2024 Coverage for SCD Summit

Convening Proceedings Report

SICK CELLS

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Introduction

Overview

On December 10, 2024, Sick Cells hosted our fourth annual Coverage for SCD Summit. This multi-stakeholder summit discusses the changing landscape of healthcare coverage for sickle cell disease (SCD) and brings 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.

Background

It is estimated that approximately 100,000 Americans suffer from SCD. Unpredictable, recurrent, and excruciating episodes of acute pain, known as pain crises, and daily chronic pain and fatigue 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.

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, patients, and community based organizations.

With the FDA approval on December 8, 2023 for two cell and gene therapy (CGT) treatments, CASGEVY™ from Vertex Pharmaceuticals/CRISPR Therapeutics and Lyfgenia™ from bluebird bio, it is more important than ever to ensure stakeholders across the SCD treatment landscape are working together to address barriers to access and coverage.

Summit Details

The 4 hour virtual event convened 150 attendees including representatives from 24 Community-Based Organizations (CBOs) and 11 different State Medicaid and Health Departments.

The 2024 Coverage for SCD Summit included seven sessions each centering perspectives from a different stakeholder group. This year's featured topics included:

- **Innovative Access Models for Sickle Cell Disease:** Learned about the Centers for Medicare & Medicaid Services (CMS) Cell and Gene Therapy Access Model and how it aims to expand access to cutting-edge treatments for Sickle Cell Disease (SCD), ensuring equitable care delivery.
- **Medicaid Access and Coverage Insights:** Heard from healthcare experts and state Medicaid directors about the current landscape of Medicaid coverage for SCD, key challenges, and actionable policy recommendations to improve access and reduce disparities.

- **State-Level Strategies for Gene Therapy Adoption:** Discovered how states are navigating the integration of cell and gene therapies into Medicaid programs, with a focus on overcoming barriers to access and enhancing health outcomes for SCD patients.
- **Patient Experiences and Community Engagement:** Gained valuable insights from patients who have accessed new therapies for SCD, along with discussions on how CBOs are addressing critical healthcare coverage gaps for underserved populations.

Session Overviews

Session 1 - Keynote

Speaker

Aurelia Chaudhury
Centers for Medicare & Medicaid Services

Aurelia Chaudhury is a Model Co-Lead for the Cell and Gene Therapy Access Model at the CMS Innovation Center (CMMI). Aurelia graduated from Rice University with a degree in Economics in 2009. She worked in healthcare consulting and for the Massachusetts Medicaid program prior to attending Yale Law School, from which she graduated in 2016. Aurelia spent several years in litigation, clerking for two federal judges and later working for a plaintiffs' side antitrust firm. Aurelia joined CMMI in 2021. Prior to taking on her current role, she served as the Model Lead of the Value-Based Insurance Design Model.

Session Goal

The keynote session featured Aurelia Chaudury, Co-Lead of the Cell and Gene Therapy (CGT) Access Model at CMS. Aurelia provided an explanation of the Model structure and the current status of the Model while outlining its core objectives: improving patient access, reducing healthcare expenditures, and enhancing health outcomes. CMS has completed the initial tasks in the 7 step process, including: Model design; announcement to the states; input gathering from states, clinician and patient groups; and manufacturer negotiations. They are currently accepting state applications in preparation for the final step which is launching the Model at the end of 2025 or beginning of 2026.

Aurelia also provided an in-depth look at the requirements and support opportunities for the states participating in the Model. The requirements are in place to address potential barriers to access and include: securing legal authority, adopting standardized drug access policies, adjusting payment structures by carving out Model drugs if necessary, and ensuring provider and managed care plan alignment with model protocols. Aurelia explained how CMS involvement also extends to support for the states through optional operation funding through a notice of funding opportunity that assists states with implementation costs or projects to enhance their ability to implement the model effectively.

The session wrapped up with a Q&A moderated by Ashley Valentine, CEO and cofounder of Sick Cells. Questions were answered to clarify that the Model is for Medicaid beneficiaries only and will not include Medicare beneficiaries. CMS funding is for operational and implementation costs as opposed to the cost of the treatments themselves. CGT will be conducted at authorized treatment centers only, but partnerships will be made with smaller centers to promote access and provide auxiliary services, and states will have the option to participate in either or both CGT treatments.

Session 2 - SCD Landscape Over the Years: Reflections of Progress, Treatment Access, and Future Hope

Speaker

Pat L Corley, RN
Nurse Coordinator

In 1979 Pat became the Nurse Coordinator for one of ten newly found National Institutes of Health (NIH) Sickle Cell Centers. She was Nurse Coordinator at LAC+USC for the Sickle Cell Disease Comprehensive Center for over three decades. Her many projects have included the "Unveil Sickle Cell" campaign, sitting on the NIH Focus Groups committee, the International Association of Sickle Cell Nurses and Professional Associates committee, and the Children's Hospital of Los Angeles Group committee. Today, Pat is the recipient of the Elliott Vichinsky Health Care Provider Award, she lectures at numerous institutions, and partners with Cayenne Wellness Center and Axis Advocacy to improve the plight of persons living with sickle cell disease.

Tristan Lee
Community Member

Tristan Lee was diagnosed with sickle cell disease at the age of 6 months old in 1983. Due to not much being known about SCD, the doctor who diagnosed Tristan told his mom and grandmother that he would not live past 20. However, being a family of faith, they trusted in the Lord understanding that God has the final say. Tristan is now 41 years old and is a professional model, actor, and has a fashion line for SCD called DiVo Stars. Tristan is a professional patient advocate for the SCDAA and Sick Cells. He had the opportunity of being NORD's Rare Disease Day Hero in 2020, presenting at the inaugural HHS Sickle Cell Summit during September 2024, and representing the SCD community locally, nationally, and internationally. Spreading sickle cell disease Awareness is a passion for Tristan just as much as fashion and acting.

Session Overview

In this session, we heard a community perspective about how the landscape for SCD has changed since the 2014 FDA Patient Focused Drug Develop meeting. Community members spoke on topics such as the monumental shifts in awareness about SCD, an increase in community advocacy surrounding access and care, an uptick in federal engagement and funding, and a lush pipeline for drug development. We spoke with Tristan Lee and Pat Corley about their reflections on the past decade for the SCD community, touching on topics including: geographic and insurance barriers, healthcare navigation, and advocacy impacts.

Session 3 - Leading the Way: Payers Discuss Sickle Cell Coverage Initiatives

Speakers

Jennifer Strohecker
Utah Dept. of Health and Human Services

Dr. Jennifer Strohecker, PharmD, serves as the Integrated Healthcare Division and Medicaid director at the Utah Department of Health and Human Services. She has direct oversight of the state Medicaid and Children's Health Insurance Program (CHIP), Office of Substance Use and Mental Health, and the Utah State Hospital. This purview includes more than 1,500 employees and an annual budget of more than $5 billion.

For more than 20 years, Dr. Strohecker has been a practicing pharmacist in a variety of settings, including the senior pharmacy director with a Medicare and Medicaid managed care plan and as full-time adjunct professor with a college of pharmacy. Dr. Strohecker has a bachelor's degree in pharmacy from the Philadelphia College of Pharmacy and Science and Doctor of Pharmacy from the University of Florida. Additionally, Dr. Strohecker participates in semi-annual medical mission trips to the Camotan Clinic in Camotan, Guatemala, which provides free medical and dental care.

Emily Tsiao, PharmD, BCPS
Premera Blue Cross

Emily Tsiao, PharmD, BCPS is the Medical Policies Clinical Pharmacist at Premera Blue Cross, where she leads drug clinical policy development across the medical and pharmacy benefit for over 2 million members. She has experience overseeing drug utilization management strategy, drug pipeline surveillance, and clinical programs.

Dr. Tsiao earned her Doctor of Pharmacy degree from Auburn University and completed a postgraduate managed care pharmacy residency with Premera Blue Cross. She is also a Board Certified Pharmacotherapy Specialist (BCPS). Dr. Tsiao has been an active Academy of Managed Care Pharmacy (AMCP) member since 2015. She previously served as President of the Northwest AMCP Affiliate and was awarded the 2024 JMCP Award for Excellence. She currently serves as a Clinical Affiliate Faculty member at the University of Washington School of Pharmacy and is a preceptor and mentor to pharmacy students and residents.

Session Overview

In this session, we heard from both a Commercial Payer and a State Medicaid Director as they shared their strategies and initiatives to improve access to care for individuals with sickle cell disease, highlighting current efforts to expand coverage and address disparities in treatment. Details are highlighted below:

In Utah, Medicaid's focus for cell and gene therapies is on pharmacy coverage and reimbursement policies. The state has a robust process for engaging stakeholders, including patients, providers, managed care, manufacturers, and regulators and is focused on serving individual member needs.

Payers focus on formulary review considerations, and engage physician experts and patient organizations as well as consulting research and literature as well as ICER reports, where available. Payers are able to incorporate the patient perspective through various engagement channels, including Pharmacy & Therapeutics (P&T) Committees, Care Management Patient Experience Education, and ICER report utilization. Specific considerations related to SCD gene therapies include significant travel needs and fertility preservation coverage.

Session 4 - The CMS Cell and Gene Therapy Access Model: Transforming Sickle Cell Disease Treatment

Speaker

Elizabeth Hassett
Leavitt Partners

Elizabeth Hassett is an Associate at Leavitt Partners, based in Washington, D.C., and through her work, an Advisor to the Sickle Cell Disease Partnership. At Leavitt Partners, Elizabeth supports clients and multistakeholder alliances by managing projects and providing strategy on health policy issue areas related to rare diseases, access to treatments, drug development and pricing, and pharmaceutical supply chain management.

Before joining Leavitt Partners, Elizabeth was a Law Clerk at the Centers for Medicare and Medicaid Services in the Provider Reimbursement Review Board, where she focused on Medicare Part A reimbursement regulation and appeals. She additionally served as a Law Clerk at the Maryland Office of the Attorney General for the Maryland Department of Health. Elizabeth received both her MPH in Health Policy and Bachelor of Arts in Political Science from the University of Pittsburgh. She is currently completing her law degree at the University of Maryland, where she is specializing in health law and policy.

Session Overview

This session provided an in-depth overview of the CMS Innovation Center's (CMMI's) Cell and Gene Therapy (CGT) Access Model, highlighting how it aims to enhance access to cutting-edge treatments for SCD, while addressing challenges related to affordability, implementation, and equitable care delivery. Elizabeth Hassett, an Associate at Leavitt Partners and advisor to the SCD Partnership, discussed the specifics of how the CGT Access Model functions as well as the policy implications that have been identified by the SCD Partnership.

Elizabeth explained that participation in the Model is optional for both states and manufacturers, reiterating that states can still apply to participate, and then addressed the eligibility requirements for the states, manufacturers, and beneficiaries. To be eligible, states must respond to a request for applications and execute an agreement with CMS. States are required to include Medicaid beneficiaries in both fee-for-service and managed care plans, with full inclusion of CHIP populations operating under Medicaid. Inclusion of beneficiaries in separate, standalone CHIP programs is optional, and states may also choose whether to pursue federal funding to support participation. Manufacturers, on the other hand, must market FDA-approved or licensed gene therapies for the treatment of severe SCD and participate in negotiations with CMS. Beneficiaries must meet clinical criteria to be eligible and receive therapy through either a participating Medicaid program or a CHIP that is part of the Model.

Elizabeth discussed how the CGT Access Model is guided by several overarching goals, including securing access to high-value therapies, promoting equitable delivery of care, reducing Medicaid expenditures, and expanding support services for beneficiaries. It also seeks to reduce the administrative burden on states and providers while establishing greater consistency in how therapies are accessed across different programs. However, several challenges and limitations were noted. Since the Model is still in its early stages, it is too soon to draw definitive conclusions about its long-term effectiveness. Access to therapy is not guaranteed for all eligible patients, and many states may still lack the clinical infrastructure needed to support successful implementation. Disparities between public and private insurance systems, regulatory concerns such as anti-kickback statutes, and limitations in fertility preservation services further complicate the landscape.

The session concluded with a Q&A moderated by Kelly Hawthorne. During this discussion, CMS's approach to data collection was clarified: manufacturers will report sales data, which will then be cross-referenced with state claims data. Questions around equitable access revealed that much of the responsibility for addressing disparities will fall to states, guided by cooperative agreements and requirements negotiated with manufacturers. It was also confirmed that the terms of the outcomes-based agreements (OBAs) will eventually be released to the public, increasing transparency and accountability.

Session 5 - Breaking Barriers: My Experience with Accessing New Sickle Cell Therapies

Speakers

Jimi Olaghere
Community Member

Jimi Olaghere is a gene therapy recipient who has developed a keen interest in the advancements of cell and gene therapies, since his transformative participation in the groundbreaking clinical trial. He is passionate about the positive impact these therapies can have on patients in need and has become a staunch advocate for increased accessibility of gene therapies for SCD patients worldwide.

Session Overview

In this session, we heard from a person with SCD as they share their personal journey of accessing groundbreaking cell and gene therapy for sickle cell disease, highlighting the challenges, successes, and the transformative impact of these innovative treatments on their health and life.

Session 6 - Building Knowledge Through the Community: The Consolidated Learning System for Sickle Cell Gene Therapy

Speakers

Nikki Braccio
Center for Medicare and Medicaid Services Innovation Center

Nicole "Nikki" Braccio, PharmD, serves as the learning system lead for the Cell and Gene Therapy (CGT) Access Model at the Center for Medicare and Medicaid Services Innovation Center (CMMI). In this role, Nikki designs and implements learning communities to support model participants and partners with improving equitable access to cell and gene therapies for people living with sickle cell disease. Prior to joining CMS, Nikki served as the policy director for National Patient Advocate Foundation and prior to that, advised life science clients with coverage and access policies at Avalere Health. Nikki began her career as a pharmacist in the community setting – practicing in New York and New Jersey. She earned her Doctor of Pharmacy degree from Rutgers University.

Session Overview

This session explored the community learning system within the CMS Innovation Center's Cell and Gene Therapy (CGT) Access Model, highlighting how collaborative networks and shared knowledge are advancing the implementation and expansion of gene therapies for sickle cell disease (SCD).

The goal of the CGT Access Model's learning system is to support the rapid and equitable adoption of effective payment and care delivery models that reduce health disparities and improve health outcomes and patient experiences. The team is working to build a learning system grounded in the priorities of the SCD community, while also encouraging state participation and identifying barriers to engagement. The model is seen as a tool to foster stronger relationships between states and the SCD community.

The learning system will support participants in integrating promising strategies and best practices to increase access to CGTs and improve outcomes for individuals living with SCD. This will be achieved through peer-to-peer and expert-led support such as webinars, case studies, affinity groups, participant spotlights, toolkits, and tip sheets.

As the team continues to develop the system, they are exploring questions such as:

- How can community-based organizations (CBOs) partnering with states best support patients in overcoming barriers to care at treatment centers?
- What types of collaborations exist between providers and CBOs to ensure patients' holistic needs are being met, and how can we ensure best practices are being shared across sites?
- What feedback are providers and treatment centers receiving from patients and caregivers regarding their experiences with gene therapy? Such input is vital for identifying necessary changes to ensure equitable care.

Nikki emphasized the importance of the SCD community building relationships with state Medicaid agency staff and continuing to have conversations with care teams about the potential of gene therapy in treating SCD. She also encouraged ongoing feedback from patients and families about their care experiences.

The team is currently in the design phase of the learning system, and there is still time to shape it to best serve participating states, providers, and community partners.

Session 7 - Navigating Coverage Gaps: The Critical Role of CBOs in Sickle Cell Healthcare

Linda Thomas Wade *Sickle Cell Association of Texas Marc Thomas Foundation*

Linda Thomas Wade is the CEO and President of the Sickle Cell Association of Texas Marc Thomas Foundation (SCAMTF). The SCAMTF was founded in 1997 by the late, great Pastor Marc Thomas, a sickle cell warrior, and his wife Linda. Pastor Marc lived with severe sickle cell disease (SCD) which took his life too soon at the age of 46. Pastor Marc never stopped believing families living with SCD needed a strong voice to raise awareness of the disease and the suffering it causes. Linda and their daughter, Alysian, have dedicated themselves to continuing his legacy and mission and have built a thriving organization with 10 staff members and 7 board members. The SCAMTF offers comprehensive support services to people living with SCD in the State of Texas including certified case management, weekly support groups, counseling, care coordination, medical home placement, youth camps, transitional services, bilingual services, adult retreats and programming, limited financial assistance, referrals, health fairs and sickle cell trait screening. The SCAMTF has received local, national and international recognition over the years.

Tabatha McGee
Sickle Cell Foundation of Georgia

Tabatha McGee is an innovator, a servant, a leader, and a woman whose strong faith has carried her through trials and triumphs. As the Executive Director of the Sickle Cell Foundation of Georgia, Inc. and Founder of the Sickle Cell Sanctuary Wellness Center - the first and only holistic center in the nation for sickle cell patients - Tabatha is tireless in her mission to ensure the highest quality of life and hope for sickle cell warriors and others with life-threatening blood disorders.

After graduating from high school in College Park, Georgia, Tabatha opted to join the U.S. Army, beginning a professional career in service. She excelled as one of the few women paratroopers in the 82nd Airborne Division at Fort Benning, Georgia. Following her military service, Tabatha found her niche in the technology field, settling in Maryland and making significant contributions. During her tenure at Xerox, Tabatha honed her leadership skills, which were crucial preparations for her future path. In 2015 Tabatha returned to Atlanta where she became a caregiver for her mother over the next eight years, a role that tested her strength and resilience. She battled back from both a heart attack and a stroke to vibrant health. She advocates a healthy lifestyle for sickle cell warriors and her employees. Tabatha's drive and determination took her from Director of Administration to Executive Director of the Sickle Cell Foundation of Georgia, Inc.

Ginger Davis
Sickle Cell Thalassemia Patients Network

Teresa Ginger Davis has been a life-long health and education advocate, and spokesperson for sickle cell disease. She has more than 25-years of experience in the health industry. Through Sickle Cell Thalassemia Patients Network (SCTPN), Ms. Davis applied her biology and naturopathic health science education towards working with twenty New York City hospitals on Health Resources and Services Administration (HRSA) grants to deliver care coordination and transitions services for pediatric hematology clients and their families, also care coordination and referral to needed services for adults lost to care after transitioning from pediatrics. On June 29, 2024, Ginger was bestowed an Honorary Doctorate of Humanities by the Anointed by God Ministries and Seminary for lifelong advocacy for education, health, youth mentorship, and community service. Ginger is now leading SCTPN as its newly elected president.

Session Overview

This session explored the critical role of community-based organizations (CBOs) in addressing coverage gaps and health disparities faced by individuals living with sickle cell disease (SCD). Panelists shared how CBOs are driving community-centered solutions to improve access to care, advocate for patients, and build trust where traditional healthcare systems have often fallen short.

The **Sickle Cell Association of Texas Marc Thomas Foundation** highlighted how CBOs serve as trusted intermediaries between patients and healthcare providers, which is particularly important given the discrimination many SCD patients face in clinical settings. The organization offers wraparound services such as transportation, meals, childcare, mental health support, and assistance navigating insurance and social services, helping patients and caregivers manage the demands of treatment, including gene therapy.

The **Sickle Cell Foundation of Georgia** emphasized a community-centered approach that includes deploying community health workers, advocating with insurers, supporting access to financial assistance, and fostering strong provider relationships. Their work also includes community education, promoting clinical trials, and forming coalitions to amplify the voice and needs of the SCD community.

The **Sickle Cell/Thalassemia Patients Network** focused on transitional care and coordination for adolescents moving into adult care. They support patients and families through education, physician referrals, advocacy, and connection to critical services addressing social determinants of health. Their efforts are grounded in cross-sector collaboration with healthcare systems, policymakers, and other CBOs to create sustainable, equitable solutions.
Together, these organizations demonstrate how CBOs are essential partners in the healthcare ecosystem, building trust, removing barriers, and ensuring individuals with SCD receive the care and support they need.

Conclusion

With the continued development and increased availability of treatment options for SCD, it is important to continue the discussions around coverage and access to treatments for people living with SCD. Sick Cells looks forward to hosting this Summit annually to raise awareness, drive collaborative efforts, highlight advances, and address gaps in access and coverage for SCD treatments.

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