Ensuring Access to Gene Therapies to Address Sickle Cell Disease in Medicaid

North Carolina Department of Health and Human Services

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Introduction

Sickle cell disease (SCD) is one of the most common genetic diseases, affecting an estimated 100,000 individuals in the United States.[1] SCD is a group of inherited red blood cell disorders that affect hemoglobin, the protein that carries oxygen through the body. The condition is marked by sickle-, or "c-," shaped red blood cells that cause blockages in an individual's blood flow, preventing oxygen from getting to the vital organs and tissues in the body, which can lead to chronic anemia, debilitating pain, infections, strokes, and organ failure.

Individuals with SCD have significantly higher mortality rates than the average population, with a projected life expectancy of only 54 years.[2] Further, mortality rates vary substantially by location; in one comparative study of four states, mortality rates from SCD ranged from three times to nine times higher than average, suggesting that local distribution of resources and quality of care may be affecting outcomes over and above the severity of disease itself.[3] These poor health outcomes are especially troubling given that SCD disproportionately affects individuals who are Black: between 2016 and 2018, 93% of individuals who were hospitalized for SCD were Black.[4] The costs and burdens of SCD are magnified because, due to the inherited nature of the disease, families who have one member with SCD are likely to have other affected family members as well.

Given the intolerable pain crises that are common to SCD, the disease can also have devastating effects on quality of life and emotional well-being.[5],[6] The effects of vaso-occlusive crises are all-encompassing and touch even the smallest aspects of a person's life: a North Carolina resident with SCD described having to distribute copies of his house keys to friends so that they could enter his house to assist him if he was immobilized due to pain.[7]

For many, SCD is a lifelong condition. There is currently only one curative treatment option for SCD – a bone marrow or stem cell transplant – but few individuals are eligible for it, and even fewer receive it.[8] There are multiple evidence-based ways to manage SCD, however, including lifestyle modifications, preventive care, and medication. Preventive care such as annual transcranial Doppler ultrasound (TCD) screening, a diagnostic test that uses soundwaves to detect blood flow to and within the brain, can identify children who might be at risk of stroke, while regular vaccines and penicillin use can reduce the risk of infections. Medications including hydroxyurea, an oral medication that must be taken once per day, can also reduce the frequency of pain crises for individuals with SCD. These evidence-based strategies can reduce SCD complications but can be intensive to maintain over a lifetime, and as with blood and bone marrow transplants, many individuals with SCD who could receive recommended care do not.[9],[10] In surveys of primary care physicians, a majority report lacking confidence to treat people with SCD, adding to the challenge of obtaining evidence-based treatment.[11]

While research funding for rare diseases has traditionally been sparse, researchers have identified disparities in how that limited funding is distributed.[12] For example, cystic fibrosis (CF) – a similarly devastating lifetime disease that affects approximately 40,000 individuals in the United States – received higher per-person federal and foundation funding between 2008 and 2018.[13] This difference is stark when considering how the demographics of CF differ from those of SCD, as roughly 91% of individuals who have CF are White.[14] The infrastructure for surveillance data for SCD – to better understand who has SCD, where care is received, and what the outcomes are – has likewise seen chronic underinvestment. The Centers for Disease Control and Prevention (CDC) has engaged in recent efforts to

address this issue via the development of the Sickle Cell Data Collection (SCDC) program, launched in 2015, though only 11 states are currently participating.

Despite historic underfunding in SCD research, exciting treatment developments are on the horizon. Two gene therapies that could be curative for SCD were approved by the U.S. Food and Drug Administration (FDA) on December 8, 2023. These therapies, and others in the pipeline, present an opportunity to address health disparities faced by individuals with SCD.

Key to ensuring gene therapies are available to all who want them, however, is ensuring state Medicaid programs can provide them. Medicaid is a dominant payor for individuals with SCD, and with prices as high as $1.8 million expected per treatment, gene therapies have the potential to strain state budgets.[15],[16],[17] This is especially true for states across the southeastern United States, where the SCD prevalence is highest and more individuals will likely be eligible for new therapies.[18] If this issue is not addressed, states will face painful choices between ensuring access to these treatments and other state budget items (including other investments in their Medicaid programs), and patients risk Medicaid programs potentially imposing official or de facto restrictions on access to the therapies that are driven by the need to balance budgets rather than on evidence-based standards of care.

The federal government will need to partner with states to ensure access to these potentially life-changing therapies. To that end, this report provides a brief overview of the implications for Medicaid of the upcoming gene therapies, steps many states are taking now to plan for the therapies, and options for additional federal and state collaboration. In January 2024, the Centers for Medicare & Medicaid Services (CMS) Innovation Center announced the Cell and Gene Therapy Model (CGT) Access Model. This model will allow states and drug manufacturers to enter into outcomes-based agreements negotiated by CMS for lower gene therapy costs beginning as early as January 2025.

Recognizing that not all individuals with SCD will be eligible for next-generation therapies or may choose not to pursue them (for numerous reasons), Appendix A also details many strategies states are taking to expand access to standard treatments for SCD as well as challenges they grapple with to improve care for this historically marginalized population.

Upcoming Gene Therapies

Emerging gene therapies are intended to be effectively curative – that is, to address the underlying disease process that causes people with the sickle cell mutation to make hemoglobin S, the hemoglobin variant that causes red blood cells to sickle under stress. Bone marrow or stem cell transplant, the currently available curative treatment for SCD, requires a donor and carries the significant risk of graft versus host disease (GvHD), a life-threatening complication and leading cause of death for bone marrow transplant patients, whereas gene therapy can be performed using the patient's own cells.[19]

Two gene therapies were approved by the FDA on December 8, 2023, with others moving into clinical trials.

Bluebird Bio, Inc. (gene-replacement therapy lovo-cel): Eligible patients for the clinical trials were 12-50 years old, with severe SCD (later definition of "severe" meant four VOEs in two years prior to enrolling). Other trials include younger individuals.

Vertex Pharmaceuticals and CRISPR Therapeutic (gene-editing-based exa-cel): Early studies included individuals age 12-35.

In addition to financing concerns, Medicaid programs will encounter an urgent need to develop policies and expedite access to reduce the risk that eligible patients will age out of the initially approved treatment parameters for these potentially curative breakthroughs.

Two therapies – Bluebird Bio's lovotibeglogene autotemcel (or "lovo-cel") and CRISPR Therapeutic/Vertex Pharmaceuticals' exagamglogene autotemcel (or "exa-cel")[20] – were approved by the FDA on December 8, 2023, and others are in the pipeline.[21] Clinical trial findings indicate that these therapies can reduce or completely free individuals from vaso-occlusive pain events (VOEs), also sometimes called "sickle cell crises" – the pain crises that are a marker of SCD – in the two years following treatment.[22]

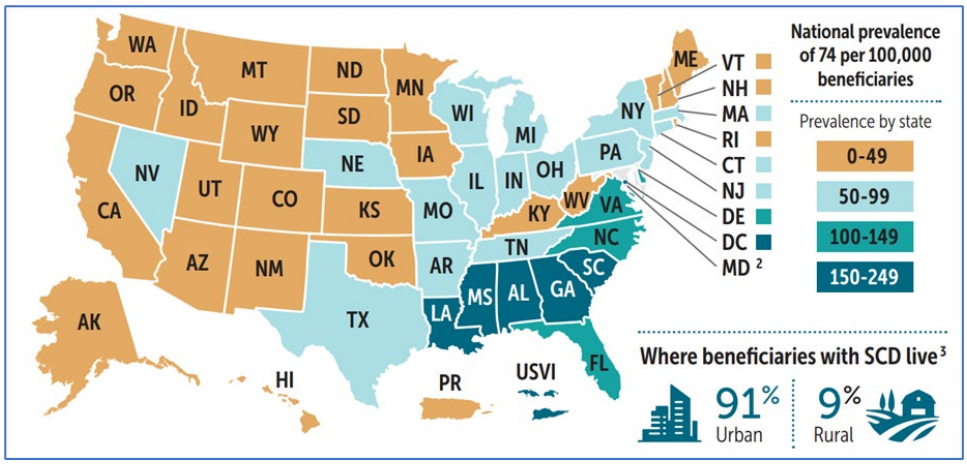
These therapies are not without their own risks and costs, however. Treatment begins with apheresis, during which cells are extracted from the patient's body and sent to the therapy manufacturer. Patients then undergo bone marrow ablation, a lengthy procedure that can require a month or more of hospitalization. During this period, patients' immune systems are suppressed, increasing the risk of common infections. In the days before the return of the patient's own modified cells, patients may face serious complications or even, in rare cases, death. In addition, patients during this time must remain in

a hospital; the vast majority, if not all, are unable to work or meet their usual household and family responsibilities. For many people with SCD, this could be an insurmountable barrier to gene therapy if appropriate supports are not built into how insurers, including Medicaid, cover the therapies.

Medicaid's Role in SCD and State Planning for Gene Therapies

While the lack of surveillance data makes it difficult to understand the true SCD population in the United States, Medicaid is a dominant payor for SCD care. Between 1999 and 2007, Medicaid covered 58% of SCD-related emergency department (ED) visits and 66% of hospitalizations.[23],[24] Similar findings have emerged from states participating in the SCDC program; in North Carolina, it is estimated that more than 5,000 residents suffer from SCD, and North Carolina Medicaid covered 48% of hospital admissions and ED visits in 2017 for SCD patients.[25] Further, individuals with SCD are geographically concentrated in the southeastern United States. In 2017, six of the seven states with the highest SCD prevalence rate among Medicaid beneficiaries were located in the southeastern United States (*Figure 1*).[26]

Figure 1. **Geographic Variation in the Number of Medicaid and CHIP Beneficiaries with SCD Per 100,000 Beneficiaries in 2017**[27]



Improving access to care and outcomes for individuals with SCD who have Medicaid coverage is challenging across the country, but these challenges are magnified in the Southeast given the higher prevalence of SCD. Medicaid programs must navigate long-standing shortages in specialists able to treat SCD, which can contribute to delays in receiving guideline-based care or people not receiving care at all.[28] For example, one of the best ways to prevent SCD pain crises is through regular hydroxyurea use, but only about one-third of children and adults on Medicaid had any days of hydroxyurea use in 2017.[29] Suboptimal use of preventive care can lead to pain crises that force individuals to seek care in the ED, where individuals with SCD experience longer wait times than other patient populations.[30] Further, states in the Southeast have, with a few notable exceptions, been less likely to expand their Medicaid programs, which has created challenges in ensuring continuity of care and improving long-term health outcomes as individuals with SCD age out of childhood Medicaid eligibility.

With the approval of exa-cel and lovo-cel, Medicaid programs in the Southeast face a new challenge. Medicaid programs are required to cover all drugs that are part of the Medicaid Drug Rebate Program (MDRP) – a federal program that provides state Medicaid programs with lower-cost access to drugs whose manufacturers have agreed to certain terms in exchange for coverage – and gene therapies are expected to be part of the MDRP.[31] Medicaid programs will be tasked with creating meaningful access

to gene therapies while seeking to avoid cuts in other program areas due to the high price tag of the therapies and ancillary services.

State officials across the Southeast are facing this challenge head-on and have begun planning to address the economic, medical, and social issues that will affect Medicaid member access to gene therapies. For some states, planning efforts have involved meeting with manufacturers to discuss potential pricing and reimbursement arrangements, with some considering value-based arrangements that condition payment on achievement of certain outcomes, such as avoidance of painful VOEs. Others are considering splitting reimbursement for the therapies themselves from reimbursement for associated hospital services, with a goal of eliminating potential financial risk to hospitals that is associated with procuring the therapy. States are also strengthening their data analysis and surveillance efforts to understand how many individuals may be eligible for and interested in gene therapies.

In addition, states can develop funding options for other supports that will need to be in place in order for individuals to access therapies (e.g., transportation, child care). States can draw on experience supporting patients through bone marrow transplant for other conditions and are expecting to provide support for family members' housing, food, and other needs during what they expect would be an extended period of hospitalization, followed by frequent checkups that would require remaining near a designated center. States that have embarked on this work note that ensuring a broad geographic distribution of centers qualified to provide gene therapy, rather than limiting its use to one or two locations in major urban centers, would be an important step to ensuring equitable access. However, ensuring broad geographic access will be challenging given the complexity of the therapies and the skills and capacities required to deliver them, underscoring the importance of providing a robust set of supports like transportation and housing.

Individuals may need ancillary services, such as fertility preservation (see "*Covering Fertility Preservation*"), which do not need to be linked to the gene therapy provider but should be accessible to those seeking care. People who have undergone gene therapy may also have unique behavioral health needs arise as they seek to navigate their very different lives. Once enough people have undergone gene therapy, states could consider developing peer navigator programs to support new patients as they go through the process.

Covering Fertility Preservation

Exa-cel and lovo-cel have been approved for use in individuals as young as age 12, raising a critical question for adolescents and their families: are they willing to undergo gene therapy if it affects their fertility?

The long-term effects of gene therapy on fertility are not yet known, but infertility is a common side effect of the chemotherapies that are part of the gene therapy regimen.[32] Including a fertility preservation benefit can play an important role in addressing enrollee concerns about treatment.

Black women with SCD have an elevated risk of severe maternal morbidity, and the burden of SCD-related risk is so high that one study found it accounts for 8.9% of the Black-White health disparity in severe maternal morbidity.[33] Covering fertility preservation will allow enrollees access to lower-risk pregnancy and delivery, should they choose to pursue these goals.

Adding fertility preservation may address a key concern with obtaining gene therapy, but states will need to consider retaining any existing reproductive counseling or related benefits for individuals who undergo gene therapy, too. Exa-cel and lovo-cel do not modify the germ line cells, which means that affected individuals can still pass the sickle cell trait on to their children.

Even if Medicaid programs are able to address the social and medical needs that may arise during gene therapy, many individuals will be ineligible or will not want gene therapy for other reasons (e.g., fears about safety and long-term outcomes, questions about effects on fertility, financial and personal difficulties as a result of the treatment period duration, among others). Critically, although gene therapy may be a transformative advance in SCD care in many respects, it will not eliminate the need for robust networks delivering traditional SCD care and other preventive services.

Given this, state Medicaid agencies across the Southeast are concurrently taking steps to ensure more individuals with SCD are receiving evidence-based standards of care. The following section ("*North Carolina's Journey to Improving SCD Care for Medicaid Members*") and Appendix A describe selected state efforts to address SCD and promote access to treatment in greater detail.

North Carolina's Journey to Improving SCD Care for Medicaid Members

Following the release of a 2022 report from the Princeton School of Public and International Affairs, *Advancing Health Equity in North Carolina: Recommendations for Improving Sickle Cell Disease and Maternal Health Care Among Medicaid Enrollees*, North Carolina Medicaid (NC Medicaid) began a series of efforts focused on improving care for its members who have SCD.

To launch its efforts, NC Medicaid formed a multistakeholder workgroup comprised of state Medicaid staff, chief medical officers of the Medicaid managed care organizations (MCOs) in the state, SCD specialists, staff from the sickle cell treatment centers, and members of the academic research community. The workgroup created a space for NC Medicaid to seek input on opportunities for improving care for individuals with SCD.

Through discussions with the workgroup, NC Medicaid has enhanced its data analyses, including adopting new criteria for identifying individuals with SCD to align with the CDC case definition. NC Medicaid also analyzed claims data to assess performance on SCD metrics and found that TCD screening rates were low: in 2021, only 37.5% of Medicaid enrollees age 2-16 with SCD received a TCD screening. In reviewing this data with the workgroup, NC Medicaid has discussed deploying an initiative focused on increasing SCD screening by primary care providers.

NC Medicaid also held listening sessions with community partners. Through two listening sessions – one with 40+ individuals with SCD and their families, and one with health care providers across the state – NC Medicaid identified barriers families were facing to access care, such as denials of key medications and imaging. These concerns spurred NC Medicaid to conduct a review of its medication clinical criteria and utilization management tools used for SCD care, and NC Medicaid was able to confirm that prior authorization for TCD and SCD therapies was either removed or not in place for SCD patients. NC Medicaid also updated its lock-in policy for opioids to exclude individuals living with SCD, to ensure they could access key pain medications, and updated its Genetic Testing, Diagnosis and Treatment Clinical Policy to include red blood cell antigen genotyping in order to assess risk of severe reactions to blood transfusions.

In addition to in-state efforts, NC Medicaid has collaborated with Medicaid Chief Medical Officers across the Southeast to develop collective quality improvement efforts to address SCD, as well as to discuss planning efforts to address the impact of upcoming gene therapies for SCD.

North Carolina expanded Medicaid on December 1, 2023. Expansion represents a critical opportunity to increase access to care and could mitigate a key challenge for individuals with SCD: transitioning from pediatric to adult care. In interviews with other Southeast state Medicaid leaders, a lack of Medicaid coverage for childless adults was identified as a barrier to ensuring that individuals continue to receive care when they reach adulthood.

Urgent Need for State and Federal Partnership

States are working not only to provide potentially lifesaving therapy for enrollees with SCD but also to pilot processes that could deliver sustainable access to future innovative therapies as they are developed. While critical, state-led efforts will not be enough to ensure access to gene therapies for all who want them. States need federal help to navigate the upfront barriers to SCD treatments, including the price of the drugs and the need for other supports (e.g., housing, transportation) to make feasible the intensive process of receiving the therapies so that people with SCD and states can gain the long-term benefits of curative therapy.

Federal partners are in the process of developing options to support state Medicaid programs in providing gene therapies. The CMS Innovation Center Cell and Gene Therapy (CGT) Access Model would create a new financing approach for CMS to negotiate on behalf of state Medicaid agencies to purchase the therapies and promote innovative, outcomes-based payment approaches.[34] These negotiations could address not only price but also assurances that states would forgo adding restrictions in access beyond those imposed by the FDA, and that they would guarantee timely approvals or denials for the treatment.[35]

Federal legislation has also been proposed in multiple sessions that would mandate a demonstration including six states, which would include coverage for all SCD-related medical expenses, including drugs,[36] though the bill has not passed to date.

In addition to these steps, to ensure gene therapies are available to Medicaid members, the federal government could create a new drug benefit for cell and gene therapies that could affect a range of payment programs, or could increase Federal Medical Assistance Percentage (FMAP) coverage for cell and gene therapies in state Medicaid programs specifically. Similar to the way the federal Medicare program manages renal transplantation for beneficiaries with end-stage renal disease, states and the federal government could work together to certify centers qualified to deliver gene therapy, and define and price a bundle of services associated with delivery of the therapy.[37] The bundle could include not only immediate services like bone marrow ablation and related hospitalization but also behavioral health care (for a defined period of time around the treatment), fertility preservation and other related services. Congress could raise the FMAP for that bundle of services or cover it entirely using federal dollars. This payment model could be developed in parallel with and augment the effects of the state-federal price negotiation strategies CMMI has proposed.

States could also work toward innovative payment models in cooperation with the federal government. For example, states may benefit from an approach where they pay for an expected volume of gene therapy treatments, using an annuity payment approach where the state pays an average annual payment each year.[38] Annuity payments could smooth payment requirements generally, by lowering payments during the initial period when states have the largest number of eligible and interested members to bring into treatment, or could buy states additional budget stability for the critical period between release of the therapies and potential launch of a CMMI payment model.

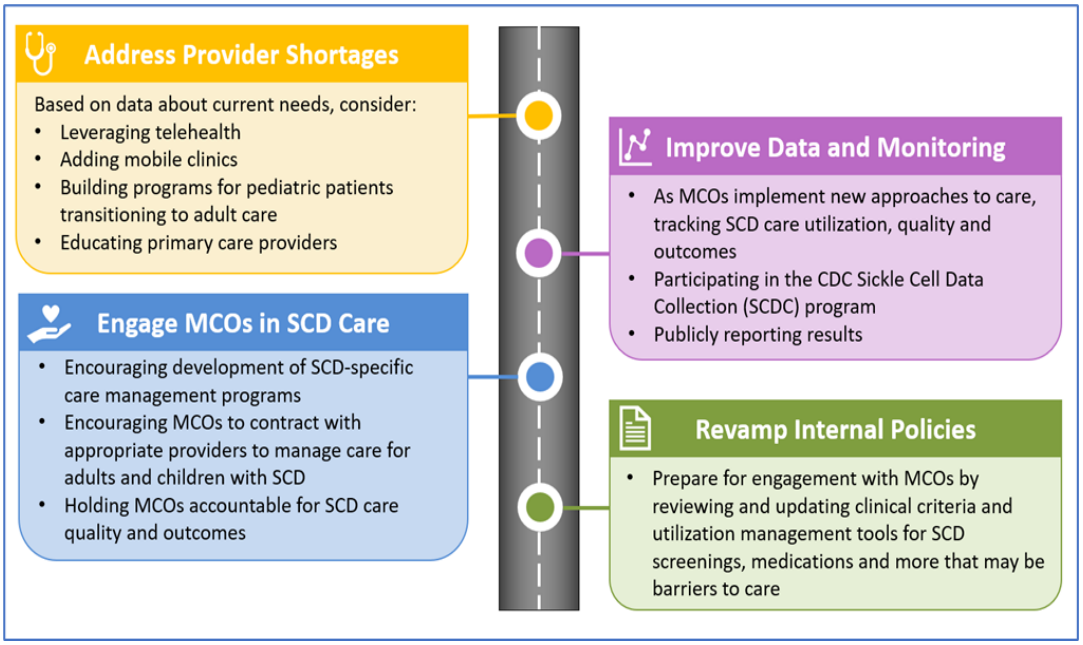
Conclusion

As states continue to confront health and broader societal disparities, improving outcomes for individuals with SCD is an important opportunity for improvement. State Medicaid programs are taking essential steps to improve care for individuals with SCD and planning for gene therapies, but the expected cost of these life-altering therapies warrants additional - and more immediate - federal support to ensure that they are accessible to all individuals who can benefit from them.

Appendix A: Improving Access to Evidence-Based Standards of Care for Individuals with SCD on Medicaid

In addition to planning efforts around gene therapies, Medicaid programs and SCD stakeholders across the Southeast are ramping up other efforts to improve care for individuals with SCD, with a goal of improving health outcomes and rates of evidence-based SCD care delivery. From June to August 2023, Medicaid officials and SCD stakeholders in five Southeast states shared the improvements they have implemented in recent years. The following are the solutions they have implemented, or intend to implement, which could serve as a road map for other states working to improve care for individuals with SCD (see *Figure 2*).

Figure 2. **A Road Map to Improving SCD Care in Medicaid**



Addressing Provider Shortages

As with other rare diseases, identifying enough specialists trained and willing to provide care can be a challenge. States have tackled this issue through numerous strategies, including leveraging telehealth and mobile clinics, building programs that address transitions where individuals are likely to lose care, encouraging more benign hematologists to participate in Medicaid, and educating primary care providers about SCD.

Leveraging Telehealth and Mobile Clinics to Reduce the "Distance" to SCD Specialists

The few SCD specialists available are concentrated in urban areas. Given the size of Southeast states, individuals who live far from urban centers may have to drive hours in each direction to attend an in-person appointment.

The expansion of telehealth may be one way to alleviate this challenge for specific appointments. For 50 years, the North Carolina Sickle Cell Syndrome Program (NCSCSP) has provided state-funded care coordination, genetic counseling education, and more to individuals in North Carolina with SCD, regardless of insurance status. Prior to the COVID-19 pandemic, NCSCSP's genetic counseling services were only offered in person. The pandemic necessitated a change in services, however, and NCSCSP began offering these services via phone. Since making this change, there has been a 300% annual increase in the number of completed genetic counseling sessions, prompting NCSCSP to update its policies to permanently offer these services via phone, highlighting the role telehealth can play in increasing access to some SCD services.

While telehealth can increase access to care for some services, individuals with SCD will need to access other services in person, and states could leverage mobile units or enhanced non-emergency medical transportation (NEMT) benefits to make accessing care easier. While all Medicaid programs provide NEMT, prior reports have highlighted the challenges patients face when using this benefit, including limited availability and late pickups; these challenges were affirmed in a 2022 listening session with North Carolina Medicaid enrollees who have SCD.[39] States, with federal support such as an enhanced match, could explore opportunities to shore up their NEMT programs, such as encouraging or requiring MCOs to provide enhanced transportation benefits through ride-sharing contracts.[40],[41] The Georgia Sickle Cell Foundation has also coordinated a form of mobile units, dubbed "Sickle Cell Clinic Days," to bring hematologists to community health centers and federally qualified health centers on a monthly basis.[42]

Building Transition Programs and Value-Based Payment Models

Of the few SCD specialists available, even fewer provide care to adults than to children and adolescents. States noted that most adult hematologists concentrate in areas like oncology rather than benign blood diseases. This is a growing problem across the country, because while mortality rates for children and adolescents with SCD have decreased over the decades, mortality rates for adults with SCD have increased in a way that appears to reflect care gaps, in addition to increased numbers surviving into adulthood.[43]

To combat challenges for adolescents transitioning into adulthood, Kentucky's Medicaid program developed a program focused on adolescents transitioning to adult care. The program is layered into existing SCD appointments and focuses on topics such as what to do if individuals experience a pain

crisis and why each different medication is an important part of their treatment plan. Adolescents are then tested on what they learned through the program.

Louisiana is considering adopting value-based payment models to encourage additional (benign) hematologists to become Medicaid providers.

Educating Primary Care Providers About SCD

Most primary care providers do not see enough patients with SCD to maintain expertise in how to manage SCD care in culturally appropriate ways for individuals from diverse backgrounds. This is particularly critical for SCD, given that intense pain crises can be frequent and trigger provider biases that affect their approach to pain management and counseling.[44]

The Sickle Cell Foundation of Georgia developed a three-pronged approach to increase access to care for individuals with SCD, with one prong focused on training non-specialty providers (e.g., family medicine providers, nurse practitioners, ED physicians) in how to care for their patients with SCD.[45] The training is delivered jointly by a hematologist and family medicine provider and covers how to treat SCD in primary care settings, including aspects such as pain management and care coordination with community health workers (CHWs).

To ensure access to culturally appropriate care for a growing refugee population, Kentucky Medicaid developed a CHW program. The program is intended to bridge gaps between providers and patients by providing better translation services during appointments and helping individuals more easily navigate the U.S. health system. Kentucky Medicaid began reimbursing for care provided by CHWs in July 2023 on a fee-for-service basis.

Improving Data and Monitoring

Multiple states highlighted the importance of having data systems in place to monitor individuals with SCD across their life span, including whether they are receiving necessary care and how they are faring in terms of long-term health outcomes. Data systems are critical for monitoring care coordination efforts and outcomes associated with new, innovative programs intended to improve care for individuals with SCD. States also highlighted that strong data monitoring would make providing gene therapies easier, by helping them understand which enrollees would be eligible and likely to receive the therapies.

Early in 2023, both Florida's and Kentucky's Medicaid programs released their first reports documenting SCD and health care utilization among their Medicaid populations. Kentucky's report combined findings from Medicaid data analyses and stakeholder interviews to document the prevalence of SCD and Medicaid health care utilization, and also outlined policy recommendations to address gaps in care. Similarly, Florida's report documented the number of individuals with SCD enrolled in Florida Medicaid each year, whether these individuals were receiving evidence-based standards of care (e.g., TCD screening) and overall Medicaid spending on SCD care.[46] These reports provide a model for other states on how to identify Medicaid beneficiaries with SCD and track whether individuals are receiving necessary care.

Other states – including Florida, Louisiana and North Carolina – have been working with partners like MCOs, state Sickle Cell Commissions, providers, and academic institutions to improve data collection and reporting efforts. This includes MCO efforts to improve coding as well as efforts to conduct epidemiological studies and change the criteria for identifying individuals with SCD to align with the CDC case definition. Others have joined the CDC's cross-state SCD data effort (see "*The CDC's Sickle Cell Data Collection (SCDC) Program*").

The CDC's Sickle Cell Data Collection (SCDC) Program

The CDC started the SCDC program more than 10 years ago to provide states with funding to develop state-based data programs focused on SCD surveillance.[47] These programs, which typically include an academic partner, draw on multiple data sources – such as Medicaid claims, newborn screening programs, and clinical sites (e.g., sickle cell centers, hospitals) – and are intended to compile demographic information on individuals with SCD and whether they are able to access needed health care. California and Georgia were the first two states to develop SCDC programs, and 11 total states are now participating, with strong representation across the Southeast including Alabama, Georgia, North Carolina, Tennessee, and Virginia.[48] The program is expected to grow, with the CDC offering an additional grant opportunity early in 2023.[49]

Georgia was an early adopter, and its SCDC program is among the most mature and has driven changes in health care access. Anecdotally, SCD stakeholders and advocates knew there were certain parts of the state where individuals were more likely to experience gaps in care. The Georgia SCDC program was able to pinpoint this gap using utilization data – allowing stakeholders to "show" what they knew with data – and led to the development of a new pediatric outpatient site for SCD. As more states join the SCDC program, there are future opportunities for cross-state analyses to further improve SCD care and identify best practices.

Engaging MCOs in SCD Care

States highlighted the need to involve MCOs in ensuring individuals with SCD are receiving adequate services, given how many Medicaid enrollees receive care through MCOs. Current MCO focus on SCD is mixed, however. For example, three of six Medicaid MCOs in Kentucky offer SCD-specific case management, with services and focus varying by MCO (e.g., one program is focused specifically on the transition from pediatric to adult care). This variation highlights the need for states to develop new ways to collaborate with their MCO partners on SCD.

To encourage more MCO engagement with SCD care, Michigan developed a regional quality collaborative with its MCOs focused on preventive service utilization for children with SCD.[50] Through the collaborative, MCOs have a financial incentive to collectively improve performance in three key areas – TCD screening, antibiotic prophylaxis, and hydroxyurea use – with performance tracked at the regional level, not by individual MCO. While results from the effort are not yet available, this could provide a model for encouraging more MCO engagement with members who have SCD.

Removing Unnecessary Barriers to Care

State Medicaid agencies can also review current policies and procedures to ensure they are not creating unnecessary barriers to SCD care. Through listening sessions with community partners, North Carolina Medicaid heard concerns from providers that they were experiencing difficulty in getting some

medications authorized and that some screenings (e.g., TCD and MRA/MRI) were not being approved together. These concerns spurred a review of medication clinical criteria and utilization management tools related to SCD care, and North Carolina Medicaid was able to confirm that prior authorization for TCD and SCD therapies was either removed or not in place for SCD patients. North Carolina Medicaid also updated its lock-in policy for opioids to exclude individuals living with SCD, to ensure they could access key pain medications, and updated its Genetic Testing, Diagnosis and Treatment Clinical Policy to include red blood cell antigen genotyping in order to assess risk of severe reactions to blood transfusions.

Appendix B: Report Interviewees

Interviewee	Affiliation
Justin Calhoun	North Carolina Sickle Cell Syndrome Program
Carlton Fabien	Individual Living with Sickle Cell
Dr. Angela Snyder	Georgia Health Policy Center, Georgia Sickle Cell Data Collection (SCDC) Program
Dr. Brandon Attell	Georgia Health Policy Center, Georgia Sickle Cell Data Collection (SCDC) Program
Jeanette Nu'Man	Sickle Cell Foundation of Georgia
Dr. Kevin Wessinger	South Carolina Department of Health and Human Services
Dr. Patricia Witherspoon	South Carolina Department of Health and Human Services
Dr. Judy Theriot	Kentucky Cabinet for Health and Family Services
Jonathan Scott	Kentucky Cabinet for Health and Family Services
Patrick Perry	University of Kentucky
Matthew Walton	University of Kentucky
Dr. Shantel Hebert-Magee	Louisiana Department of Health
Dr. Christopher Cogle	Florida Agency for Health Care Administration
Dr. Ofelia Alvarez	University of Miami

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