quality of life, and pain episode rates. The most reported transition-specific metrics include written transfer summaries being sent to adult providers, patients keeping clinic appointments, counseling about transition taking place prior to transfer, overall quality of life, and ongoing patient adherence to treatment and medications.

FIGURE 4.
PERCENTAGE OF PHYSICIANS INDICATING THEIR INSTITUTION UTILIZES QUALITY METRICS OR TRANSITION GUIDELINES

	Across All Center Types (N=150)	Comprehensive Sickle Cell Center (N=50)	Non-Comprehensive Sickle Cell Center (N=100)
% Physicians indicating institution has **SCD management quality metrics** in place	34%	58%	22%
% Physicians indicating institution has **transition guidelines**	27%	48%	16%
% Physicians indicating institution has **transition quality metrics** in place	13%	22%	9%

Key Barriers to Effective Transition

Barriers patients face that impede successful transition include: difficulties finding adult providers, challenges accessing care ranging from limited insurance coverage to transportation access, and needing to adapt to a dramatically different healthcare landscape from their familiar pediatric settings of care. **Just 37% of surveyed physicians (42% of surveyed adult hematologists and 19% of surveyed pediatric hematologists) believe that young adults are well-prepared to manage their disease through transition**.

SCD QUALITY METRICS
tracked tended to include:
- Number/length of hospital and/or ED admissions
- Number of transfusions
- Number of VOCs per year
- Quality of life
- Pain episode rates

37% of physicians (42% of adult hematologists and 19% of pediatric hematologists) **BELIEVE** young adults are well-prepared to manage their disease through transition.

Finding an available adult SCD provider can be a serious challenge for patients, although pediatric and adult practitioners differ in their perceptions of this. 92% of pediatric physicians agree that the lack of adult hematologists who accept SCD patients is a major barrier to care compared with 60% of adult physicians. "*Many parts of the country do not have an adult provider who is equipped, trained, and specialized in handling young adults with sickle cell disease,*" says Johnson. "*This is ultimately a very big deal,*" explains Dr. Shah, "*since there is no immediate answer on how to fix it… There have been efforts by ASH and other programs aiming to increase the number of adult SCD providers through training, but the problem remains.*" Dr. Osunkwo suggests an option is to "*identify and train advanced practice providers and primary care providers to fill this gap by working with SCD specialists as mentors.*"

> ***Finding a great doctor to work with is hard****... not many specialists focus on sickle cell disease. They know about the illness, but do not truly understand the patient experience.*
>
> **– Patient with SCD**

78% of patients report that **DISCRIMINATION & STIGMA** prevent them from receiving the SCD care they need.

Even when young adults can find available SCD treaters, accessing care can be a challenge. 53% of surveyed patients report challenges with getting to appointments and 29% report it takes over an hour to travel to their clinic. Additionally, 68% of physicians agree that lack of adequate insurance coverage creates a major barrier for transitioning patients.

Crucially, 78% of surveyed patients report discrimination and stigma prevent them from receiving the SCD care they need.

When asked about the most critical challenges in transitioning from pediatric care to adult care, patients spoke strongly of these barriers and the culture shock of transitioning into an adult care setting. One patient shared the following:

> ***You go from seeing a pediatric hematologist who knows everything to seeing a general physician who knows nothing or very little about the disease****... I loved my pediatrician. I knew she knew her job. These clinical [adult] nurses don't know much [about SCD] and look at us as drug seekers.*
>
> **– Patient with SCD**

Several patients commented specifically on the perception that they are viewed as drug seekers when they seek acute care, sharing the following:

> *You go from getting **EVERYTHING you need (meds, opioid, oxygen, nice nurses) to being a threat** and [getting] less narcotics than you would have if you were a toddler screaming in pain. It just doesn't make sense. **How can someone go from 21 to 22 and just not get the same care plan any longer?** Fighting for your rights? It's quite sad honestly. **It's like the opioid stigma has people thinking sickle cell patients are only coming to the ER for a quick high**, when in fact we only want pain relief. No one is up at 3am like 'oh, let me go wait 4 hours in a waiting room for 1 mg of Dilaudid.'*
>
> **– Patient with SCD**

"*It is well-known there is bias and stigma towards patients with SCD, and there are also many providers stepping up to try and improve the medical care of patients,*" explains Johnson. "*It's important to continue to encourage institutions to be involved in health disparities initiatives and work on inclusivity of sickle cell in these programs.*"

Supporting a Successful Transition

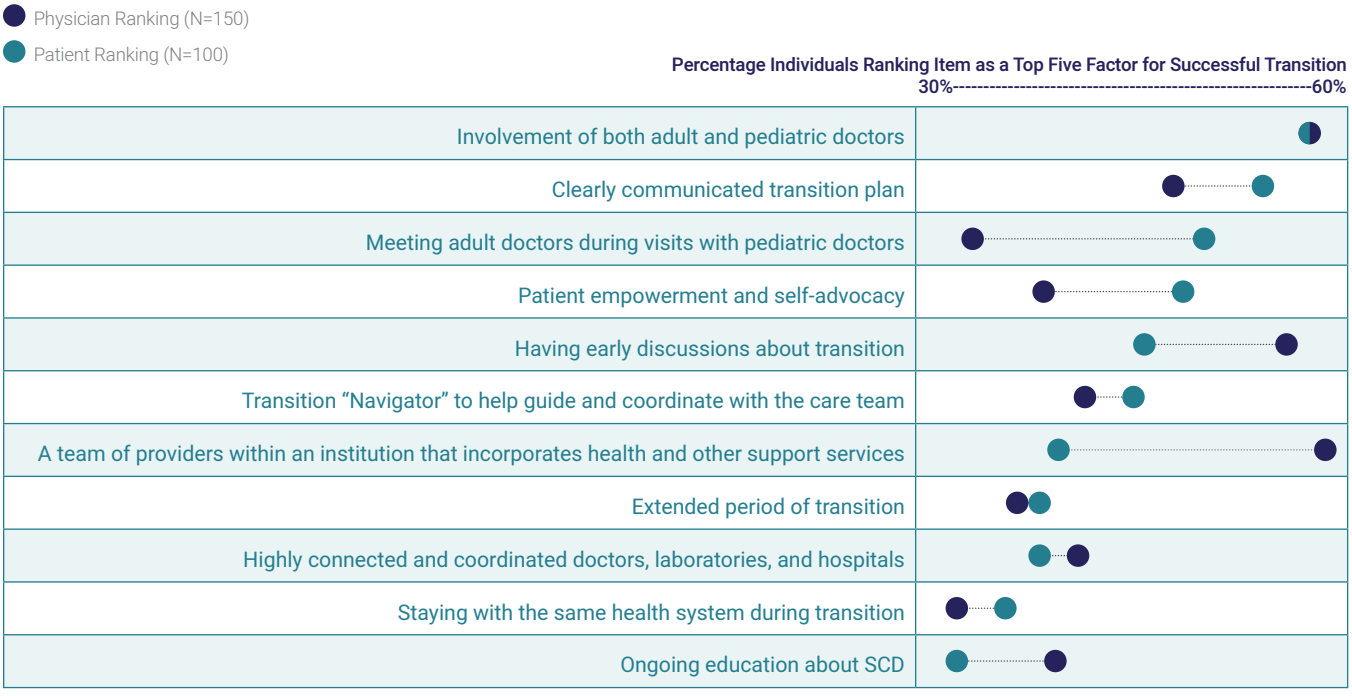
It is important to recognize the specific priorities patients and providers have for transition and find the middle ground that will make transition from pediatric to adult care easier and more successful.

Surveyed patients overwhelmingly agree with definitions of successful transition that involve *being able to engage and navigate their SCD care independently* (84%); *feeling consistently supported by their providers, families, and communities* (77%); and *having their symptoms well managed* (75%). When asked to then rank the importance of factors that contribute to a successful transition, **patients often ranked *both adult and pediatric doctors being involved* (57%) and/or *meeting adult doctors during visits with pediatric doctors* (51%) in their top five solutions**. "*We all need to learn how to accept people in terms of what they value as important, as different from what we think is important,*" says Dr. Osunkwo. "*There's a difference in how you do pediatric care, which is more paternalistic, and adult care and we've got to figure out a way to find that nice middle ground as providers.*" One patient shared the following:

> *"I think that when doctors are transitioning patients there should be a meeting with the patient, pediatric doctor, and adult care doctors. I also think that during this meeting they should come up with a healthcare plan instead of the adult care doctors just guessing in the beginning of the transition.*
>
> **– Patient with SCD**

"*Our most successful strategy over the past decade to make transitions smoother has been the inclusivity of the adult team with the pediatric team. The patients knew who they were going to see, what the clinic space looked like and who*

FIGURE 5.
FACTORS THAT MAKE FOR A SUCCESSFUL TRANSITION AS RANKED BY PATIENTS AND PHYSICIANS



they can contact," says Johnson, whose transition strategy mirrors that of the "warm hand off" style that has been shown to help ensure continuity of care and avoid potential declines associated with inconsistent medical care[15].

With respect to continuity of care in transition, 81% of providers consider *seeing an adult provider within 6 months of the patient's last pediatric visit* a sign of a successful transition, with 77% of providers viewing *3 years of consistent care with an adult SCD care provider* as a good metric of success. "*With adequate program resources for the adult providers, this is a reachable and sustainable outcome*," says Johnson, but "*adult physicians are not currently equipped to deal with all of these problems and still provide medical care to a large population.*"

A majority of physicians believe the following are metrics of a successful transition:

- **6 MONTHS** between pediatric and adult visits
- **3 YEARS** of consistent care

Support Systems for Patients in Transition

Young adults with SCD rely on a range of resources for support and information when transitioning to adult care. Throughout the process, a patient's family and community can provide an important support network. However, in this survey, we found patients and physicians differ in their perceptions of the strength of available support systems. While 78% of patients feel they have sufficient support from family/caregivers to manage their SCD, only 48% of physicians agree. "*Social determinants of health greatly impact the support that patients receive*," explains Dr. Crosby. "*Patients may see peers as supports, when in actuality they are not necessarily the type of support a patient may need in a crisis and for continuity of care*," adds Johnson. "*Support can come in all shapes and sizes, but having a dedicated, knowledgeable, and well-equipped support system can really benefit the patient.*"

Access to quality information is critical for young adults with SCD. Although patients vary in their preferred sources of information, patients report relying most on their SCD providers and social media (Figure 6). Facebook groups emerged as particularly popular sources of both community support and information. "*Social media groups can be risky due to the overwhelming dissemination of unsolicited medical advice*" explains Harris, with Johnson echoing that it "*can also lead to a great deal of false information and maybe prevent someone seeking care, taking medications, etc.*" However, social media also provides "*a positive way to set up peer-to-peer groups and circulate information*," explains Johnson, with Harris also mentioning that "*there are legitimate advocates that use social media to engage the community and it is a wonderful way to keep up with opportunities and events in the community.*"

Transition coordinators at clinical sites have also been shown to be positive support for patients in transition[3]. Unfortunately, most institutions do not have anyone in this role due to resource constraints. Of surveyed physicians, **26% of those in CSSCs have a transition coordinator compared with only 7% at non-CSCCs**. Introducing dedicated SCD transition coordinators is a promising way to improve quality of care. "*The single most important factor from the medical side that helps transition is having a dedicated person to coordinate and connect with the patient, and serve as a liaison between the patient, community, and providers, followed closely by strong social connections and support*," explains Dr. Osunkwo.

FIGURE 6. PATIENT SOURCES OF SCD INFORMATION (N=100)

PHYSICIAN (63%)

ONLINE SOURCES (49%)

NURSE PRACTITIONER/ PHYSICIAN ASSISTANT (43%)

SOCIAL MEDIA (43%)

The Way Forward

Improving outcomes for young adults with SCD will require continued proactive attention by stakeholders and the development of solutions that meet the specific needs of the community. By involving patients in the process, collaborating with community-based organizations, and pushing for effective policy changes, meaningful advancements can be made. Advancements may range from "*disseminating effective transformation strategies that engage the community-based organizations actively both during transition and after transfer to adult care*," as Dr. Osunkwo explains, to "*innovative technologies that aid in increasing patient engagement in healthcare, optimize adherence, enhance self-management, and effectively treat pain and mental health symptoms*," according to Dr. Crosby.

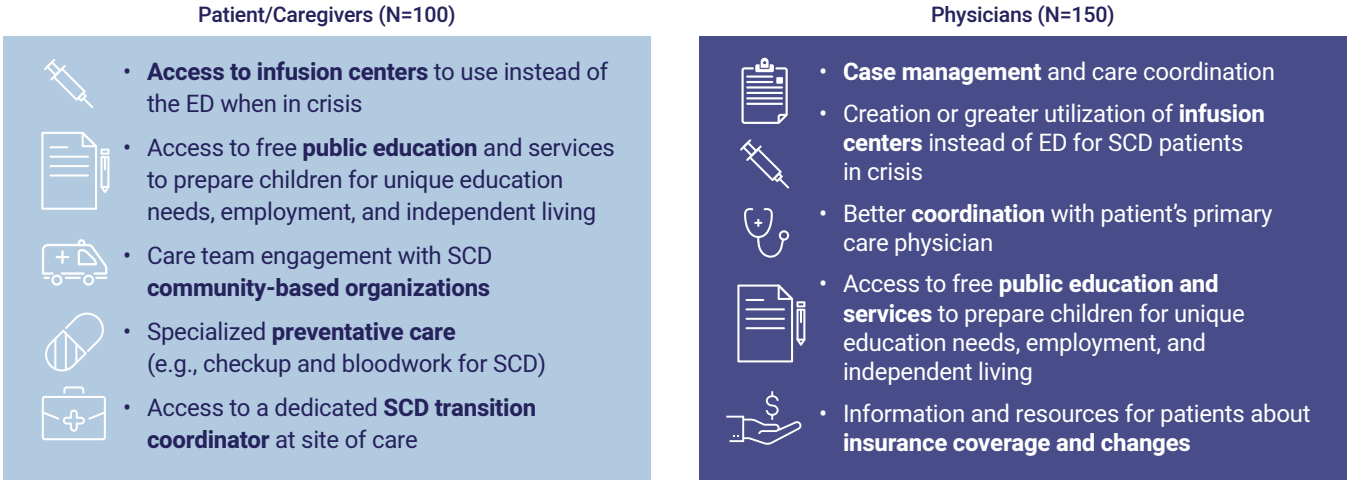
Co-creating Patient-centric Solutions

When considering the potential solutions to improve management of SCD, patients and physicians generally align on their ideas and preferences. Proposed solutions are consistent with the top challenges faced by patients, revolving around excess time in hospitals, lack of education, and lack of transition and mental support.

"*It's clear that having an infusion center helps, but these are usually only available in CSCCs*[16]" explains Dr. Shah, a point emphasized by 89% of surveyed patients and 73% of surveyed physicians who felt the most helpful solution to SCD management would be access to infusion centers instead of the ED for vaso-occlusive crises. Dr. Shah continues: "*education, I believe, is under-appreciated in the context of preparing patients and families for the adult world and fostering independence/patient advocacy.*" This is a sentiment that is shared amongst most surveyed patients and physicians with **88% of patients and 65% of physicians indicating they would find increased education and services to better prepare SCD patients for transition helpful**. Dr. Osunkwo agrees: "*universal access to infusion centers may not be pragmatic based on the geography of the patients, but universal education and awareness of disease modifying therapy, self-management, and advocacy to access what you need no matter where you are is critical.*" Patients (79%) and physicians (74%) also both believe *increased access to case management and care coordination services* would be helpful (Figure 7).

While the solutions posed by patients and physicians can all serve to improve the management of SCD, identifying and reconciling differing perspectives of physicians and patients can narrow the gap and allow for joint stakeholder collaboration and alignment on strategies to optimize the transition process. Several research programs are underway to assess the true efficacy of various potential solutions when implemented, including the ST3P-UP study (NCT03593395) led by Dr. Osunkwo and the ENGAGE-HU study (NCT03442114) led by Dr. Crosby, both supported by the Patient-Centered Outcomes Research Institute.

FIGURE 7.
TOP 5 RATED SOLUTIONS TO IMPROVE CARE FOR INDIVIDUALS LIVING WITH SCD DURING TRANSITION



Importance of Patient Advocacy and Community Engagement

Many surveyed patients mention their local community-based organization (CBO) as a resource for information and guidance, highlighting the critical role these organizations play in SCD care. Out of all patients, **85% believe that it would be "helpful" or "very helpful" for their care team to connect with community-based SCD organizations**. Surveyed physicians similarly agree to the importance of CBOs, with 63% indicating that patients connecting with community-based organizations/advocacy organizations would be "helpful" or "very helpful."

85% of patients **BELIEVE** it would be "helpful" or "very helpful" for their care team to connect with community-based SCD organizations.

> *[My support group] helps me gain a lot of knowledge and ask questions for everyone around the world to answer. I'll rather go to the support group before I ask anyone a question about SCD.*
>
> **– Patient with SCD**

"*CBOs are key to the ongoing support for patient care, but they need to be well-established and available*," Johnson explains. Dr Osunkwo adds that "*CBOs must work collaboratively with the medical teams to ensure that both groups are working synergistically rather than in parallel*."

Call to Action for Policy Change

Reducing the challenges faced by patients with SCD will require policy change, as policies can serve to support the broader SCD community, healthcare services, and social programs[17].

One such policy change is discussed concretely by the National Academies of Sciences, Engineering, and Medicine (NASEM)[2]. Children with SCD have unique learning needs and require additional support to better prepare for future employment and living independently. Qualifying SCD for early intervention (Part C of the Individuals with Disabilities Education Act) under the "at-risk" criteria will enable children with SCD to gain access to special education schools that can address these unique needs. Across surveyed respondents, 88% of patients and 69% of physicians believe this would provide meaningful help in easing transition. This would additionally help mitigate some of the social disparities that drive the educational gap challenges patients and physicians have identified. "*It is my belief that every patient with SCD should be eligible for social security disability benefits, and subsequent Medicaid and Medicare coverage*," adds Harris.

Additional initiatives such as the 2018 Sickle Cell Disease And Other Heritable Blood Disorders Research, Surveillance, Prevention, And Treatment Act serve to codify an expansion of the Centers for Disease Control and Prevention's (CDC's) Sickle Cell Data Collection (SCDC) program. This would provide data needed to help healthcare providers understand how patients interact with the healthcare system through transition, as well as know where to target activities and programs that will result in improvement in health care, including better access to SCD clinical centers, an issue often raised by patients[18].

Policy initiatives at the institutional and state level can support patients in transition, as in California, where providers used CDC data to gain $15 million in funding from the state to establish new sickle cell centers[6]. Even in the absence of policy changes, individual practitioners and community members have successfully championed changes at the institutional level, and as more individuals do so, institutions may see improved care for many patients. As Johnson explains, "*Over two years ago, we located champions in the adult ED willing to work with our team in improving care for SCD patients. They were well aware that there are many issues to this patient group, but reported that the SCD providers were the experts. We shared numerous medical guidelines from NHLBI and ASH with them, began working on individualized treatment plans for those continuously in the ED for them to know how to treat them, and met monthly to discuss challenging patients and needs. In addition, our 24/7 clinical on call team would be available for any questions on patients reporting to the ED. Although there remain issues, especially during COVID, there has been an improvement in care for many patients.*"

Conclusion

The challenges faced by young adults with SCD are numerous and multifaceted. As such, successful transition from pediatric to adult care requires a collective and concerted effort in the face of numerous systemic barriers. No single solution has the potential to erase the barriers young adults with SCD face, and there is ongoing need for additional studies to examine how to best improve and deploy transition programs efficiently and effectively. In an ideal model, Dr. Osunkwo proposes, a child with SCD may start receiving transition education at age 12 from their medical team, be assigned a transition coordinator, and be plugged into a CBO to learn life skills and about the differences they will experience in adult medicine compared to their pediatric experience, and also to equip them with the tools to manage these changes. As a teenager, the patient may meet with their pediatric and adult provider team together, tour the adult facility, engage in dialogue with their pediatric and adult providers about living with SCD as an adult, and have a neuropsychology evaluation to determine any cognitive gaps. With sufficient preparation, the transfer event can kick off a successful care experience in the adult environment, with ongoing reinforcement from the transition coordinator for an additional 2–3 years.

Although developing these new models of transition in SCD is an uphill battle in the present-day framework of care, meaningful change is possible when engaged communities of patients, caregivers, providers, and advocates come together to develop innovative solutions and push for change. Multiple useful frameworks have been developed that highlight some of the multidisciplinary strategies needed to support "long, healthy, productive lives for those living with SCD," including reports from NASEM[2] and Sick Cells/Avalere Health[19]. Our findings are largely aligned to the recommendations of these road maps: according to Dr. Crosby, developing ideal transition and care will require:

- *"multidisciplinary teams*
- *targeted education efforts*
- *building a pipeline of prepared providers*
- *training providers to dismantle racist and biased policies and practices*
- *programs to help individuals manage SCD and advocate for evidence-based care*
- *flexibility in care models (e.g., mix of telehealth and in-person visits)*
- *holistic care approach that considers and attends to the social determinants of health and mental health"*

The challenge may be daunting, but there are many who have the determination and the tenacity to improve SCD care, either for themselves or for others, and meaningful change may occur as the experts who have made the commitments to serving their patients can bridge to the community and enact change together. Everyone involved in SCD care can play an important role in improving outcomes for all individuals living with SCD.

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