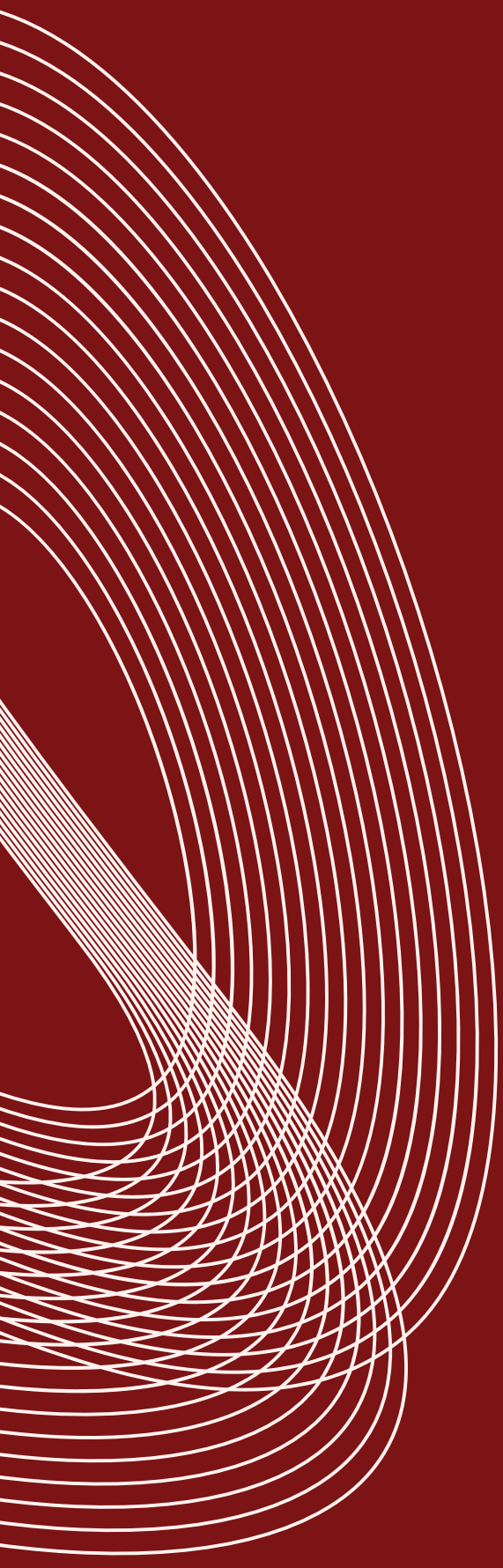

Advancing Stakeholder Engagement with Medicaid

CENTERING THE PATIENT VOICE IN COVERAGE DECISIONS

Sick Cells

MARCH 2022

Executive Summary

Sickle cell disease (SCD) is a lifelong inherited blood disorder that affects an estimated 100,000 Americans.[1] While SCD impacts all racial and ethnic groups, Black and Brown populations are disproportionately impacted.[2] SCD can cause extremely painful episodes, called pain crises, and can lead to a range of serious health issues. Due to racism and patterns of health inequities in the United States, the SCD population has been marginalized in the realms of research, data collection, education, and access to quality care across the healthcare continuum.

More than half of the adults and children living with SCD in the United States rely on government-based health insurance programs, including **Medicaid**, and covered services vary across states.[3] There are several areas of unmet needs, including insufficient insurance coverage, high **out-of-pocket costs**, and under-utilization of SCD treatments.[4] Effective strategies for managing pharmacy benefit coverage, including **preferred drug lists (PDLs)** and **utilization controls**, can improve the ability for individuals with SCD to access more affordable treatments. Controls such as **prior authorization** and **step therapy** are used as cost-containment strategies to limit and restrict access to expensive services.

Within state Medicaid programs, decisions impacting pharmacy benefit coverage (referred to in this paper as "**coverage decisions"**) can vary by state. These coverage decisions are often made by an organized group of medical professionals on coverage decision committees such as the **Drug Utilization Review Board (DUR Board)** or the **Pharmacy & Therapeutics (P&T) committees**. While members of these committees work hard to make informed decisions, most are unfamiliar with SCD and therefore face challenges when deciding access to and coverage of SCD treatments.

Since 2019, Sick Cells has worked to highlight the patient voice in coverage decision meetings by training advocates to share their story. To date, Sick Cells has trained over 25 individuals to advocate for sickle cell disease coverage in their state. This paper outlines the process Sick Cells follows to ensure the patient voice is reflected and prioritized in Medicaid coverage decisions across the country, and reviews current barriers and potential solutions to improve stakeholder engagement at these committee meetings.

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[1] Hassell, K.L. Population Estimates of Sickle Cell Disease in the US. American Journal of Preventive Medicine, 2010. 38(4): p. S512-S521

[2] Centers for Disease Control and Prevention. Data & Statistics on Sickle Cell Disease. https://www.cdc.gov/ncbddd/sicklecell/data.html. Updated December 16, 2020, Accessed November 24, 2021.

[3] Campbell A, et al. The Economic Burden of End-Organ Damage Among Medicaid Patients with Sickle Cell Disease in the United States: A Population-Based Longitudinal Claims Study. J Manag Care Spec Pharm. 2020 Sep;26(9):1121-1129. doi: 10.18553/jmcp.2020.20009. Epub 2020 Jun 29. PMID: 32597290.

[4] National Academies of Sciences, Engineering, and Medicine. 2020. Addressing Sickle Cell Disease: A Strategic Plan and Blueprint for Action. Washington, DC: The National Academies Press.https://doi.org/10.17226/25632.

Glossary of Common Terms

Coverage decisions	The review process a DUR Board (or similar committee) goes through to decide how, and whether, that state Medicaid will cover a certain drug.
Drug Utilization Review Board (DUR Board)	The state-required group of clinicians (doctors, pharmacists, etc.) that reviews new and existing drugs to decide whether, and how, they will be covered by that state's Medicaid program. In some states, this program may be called a P&T Committee or D&T Committee.
Medicaid	A federal and state public health insurance program for eligible individuals.
Out-of-pocket costs	Expenses from medical care that are *not* covered by an individual's insurance and the individual must pay for on their own.
Preferred drug list (PDL)	A state Medicaid's list of recommended drugs. This list is created by a state's DUR Board/P&T Committee and the State Medicaid Director after comparing clinical data and cost of drugs.
Prior authorization	The requirement that a certain drug go through an approval process to decide whether it is medically necessary for an individual before Medicaid will cover its cost, or part of its cost.
Pharmacy and Therapeutics (P&T) Committee	Another type of state Medicaid coverage decision committee that reviews how, and whether, that state Medicaid program will cover a certain drug. In some states, this committee may be referred to as a Drug & Therapeutics (D&T) Committee.
Step therapy	The requirement that an individual must first try a "preferred" drug (indicated on the state's PDL) before Medicaid will cover the costs of an alternative drug.
Utilization controls	Practices like prior authorization and step therapy that are used by insurance programs to manage the costs of healthcare by deciding the medical necessity of certain drugs. Also referred to as "utilization management (UM) practices" and "utilization review."

Introduction

Health insurance affects how individuals with SCD are able to access and afford therapies. According to the Center for Medicare & Medicaid Services (CMS), just under 42,000 of the estimated 100,000 individuals living with sickle cell disease in the United States rely on Medicaid as their primary insurance.[5] When temporary Medicaid coverage is taken into consideration, this number only increases. For example, an analysis of individuals with SCD who were insured by Medicaid for at least one month from 2014 to 2016 shows that 66% of individuals with SCD in California used Medicaid, as did 56% of Georgia residents with SCD.[6] National estimates of health insurance coverage for SCD patients are missing from the literature, however Medicaid likely covers the majority of SCD patients.[7]

In order to understand the importance of advocacy related to access and coverage, one must first understand the landscape of available treatments for SCD. While there are currently four SCD drug types that have gained regulatory approval in the US, utilization remains low, highlighting that many individuals with SCD are not able to access potentially life-saving therapies. Until 2017, only one FDA-approved treatment, hydroxyurea, was available to patients across the United States. Three more treatments were approved by the FDA between 2017 and 2019. All therapies currently available are outlined below (Table 1).

Table 1: Available Treatments for SCD

Drug Type	Brands	FDA Approval Date
Hydroxyurea	Siklos®, Droxia® and Hydrea®	1998 (adults) 2017 (pediatric)
L-glutamine	Endari®	July 2017 (Ages 5+)
Crizanlizumab	Adakveo®	November 2019 (Ages 16+)
Voxelotor	Oxbryta®	November 2019 (Ages 12+) December 2021 (Ages 4+)

[5] Center for Medicaid and CHIP Services, Division of Quality and Health Outcomes. At a Glance: Medicaid and CHIP Beneficiaries with Sickle Cell Disease (SCD), T-MSIS Analytic Files (TAF) 2017. Centers for Medicare & Medicaid Services. Baltimore, MD. 2020.

[6] Medicaid Coverage Patterns Fact Sheets from the Sickle Cell Data Collection Program, Centers for Disease Control and Prevention. https://www.cdc.gov/ncbddd/hemoglobinopathies/scdc-fact-sheet-medicaid-data.html

[7] Campbell A, et al. The Economic Burden of End-Organ Damage Among Medicaid Patients with Sickle Cell Disease in the United States: A Population-Based Longitudinal Claims Study. J Manag Care Spec Pharm. 2020 Sep;26(9):1121-1129. doi: 10.18553/jmcp.2020.20009. Epub 2020 Jun 29. PMID: 32597290.

Medicaid programs use various cost containment and utilization controls to manage prescription drug benefits. As with other payers, state Medicaid agencies work to decrease their costs while still trying to ensure that patient care is not jeopardized. The most common form of utilization controls is a preferred drug list (PDL), which lists outpatient prescription drugs that are considered "preferred" or encouraged by the state for providers to prescribe over other drugs. Non-preferred drugs may still be available to patients, though may have greater limitations such as prior authorization or some form of step therapy. Committees such as the DUR Board and/or the P&T committee play an important role in the oversight of the PDLs, and often determine the placement of therapies. The committees themselves, which are required by federal law, are composed of paid physicians and pharmacists who review evidence and make coverage decisions for therapies.

States have broad discretion to decide which particular treatments will be considered preferred treatments. There is no federal guidance as to the standards states should apply, the evidence they should follow, or the process they should follow in making these coverage decisions. Additionally, while CMS encourages public comments to inform national coverage determinations, there is no national mandate for states to incorporate input from relevant stakeholders including patients, their families, clinicians, and health systems. Once a review has been initiated, the opportunity for public input and participation in the coverage determination process varies greatly by state. Several states hold public meetings and accept written and oral testimony from the public during a public comment period. To this end, Sick Cells has worked since 2019 to increase awareness of these opportunities within the sickle cell disease community, and train advocates to incorporate their input into these meetings.

Existing Barriers to Engagement

Sick Cells believes that patients remain underrepresented throughout coverage decision processes, creating a disconnect between decisions that affect the community and the community itself. Through our engagement with coverage decision committees, Sick Cells has identified four elements of the coverage decision process that were unfavorable for patient engagement. They were (1) lack of specific expertise (2) restrictive public engagement procedures; (3) limited transparency; and (4) insufficient use of existing expert groups. Below we further discuss each barrier identified above.

Lack of Specific Expertise

One major challenge we have found is that often committee members have limited disease-specific knowledge and expertise, which restricts their ability to ensure that patients' experiences, perspectives, and needs are evaluated in their coverage decision making. In order to provide guidance on coverage for SCD, one must consider pertinent issues in SCD such as health care disparities, stigma, race and biases, access to care, mechanisms of action for novel therapies, and serious complications of the disease. There are major gaps in data for SCD that do not exist for similar diseases, and the lack of a national-level SCD data collection hinders the committees' overall ability to assess all of the benefits and costs associated with a particular therapy. In order to address these challenges, committees should consult with individuals with

expertise required to determine what is the most appropriate and cost-effective treatment for their beneficiaries.

Restrictive Public Engagement Procedures

While CMS states that "public participation" increases the quality of coverage decision making, we have found that states' processes for including public feedback often make it difficult for patients to meaningfully engage with these committees. Many DUR Board and P&T committees host meetings that last several hours, requiring participants to take off work or skip other priorities in order to attend. Meeting agendas often do not indicate the specific time that a product or class of drugs will be reviewed, so public members must sit through hours of the meeting listening to irrelevant discussions waiting for their name to be called. Some meetings place public comments near the front of the agenda, which reduces the participation burden for advocates. Participants typically have between three and five minutes to speak, and in several occurrences, the committee members were not able to ask questions directly to the members of the public. In one instance, committee decisions were already voted on prior to public testimony. Registration processes can also be challenging to navigate, as it is difficult to find guidance on how to sign up as a speaker, what evidence is considered meaningful, and how this should be incorporated into public comments. Since the start of the COVID-19 pandemic, committee meetings have pivoted to utilizing videoconferencing or virtual meeting settings, which has helped to facilitate stakeholder participation. We hope this trend towards making virtual events more accessible to the public becomes "the new normal." Committees should consider other ways they can adjust the engagement procedures discussed above in order to facilitate more meaningful engagement of patients, caregivers, and clinicians.

Limited Transparency

Limited transparency across committee procedures further reduces public engagement in this process. Membership lists of committee members and their affiliations are often not made public, therefore creating concerns over who is making these decisions, as well as their specific expertise. Additionally, it is challenging to identify when committees will be discussing a therapy or class of drug of interest due to lack of transparency and advance notification. During the meetings themselves, many committees host closed sessions to discuss sensitive topics such as pricing and rebates that are not disclosed publicly. During these closed sessions, committee members ultimately make the decisions that impact access including placement on a PDL or development of prior authorization and reauthorization criteria. The lack of transparency into these decisions limits stakeholders' ability to provide feedback or highlight considerations such as analytical methodology or available data. An example of this barrier is further discussed in Case Study #1. Better transparency will help ensure fair and balanced decision-making processes for these committees.

Case Study #1: Texas Medicaid DUR Board Meeting

Sick Cells prepared advocates for participation in a Texas Medicaid DUR Board meeting on April 23, 2021. The Board was reviewing the PDL placement and utilization controls for all SCD treatments. Five SCD advocates representing local patients and community-based organizations shared testimony for the virtual Board meeting. The Board seemed receptive to their comments, engaged in discussion with advocates, and thanked them for their participation.

After the public session, the Board moved into a closed executive session to discuss recommendations. Once completed, the Board members returned to the public session to announce recommendations:

- **Board Recommendation**: for Siklos®, Endari®, and Oxbryta® to move the PDL from the previous "non preferred" status and remove prior authorization.

Recommendations were provided to the Executive Commissioner at Texas Health and Human Services Commission (HHSC), who makes all final coverage decisions. When the updated PDL was released a few months later, no changes were made to the placement of SCD drugs.

- **Final Decision**: HHSC determined no change and Siklos®, Endari®, and Oxbryta® remain listed as non-preferred treatments.

Private board meetings make it difficult to understand what considerations are prioritized and included in the decision-making process. This case highlights challenges for stakeholders to engage with the committees with limited transparency.

Insufficient Use of Existing Expert Groups

Due to recent success across rare disease advocacy efforts, several states have established expert advisory committees or task forces whose role is to provide necessary consultation to the DUR Board or other committees when making recommendations or determinations regarding therapies for rare diseases. These existing groups help advise on coverage decisions when the committee is lacking the specific scientific, medical, or technical expertise needed. In our work, we have found that these advisory councils or task forces were, however, not notified or involved in making recommendations or determinations for sickle cell disease (discussed in Case Study #2). These work groups are often comprised of expert patients, caregivers, advocates, and providers, and should be consulted or approached when developing criteria and recommendations for rare disease drugs, including sickle cell treatments.

Case Study #2: Missouri Medicaid Prior Authorization Committee Meeting

Sick Cells conducted outreach to local advocacy groups in Missouri prior to the Medicaid Prior Authorization Committee Meeting on June 17, 2021. One local advocate, the executive director of a local SCD organization, was surprised to learn that SCD therapies were listed on the agenda, as she was a member of the Rare Disease Advisory Council for Missouri HealthNet, the state Medicaid carrier. She had not been informed about the coverage decision review, despite her role as SCD community leader and active council member. The advisory council was created to "serve as an expert advisory committee to the drug utilization review board, providing necessary consultation to the board when the board makes recommendations or determinations regarding beneficiary access to drugs or biological products for rare diseases." Disconnects like this reduce the effectiveness of these expert groups.

Moving Towards Solutions

Working to improve engagement with Medicaid programs has become an essential part of Sick Cells' advocacy initiatives in order to reach our organizational goal of improving access to care for all people with SCD. Through reviewing published information and guidance documents from others in the rare disease advocacy space, like the Cystic Fibrosis Research, Inc. (CFRI), Hemophilia of Georgia (HoG), and the Everylife Foundation, Sick Cells' has worked to advance a new strategy within the sickle cell community to improve engagement with state Medicaid programs.

Engaging the SCD Community in Advocacy

Sick Cells works through our Ambassador Program, one of our two keystone advocacy programs, to create change by training advocates and providing education on advocacy opportunities through Medicaid programs. We believe that advocates' experiences, paired with research and real-world data, have the power to influence coverage decisions and improve access for beneficiaries. Sick Cells activates with our network of advocates, providers, non-profit leaders, and community-based organizations (CBOs) to carry out five main goals:

1. **Affect positive change** in state Medicaid coverage of sickle cell disease treatments;
2. **Promote meaningful patient engagement** to inform coverage decisions;
3. **Build relationships** with state Medicaid directors;
4. **Increase patient** *access* to SCD therapies;
5. **Educate advocates** on state-level advocacy opportunities.

Sick Cells has developed a five-step process to educate and prepare SCD advocates for advocacy at Medicaid committee meetings:

1. **Identify** meetings with SCD on the agenda
2. **Conduct outreach** to local advocates and Medicaid meeting coordinators
3. **Organize a planning meeting** with interested Ambassadors, advocates, CBOs, and clinicians in the state
4. **Assist with registration for local advocates** including oral and written testimony
5. **Attend virtual meeting** and conduct follow-up

Since 2019, Sick Cells has trained over 25 advocates and coordinated public comments at 11 coverage decision meetings. Case Study #3 and #4 highlight two powerful efforts of engagement from SCD advocates at Medicaid committee meetings. Sick Cells hopes that through continuing this work and empowering local organizations to become leaders in their states, this coordinated, nationwide strategy that extends to the rare disease community as a whole, can facilitate meaningful engagement with patients and Medicaid committees and positively impact coverage decisions.

Case Study #3: Illinois Medicaid D&T Committee Meeting

The Illinois Drug and Therapeutics (D&T) Committee hosted a meeting on April 15, 2021, to discuss the PDL placement and utilization controls for Oxbryta®. As of early April 2021, hydroxyurea was listed as preferred on the PDL while Endari®, Oxbryta®, and Adakveo® were all listed as non-preferred. Sick Cells staff conducted outreach to local patient advocates and clinicians in the state, educating them about the advocacy opportunity and coordinating a planning meeting. Six advocates registered to provide personal testimony:

- **Two hematologists** from local academic institutions
- **One nurse practitioner** from a local sickle cell center
- **Two advanced practice nurses** from pediatric and adult centers
- **One community-based organization leader** and **SCD caregiver**

Each shared compelling testimony from their perspective reflecting on themes such as the complexities of the disease, the inappropriateness of step therapy protocols due to different mechanisms of action for Oxbryta®, and the barriers to access that patients face. Their testimonies included a call to action for the committee members to prioritize strategies that reduce barriers to access for SCD beneficiaries. While the committee did not recommend making any changes to PDL, this was an impressive opportunity for these advocates to join together and elevate the voices of the community. Effective advocacy begins with strong relationships, and through this opportunity the individuals become passionate about staying engaged with the Medicaid committee and program staff.

Case Study #4: Wisconsin Medicaid DUR Board Meeting

Sick Cells worked with two advocates in Wisconsin to prepare for a DUR Board meeting on November 3, 2021. Prior to the meeting, Sick Cells contacted Medicaid coordinating staff to ask questions and register the advocates. Staff was responsive and engagement processes were clear, setting in place a strong foundation for advocates to engage in the meeting.

The Board was reviewing four sickle cell drugs: Hydroxyurea, Droxia®, Endari®, Siklos®, and Oxbryta®. The day of the meeting, two patient advocates each shared their personal testimony and included a call to action for the committee to add all SCD drugs as "preferred" on the PDL without restrictions. Their stories highlighted how each individual experiences SCD differently, and how different therapies can work differently for each individual. The DUR Board was receptive to their testimonies and engaged in conversation, demonstrating their understanding of the discussed topics.

- **Final Decision:** The Wisconsin DUR Board recommended moving three of the four treatments (Hydroxyurea, Droxia®, Siklos®, and Endari®) to the drug formulary as "preferred" drugs.
- **This recommendation was adopted** in the subsequent update to the PDL.

This case serves as an example of the power of patient testimony at state Medicaid meetings to influence coverage decisions and improve access to effective coverage.

Recommendations for Stakeholder Groups

Sick Cells has developed actional recommendations, both for the SCD community and for state Medicaid programs. These recommendations are meant to increase communication between the two and encourage active participation from SCD advocates.

<u>For members of the SCD Community:</u>

1. **Join the Sick Cells Ambassador Program** to connect with other Ambassadors in your state and take part in monthly advocacy trainings.
2. **Attend coverage decision committee meetings** using the guidance outlined in this paper.
3. **Write letters** to your state Medicaid Director supporting new therapies being added to the PDL and list your organization as a local expert in SCD.
4. **Apply for membership** to local advisory committees or task forces that participate as key stakeholders in decision making.
5. **Talk to state legislators** or the Governor about necessary changes to Medicaid committees' operations and/or procedures in order to facilitate better stakeholder engagement.

For state Coverage Decision Committees:

1. **Perform outreach to community experts** (i.e., build relationships, utilize advisory panels).
2. **Incorporate processes which facilitate meaningful stakeholder engagement** (i.e., virtual meeting agendas, allotted time for discussion, accepting written and oral comments).
3. **Create an alert system** for meeting notifications and disease-specific agenda announcements.
4. **Publish guidance to providing input** (i.e., highlight information needed, identify data gaps or evidence needed to support decision making).
5. **Offer additional opportunities for engagement and partnership** (i.e., advisory committees, annual meetings with community leaders).
6. **Promote transparency** in decision making (i.e., summarize methodology, data, and patient priorities considerations).

Conclusion

Sick Cells' experiences working with different coverage decision committees in various states has highlighted the individuality of this work. Sick Cells will continue collaborating with advocates and organizations across the country to build relationships with their state Medicaid programs and share best practices. In addition to advocating for changes that facilitate better access for SCD treatments, Sick Cells will also prioritize advocating for engagement standards that improve coverage decisions procedures and meaningfully engage community members. We hope that by continuing this work our community can shape how Medicaid beneficiaries with SCD are able to access and afford their treatments, and ultimately improve outcomes for SCD patients across the country.

Acknowledgements

Sick Cells would like to thank all of the SCD warriors, caregivers, providers, and advocates who worked tirelessly to improve access to and coverage of SCD treatments across the country. We are infinitely grateful to you for your passion and willingness to share your stories. Your hard work has already improved the lives of so many, and we are honored to work alongside you.

About Sick Cells

Sick Cells is a national sickle cell disease advocacy nonprofit founded by sibling duo Marqus and Ashley Valentine. Our mission is to elevate the voices of the SCD community and their stories of resilience. In highlighting the grave disparities this community faces, we hope to influence decision makers and propel change. Our narrative work has ignited public interest; humanized SCD; inspired the general public to advocate for SCD; influenced policy makers; driven research and drug development; and empowered the sickle cell community. For more information, visit www.sickcells.org or find us on all social media platforms at @sickcells.

To get directly involved with our Medicaid advocacy work, join the Ambassador Program.

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