COVERAGE FOR SCD SUMMIT

October 2021

Convening Proceedings Report

Table of Contents

Introduction

Overview

On August 31st, 2021, Sick Cells hosted a first-of-its-kind multi-stakeholder summit to discuss the changing landscape of healthcare coverage for sickle cell disease (SCD). The **Coverage for SCD Summit** brought together payers, providers, pharmaceutical manufacturers, patient advocacy groups, pharmacists, policymakers, and researchers to discuss ways to tackle the complexities of coverage and access to SCD treatments and care.

With rising costs, persistent health inequities, and gaps in care access, there is a heightened focus across sectors on new approaches to achieve better health outcomes for patients with SCD. The four-hour virtual summit explored innovative ways stakeholders are approaching these challenges. Panelists shared perspectives and highlighted opportunities to improve outcomes, measure value, and better navigate healthcare costs.

This report contains proceedings and recommendations from this summit, which are intended to inform next steps to drive collaborative efforts and address gaps in access and coverage for SCD treatments.

Background

It is estimated that approximately 100,000 Americans suffer from SCD.[1,2] Unpredictable, recurrent, and excruciating episodes of acute pain, known as pain crises, and daily chronic pain are responsible for most of the psychosocial devastation of the disease. SCD has profound effects on every organ and system of the body, and results in a range of serious health complications. While SCD impacts all racial and ethnic groups, Black/African American and Hispanic populations are disproportionately impacted.[3] Systemic racism within the US healthcare system greatly impacts the type of care SCD patients receive and influences access to treatment.[4]

As new disease-modifying and curative treatments become available, payers must make decisions regarding coverage policies and utilization controls. Patients and caregivers remain worried about the affordability and accessibility of treatments. Understanding and defining value for sickle cell treatments has become a priority across diverse stakeholder groups including payers, manufacturers, researchers, health economists, and patients.

SCD disease is costly, and total medical costs for SCD beneficiaries can be a significant burden to public and private payer systems.[5,6] The Centers for Medicare & Medicaid Services and state Medicaid programs are the leading payers for SCD care. Even though they pay for most SCD services, there is limited coverage for therapies to treat comorbidities of SCD and novel therapies. Total medical costs for patients with SCD are 1400% higher than in matched control groups.[7]

To address these broad coverage, cost, and access challenges, Sick Cells hosted the **Coverage for SCD Summit** with the goal of identifying best practices to manage coverage and access from diverse perspectives and to come together to find opportunities for collaboration. The four-hour virtual Summit invited stakeholders to share their ongoing work and highlight opportunities for improvement.

Summit Details

The Coverage for SCD Summit was organized into four sessions, each centering perspectives and strategies tailored to a different stakeholder group. Patient advocates and clinicians opened the Summit highlighting national-level advocacy efforts. The first panel hosted pharmacists, payer representatives, and seasoned healthcare professionals to outline three payer-focused strategies to reduce care gaps. The second panel brought together researchers, health economists, and data scientists to discuss efforts for generating meaningful data to measure value for coverage decisions. During the final session, pharmaceutical manufacturers and industry groups call for collaboration to ensure health policies remove hindrances to patient access for future gene and cellular therapies.

Opening Session: Efforts to Address Access to Sickle Cell Care

Dr. Emily Riehm Meier
Co-Chair
American Society of Hematology, Committee on Government Affairs
Speaker's Page

TERRI Booker
Board Member
Sick Cells
Speaker's Page

The Summit opened with a welcome and updates from **Ashley Valentine MRes**, co-founder and President of Sick Cells. Ms. Valentine identified past and ongoing advocacy efforts led by Sick Cells aiming to improve access and coverage for individuals with SCD. Details are highlighted below:

- **My Life with Sickle Cell: Patient and Caregiver Survey**. Sick Cells engaged with the Institute for Clinical and Economic Review (ICER) during their 2019-2020 value assessment for three new SCD medications: Crizanlizumab, Voxelotor, and L-Glutamine. To address data gaps, Sick Cells developed and fielded an online survey – the My Life with Sickle Cell: Patient and Caregiver Survey – to gather SCD patient and caregiver work and activity impairment and out-of-pocket costs data. Analyses included 452 respondents, representing 287 patients and 165 caregivers. Findings show the importance of elevating the lived experiences of patients and caregivers in health economic assessments to inform coverage decisions.
- **Advancing Care for Sickle Cell Disease: A Strategic Roadmap**. In November 2020, Sick Cells, in partnership with Avalere Health, convened a multi-stakeholder dialogue and asked participants to share potential solutions to address the persisting gaps in SCD care. The culmination of these varying ideas resulted in the development of this Strategic Roadmap. The Roadmap includes recommendations for actions that patients, advocacy organizations, providers, researchers, payers, and manufacturers can take to improve the quality of care across the healthcare continuum.
- **Patient Testimony at Medicaid Drug Utilization Review Boards**. Since 2020, Sick Cells has trained advocates across the country to share their story and advocate for fair access and coverage for SCD treatments. Within state Medicaid agencies, preferred drug lists (PDL) can vary by state and affect how individuals with SCD are able to access and afford therapies. Coverage decisions are often made by the Drug Utilization Review Board (DUR Board) or the Pharmacy & Therapeutics Board (P&T). Several states hold public meetings and accept testimony from the public during decision-making. To this end, Sick Cells has worked to

increase awareness of these opportunities within the sickle cell disease community, and train advocates to incorporate their input into these meetings.

Next, **Dr. Emily Meier MD**, Vice-Chair to the American Society of Hematology's (ASH) committee on Government Affairs, provided updates on the current state of access to care and opportunities for improvement. Three important resources were used to inform the current state of access for SCD patients:

- **The State of Sickle Cell Disease Report Card (2020)**. To highlight the urgent need for change, ASH and SCD stakeholder groups issue a biannual report card on the state of sickle cell disease based on surveys of community leaders. In 2020, the survey indicated a 5.7 rating out of 10.0 scale for "Access to Care." While this 2020 report card suggests we are making progress, the scores also indicate that we have much to do to improve the state of care for those living with SCD. Barriers to access can include lack of insurance, transportation needs, and provider inexperience and lack of knowledge about SCD.
- **Addressing Sickle Cell Disease: A Strategic Plan and Blueprint for Action (2020).** The NASEM report explores the current guidelines and best practices for the care of patients with SCD and recommends priorities for programs, policies, and research. It also discusses limitations and opportunities for patient advocacy and community engagement groups.
- **Medicaid and CHIP Sickle Cell Report (2020).** The SCD Report includes detailed state-level analyses for demographic, health characteristics, and health care utilization patterns among Medicaid and CHIP beneficiaries who are under age 76 and living in the United States or in the territories. To the extent possible, findings are reported for beneficiaries with and without SCD. The SCD Report also features information on recommended screenings and preventive care for children with and without SCD to improve understanding of the diverse populations served by state Medicaid and CHIP programs and highlight opportunities for quality improvement.

Dr. Meier shared several of ASH's initiatives to address care gaps including: (1) provider education and training, (2) advocacy for federal policy strategies, and (3) coordinating the Sickle Cell Disease Coalition. The opening session concluded with a moderated discussion between **Terri Booker Esq**, Board member of Sick Cells, and Dr. Meier to discuss ways the SCD community can advocate for improved access and adequate, affordable coverage for patients.

Panel 1: Emerging Approaches to Reduce Care Gaps

Maia Z. Laing, MBA
Vp of Product
Optum
Speaker's Page

Ashley Valentine, MRes
President and Co-Founder
Sick Cells
Speaker's Page

Matt Powers
Managing Director
HMA
Speaker's Page

Terri Newman
Assistant Professor
University of Pittsburgh
School of Pharmacy
Speaker's Page

The first panel invited payer and pharmacy stakeholders to discuss approaches to improve affordable access to high-quality care for SCD beneficiaries. Through the panelist presentations, attendees could identify strategies that can be used by other organizations and payer systems to address affordability challenges and improve the patient experience.

- **Matt Powers**, Managing Director at Health Management Associates, opened the panel discussing innovative payer-based approaches within Medicaid managed care plans. As states are moving from fee-for-service to managed care plans, trends in state-level managed care plans have major impacts on SCD beneficiaries. Additionally, Mr. Powers highlighted the importance of aligning strategies with other state priorities, including addressing health disparities and social determinants of health, integration of behavioral health for high-need and vulnerable populations, and using data to drive health equity programs.
- **Terri Newman PharmD**, Assistant Professor at the University of Pittsburgh School of Pharmacy, spoke on strategies to identify real-world barriers to treatment use for SCD. Her presentation was a call to action for stakeholders to identify barriers to access, characterize how they affect treatment use, and develop strategies to circumvent challenges. Through exploring coverage criteria among different insurance plans, Dr. Newman highlighted how prior authorization criteria can limit access and lead to unequal access across populations. The University of Pittsburgh has ongoing research utilizing mixed methods of claims data, surveys, and interviews to examine patterns of treatment use and identify barriers to use among patients and providers. Results will be shared with the SCD community in Summer 2022.
- **Maia Laing MBA**, Vice President of Product at Optum, joined the conversation discussing efforts to address health equity through patient-centered, data-informed innovation at UnitedHealth Group. Optum provides care delivery, consumer engagement, and health financial services for UnitedHealthcare members, which includes 3,635 beneficiaries with a sickle cell diagnosis. Ms. Laing presented several strategies used at Optum to proactively optimize care for these individuals by ensuring access to the right experts and treatments for their condition. Optum conducts direct engagement with beneficiaries, families, and providers to provide support, partners with expertise, and assistance for complex health needs. Additionally, the program proactively follows prior authorizations and resolves insurance issues or questions.
- The session concluded with a moderated discussion led by **Ashley Valentine** from Sick Cells.

Panel 2: Generating Meaningful Data to Measure Value

Maggie Jalowsky
Director of Advocacy
Sick Cells
Speaker's Page

Kate Johnson, PhD
Postdoctoral Fellow
The Comparative Health Outcomes, Policy, and Economics (CHOICE) Institute, University of Washington
Speaker's Page

Morenike AyoVaughan, MHA
Senior Manager
Avalere Health
Speaker's Page

Randall Curtis, MBA
Project Manager
Hematology Utilization Group Study (HUGS)
Speaker's Page

The second panel invited researchers and health economist stakeholders to discuss current methods and data available for value assessments for SCD. Through presentations and discussions, this panel highlighted advances being made across the broad research efforts to gather patient inputs and incorporate the patient perspective in value assessments and coverage decisions for SCD.

- **Morenike AyoVaughan MHA**, Senior Manager at Avalere Health, discussed the need for a patient-powered data hub to address the lack of national-level SCD programs and resulting

data and knowledge gaps. A patient-driven data hub would collect unique patient information such as social needs impacting access to care, experiences at different points of care, and management of crises, ultimately improving payers' ability to assess all of the benefits and costs associated with a particular therapy. Additionally, Ms. AyoVaughan shared strategies to promote community-based participatory research, as SCD will benefit from this model by enabling researchers to connect better with the SCD community, especially Black and Brown communities who are most affected. These strategies can provide an avenue to bridge the trust gap with the minority community and involve them in every step of the data collection.

- **Randall Curtis MBA**, Chair of the Hematology Utilization Group Studies (HUGS), shared early findings from the HUGS research collaborative aiming to translate data into costs for SCD. Cost of care was calculated utilizing measures such as cost of regular doctor office visits, emergency room visits, and hospitalizations. Additionally, Mr. Curtis calculated indirect costs estimates from missed work due to SCD, which was gathered from a recent survey utilizing the work productivity and activity impairment (WPAI) instrument. Mr. Curtis finished with a call to action for researchers and patient advocates to ensure data are published in peer-reviewed academic journals in order to advance rigor and facilitate cross-stakeholder utilization of meaningful data sources.
- **Kate Johnson PhD**, Postdoctoral Fellow from University of Washington, Seattle, concluded the presentations summarizing her work to evaluate the clinical and economic impact of curative therapies for SCD. In collaboration with the Cure Sickle Cell Initiative, Ms. Johnson shared findings from a landscape analysis for cost-of-illness studies in SCD and cost-effectiveness analysis of genetic therapies for SCD, and ongoing work to develop a simulation model to produce lifetime estimates of outcomes and costs by modeling state-specific estimates of payoffs in health and healthcare utilization. Model inputs will include patient-important factors such as health-related quality of life, out-of-pocket medical expenses, caregiver burden, and productivity loss. The simulation model will inform economic burden measurements, information for policymakers, and comparative value for future curative treatments for SCD.
- The session concluded with a moderated discussion led by **Maggie Jalowsky** from Sick Cells.

Panel 3: Determining the Value of Future Innovations for Sickle Cell Disease (Gold Sponsor Panel)

Vertex Pharmaceuticals
Gold Level Sponsor
Speaker's Page

bluebird bio
Gold Level Sponsor
Speaker's Page

Nola Juste
Ambassador
Sick Cells
Speakers

The final session of the Summit hosted our Gold Sponsor sub-sessions. The overarching theme discussed preparation for the value assessment process of emerging advanced therapies and identifying opportunities for the SCD community to ensure patients will have access to and coverage for new therapies in the future.

Sub-session: Presentation from bluebird bio

Ravi Singh, Senior Director of Access, Value, and Evidence Strategy from bluebird bio, shared organizational updates and opportunities to advance policy and coverage for future gene therapies. LentiGlobin™ for sickle cell disease (bb1111) is an investigational treatment being studied as a potential treatment for SCD. While this investigational product is not approved for use by the US Food and Drug Administration (FDA) and safety and effectiveness have not been established in the US, many complex questions are already arising related to pricing, reimbursement, and value-based agreements. The “bluebird approach” focuses on cost-effective analysis and value measurements that quantifies and incorporates the potential impacts on patient quality of life, survival, treatment cost and society. By utilizing established modeling techniques, bluebird bio is working with payer stakeholders to better understand how to address potential coverage issues in Medicaid agencies and other payer networks. Through early considerations of factors that will impact value and access, they hope that future innovations in SCD will not face barriers for access and coverage.

Sub-session: Presentation from Institute for Gene Therapies (Sponsored by Vertex)

The Institute for Gene Therapies (IGT) joined for the final presentation to discuss the need to advocate for a modernized policy framework that encourages innovations and promotes patient access to the treatments they need. IGT represents innovators and patients, business leaders, and academics working to ensure policies reflect medical advances, creating a new reality for patients. In this session we were joined by the following presenters:

- **Donna Christian-Christensen MD**, Chair of Scientific, Academic, and Medical Advisory Council, Institute for Gene Therapies
- **John Feore**, Director of Health Policy & Advocacy, Institute for Gene Therapies
- **Samantha Holland**, Manager of Health Policy & Advocacy, Institute for Gene Therapies

Speakers discussed IGT’s priority to address regulatory and reimbursement hurdles to speed near-term access to gene therapies. With the FDA’s prioritization of the acceleration of gene and cell therapy approvals, there are rising concerns that the current reimbursement system will not be able to accommodate the influx of approvals, which will adversely impact patient access. To help ensure these transformational therapies can be approved in a timely way and patients can access them when they are available, novel development pathways need to be embraced and new value-based arrangements must be tested. IGT’s work highlights the strategy to partner with policymakers and advance policy solutions to modernize the reimbursement pathways.

The session concluded with a moderated discussion hosted by **Nola Juste** from the Sick Cells Ambassador Program. Afterwards, Ashley Valentine provided closing remarks and a call to action for

the SCD community to come together to improve coverage for care and treatment for SCD

Key Recommendations for Stakeholders

Improving access and coverage for sickle cell disease requires a multi-stakeholder approach. In order to ensure patients with SCD can access and afford treatment, each stakeholder type will need to embrace a unique set of roles and responsibilities. Table 1 presents key opportunities for stakeholders.

Table 1: Recommendations for Various Stakeholders to Improve SCD Access and Coverage

Stakeholder	Recommendations
Community-Based Organizations and Patient Advocacy Organizations	• Lead the development of patient-powered data hubs to collect outcome measures and economic burden data, and translate that information to inform value assessments • Advance policy solutions to improve patient engagement in coverage determinations and to modernize reimbursement pathways • Advocate for better incorporation of patient priorities and preferences in cost-effectiveness methodologies • Engage with researchers in community-based participatory research (CBPR) to collect patient preference data and publish findings • Partner with payers to align priorities and identify formulary management strategies to address health disparities
Payers and Pharmacists	• Conduct proactive outreach to beneficiaries with SCD to optimize care and ensure access to the right experts and treatments • Establish a care team of experts to provide support and assess coverage criteria such as prior authorization requirements • Collaborate with health systems to increase care coordination • Partner with CBOs to align strategies for addressing health disparities and social determinants of health and drive health equity programs
Researchers and Health Economists	• Collaborate with patient advocacy organizations to develop patient-powered data hubs and design research agendas focused on data gaps for value assessments • Conduct research focused on non-clinical outcomes (patient experience, economic burden) in collaboration with community members under community-based participatory research. • Promote the use of societal perspectives in value assessments
Manufacturers	• Align clinical trial outcomes with patient preferences • Engage in CBPR to understand the impact of therapies on patients' outcomes and communities' barriers to accessing therapies • Develop SCD-focused comprehensive patient support services to mitigate access and affordability barriers to treatment
Clinicians and Professional Organizations	• Advance federal SCD advocacy and policy strategies to improve reimbursement for SCD care and treatment • Identify barriers to patient use of SCD treatments

Conclusion

In this era of increasing availability of treatment options for SCD, there is a pressing need to develop multi-stakeholder approaches to mitigate access barriers and ensure individuals with SCD receive adequate coverage for their treatment and care. This Convening Proceedings Report provides a call to action for stakeholder groups to come together, advance care, and improve access for individuals with SCD. Specific strategic recommendations are provided as a patient-centric starting point, but ensuring fair coverage for SCD will require contribution from the entire healthcare, medical, and patient community, and consistent advocacy efforts to reflect the changing policy landscape. Sick Cells looks forward to hosting this annual Summit to drive collaborative efforts and address gaps in access and coverage for SCD treatments.

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