SICK CELLS THERAPEUTICS CONFERENCE

"Therapeutics Unlocked"

SUMMARY REPORT

A LETTER FROM OUR CEO

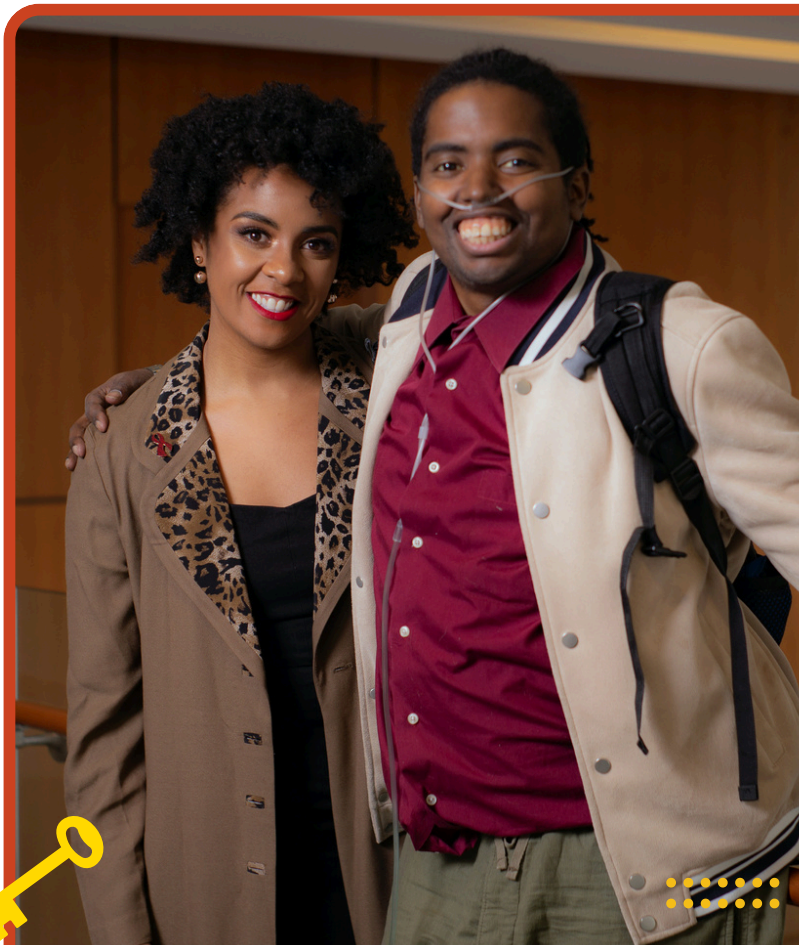
We are proud to host our inaugural Therapeutics Conference, "Therapeutics Unlocked." Many of you know my late, great brother, Marqus, the co-founder and inspiration for Sick Cells. Growing up in the 80s and 90s, the options available to him were limited.

The landscape is changing. This didn't happen overnight. Real innovation in medicine takes at least ten years and more than $1 billion in investment. That's a decade of trials, learning, refining, and listening to the sickle cell community. A decade of scientific persistence and community advocacy intertwined.

If there is one message to echo, it is this: continued advocacy and scientific progress are essential to ensure that every person living with SCD — every genotype, every age, every background — has access to treatments that truly meet their needs. Innovation alone is not enough. Access, equity, education, trust, and community partnership must grow with it. That requires all of us — clinicians, researchers, families, industry partners, policymakers, and people living with SCD — working together, sharing honestly, and being active pieces of the solution.

To every attendee who lent your voice, your expertise, your story, or your presence: thank you. You made this first event a space for education, for connection, and for possibility. I am deeply grateful. This is just our beginning, but it is built on decades of lived experience and a future we are determined to shape together.

With gratitude,
Ashley Valentine
Sick Cells Co-Founder & CEO



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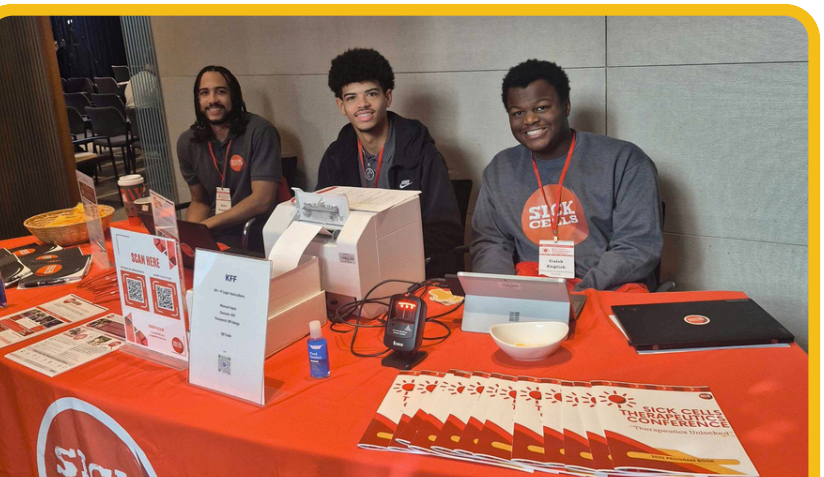


WHO WE ARE

Sick Cells is a national sickle cell disease (SCD) advocacy nonprofit founded in 2017. 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.

Sick Cells is widely recognized for our leadership in multi-stakeholder education, advocacy, and storytelling. Through cornerstone programs such as the Faces of SCD Storytelling Program and the Ambassador Program, along with multi-stakeholder convenings that include the annual Coverage for SCD Summit and the 2020 Roundtable titled "Taking Action on Improving Quality of Life for Individuals with Sickle Cell Disease," we bring lived experiences to the forefront. These insights inform clinicians, researchers, payers, policymakers, and industry leaders about the real-world impact of SCD.

Sick Cells continues to serve as a trusted convener and educator across the healthcare ecosystem, ensuring that the community voice remains at the center of therapeutic development, policy discussions, and systems-level changes.

NOV 12-13, 2025 WASHINGTON DC

OVERVIEW

325
Registrants

174
Virtual attendees

57
in-person attendees*

Our first ever Sick Cells Therapeutics Conference was **hosted on November 12 & 13, 2025, in Washington, DC** — with a virtual option for those joining remotely.

This year's theme was "Therapeutics Unlocked" as we aimed to empower the sickle cell community with knowledge about existing and emerging treatment options, the value of and safety in clinical research, and policy efforts shaping equitable access to care.

*During the week of the conference, a government shutdown came to an end. Flights were reduced significantly due to staffing shortages, impacting travel for speakers and attendees.

Clinical trials and access to care remain ongoing challenges for those living with SCD. At the same time, clinical trial education and awareness continue to decline across the SCD community. As new gene therapies and disease-modifying treatments progress through research and regulatory approval, **the need for clear, accessible, and trustworthy information has never been greater.**

The Sick Cells Therapeutics Conference was designed to bridge the gap between people living with SCD, caregivers, clinicians, researchers, and industry partners. The conference provided an opportunity to hear directly from clinical trial participants as they reflected on their experiences, learn from industry experts about emerging drug and therapy pipelines, explore opportunities for trial participation, and engage in discussions on policies and regulations shaping access to therapeutics.

Through engaging presentations, interactive panels, and community-driven dialogue, attendees gained knowledge and tools to support informed decision-making, strengthen confidence in the treatment development process, and deepen understanding of how innovation is advancing care for SCD.

SESSIONS

THERAPEUTICS LANDSCAPE & CLINICAL TRIAL OVERVIEW

Speakers

Mike Callaghan, MD
Senior Medical Director, *Agios Pharmaceuticals*

Andrew Campbell, MD
Director of the Comprehensive SCD Program, *Children's National Hospital*

Norris Turner, PharmD, PhD
President & CEO, *Turner Healthcare Quality Consulting, Inc.*

Maia Laing, MBA
Chief Policy Officer, *Sick Cells*
(Moderator)

Session Highlights

- Clear, practical communication is critical
- Informed consent should be thoughtful, not rushed
- Community engagement improves research quality
- Opportunities to rethink trial design and endpoints are necessary

Overview

The conference opened with an overview of clinical trials, which play a vital role in advancing therapies for SCD given the limited number of effective treatments currently available.

The panelists explained the trial process, including safety and efficacy, and highlighted that **clear, practical communication** is essential to helping individuals and families with SCD make informed decisions.

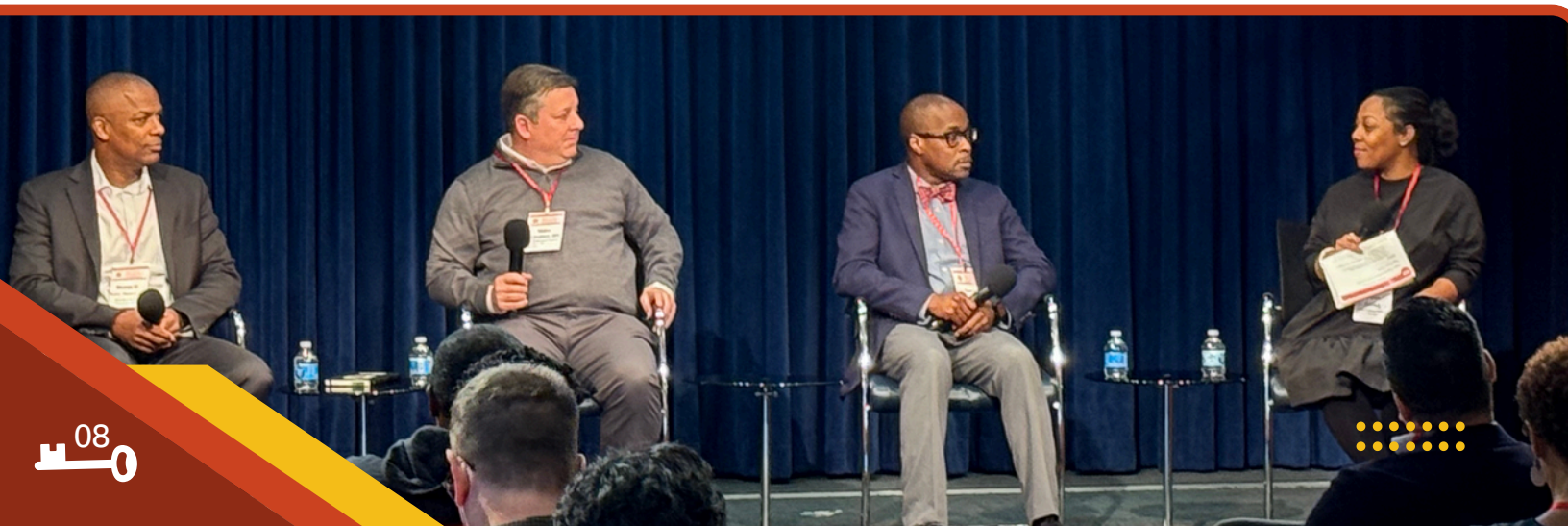
- Providers must communicate the full picture—how trials work, potential risks, realistic benefits, time commitment, and what participation practically looks like.
- Informed consent requires candid, transparent discussion of side effects, uncertainties, and expectations.
- Lack of information can drive hesitancy, and participants need complete information on how a clinical trial can connect with their current regimen.

The panelists also suggested that **informed consent should be thoughtful and not rushed**. Patients should be encouraged to bring support (family members, friend, partner, etc.) into the clinical trial process. Patients should receive information ahead of time, have side effects explained clearly, and be given space to consider participation. Decision-making continues throughout the trial as patients weigh clinical benefit against side effects, burden, and personal goals.

- Navigating the growing therapeutic landscape requires support and often impacts others who are supporting that patient. Patients need care teams they trust.
- Providers play a crucial role through remaining up to date on research, attending conferences, reading current literature, and engaging directly with trial investigators.
- It is important to recognize that clinical trials do add burden (extra appointments, time, monitoring) both for patients and clinicians.

A highly **engaged community of patients, caregivers, and clinicians can drive innovation and quality**. Panelists suggested that the pharmaceutical industry could connect earlier and more often with patients and physicians, as working in isolation can lead to misaligned trials.

- Patients living with SCD should be engaged throughout development. Without this feedback loop, developers and providers miss critical insights.
- To combat status-quo thinking, outreach needs to extend beyond the current circles of participants. Conferences and community forums can help prevent the pharmaceutical industry from working "in a vacuum".



Innovation in SCD research is advancing rapidly, but challenges remain. SCD presents significant variability across genotypes and individuals, making trial design challenging. However, panelists suggested that there are **opportunities to rethink trial design and endpoints.**

- Trials can exclude significant portions of the SCD community, such as those with genotypes SC and Sβ$^+$, or those who experience too few or too many vaso-occlusive crises. These individuals are still experiencing the negative effects of the disease, and this limits who can access new therapies.
- Selecting appropriate endpoints can be difficult with SCD (for example, increased hemoglobin or fewer pain episodes). Selecting trial endpoints and patient criteria influences whether the study can meaningfully demonstrate benefit.
- Some positive effects do not count as formal endpoints. While regulatory limits restrict what the pharmaceutical industry can claim, secondary endpoints and future trials can capture broader benefits.

CLINICAL TRIAL EDUCATION WITH A PATIENT EDUCATOR

Speakers

Brenda Martin, RN, MSN, CPNP
Pediatric Nurse Practitioner and Research Coordinator

Doris Polanco
SCD Community Member

Melissa Akinlawon, MA
Senior Director of Community Programs, *Sick Cells*
(Moderator)

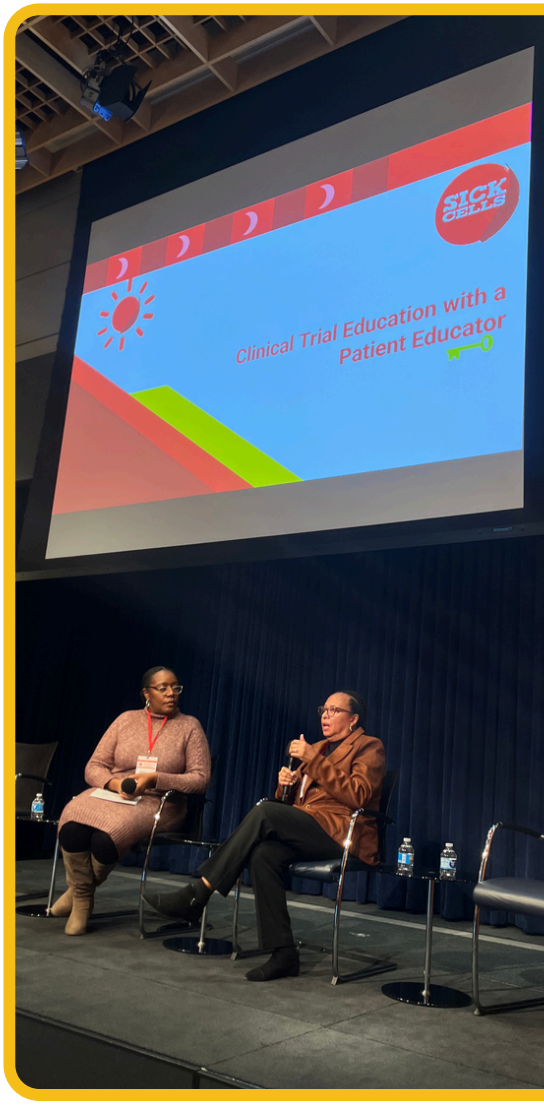
Session Highlights

- Informed consent is a continuous, essential element of clinical trials
- Significant barriers to participation persist, but there are clear strategies to address them
- SCD community members recognize the value of clinical trials

Overview

This session explored the barriers to clinical trial participation, provided clear explanations of how people living with SCD can get involved, and highlighted why participation is meaningful for individuals and the broader SCD community. Panelists noted that, given the limited availability of effective therapies, clinical trials are essential to advancing treatment options for people with SCD. They emphasized that **informed consent is critical to have effective clinical trials and that consent is not a one-time event,** but a continuous, ongoing conversation.

- Because SCD affects the whole family, involving family members and caregivers is critical to understanding trial protocols and navigating multi-year commitments. Participants should be given information well in advance, encouraged to ask questions, and supported in discussing participation with family or caregivers.
- Safety, benefits, and risks must be explained in a clear and accessible manner. Panelists emphasized that safety is a central concern, and noted the importance of communicating strict ethical and regulatory guidelines, as well as independent data monitoring processes, to help reassure participants.
- As clinical trials are often multi-year, information should be revisited throughout the study period. Participants should be clearly informed that they can withdraw at any time without penalty.



People with SCD face well-documented barriers to clinical trial participation, but despite these obstacles, **there are proven strategies that can meaningfully improve participation.**

- Clinical trial websites and search tools are often not user friendly, making it difficult for patients and providers to find relevant studies. Many people living with SCD also lack access to providers who are knowledgeable about SCD, let alone clinical trials. Education, improved trial search tools, and stronger patient-provider relationships can help address these barriers.
- Acute and chronic pain events can prevent participation when research protocols are overly strict or inflexible. Protocols should better reflect the daily, lived experience of people living with SCD.
- Clinical trials must account for the additional personal and financial burdens faced by participants and their families, including transportation, childcare, and missed work or school.

Sickle cell warrior and clinical trial participant Doris Polanco shared her motivation for participating in a study, and emphasized that the SCD community recognizes the value of clinical trials.

- SCD patients are inspired to help fellow warriors, and understand that through participating in clinical trials, even in the placebo group, they are contributing meaningful knowledge that will help future generations.
- Participants understand the value of more frequent and specialized medical attention, combined with access to new or emerging treatments that are not widely available, that trial participation may offer.
- Pharmaceutical companies actively working to develop new treatments for SCD are a powerful motivating force for patients, as is learning about new opportunities from trusted providers.

COMMUNITY EXPERIENCE WITH CLINICAL TRIALS

Speakers

André Harris, MSW
SCD Community Member

Antuan Sartin
SCD Community Member

Jessica Sims
SCD Community Member

Adrienne Shapiro
Caregiver, Founder
Axis Advocates
(Moderator)

Overview

In conversation with community members, this session featured firsthand experiences from three individuals who have participated in clinical trials and/or successfully gained access to an emerging therapy. Participants shared what the recruitment process looks like, reflected on their experiences as study participants, discussed the supports available during participation, and described what happens at the conclusion of a trial. Their key takeaways included:

Trusted provider relationships are critical

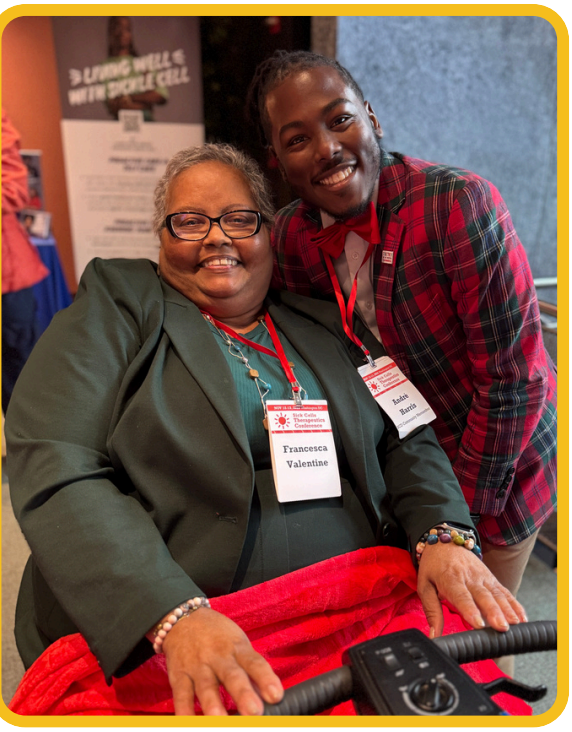
Trust is not a soft concept; it is a clinical necessity. Panelists described years of dismissal or disbelief from medical providers, and shared how finally finding clinicians who "see" them reshaped their willingness to engage. Every participant noted that having providers they trust is what led them to join and remain in clinical trials or to try newly available therapies. Open communication and supportive healthcare teams were identified as essential to building confidence in the research process.

> *I decided to [participate in a clinical trial] because number one, the trust that I had built with my hematologist… [he] would bring clinical trials to us and say that it looks promising, and you would be a great candidate…it's so important to have providers and a health system that you trust.*

Practical and social support matters

Trials require time, discipline, and navigation of logistical challenges. Panelists spoke about the essential role of family, peer networks, community health workers, and patient advocates who help bridge gaps and interpret complex medical information. When patients have help with logistics and emotional burdens, they are better able to successfully engage in research.

> *See if they have case managers that are available for you as a resource that will actually help you go through the process of getting approved…and make sure you have that support system outside of the hospital or the trial whether it be your family or your friends.*



Education and awareness are key

Understanding the full scope of trials, from the expectations to potential side effects, is essential. The healthcare system is already overwhelming and confusing, and panelists stressed the importance of plain-language explanations and culturally competent communication. Patients and their families must be empowered with clear information to make informed decisions and manage expectations over multi-year trials.

> *Ask all the questions that you can possibly think of, even if it's the same question, until you get a clear understanding…just educate yourself and get all the information as you can about these trials, because this is a multi-year journey.*

Participation is a form of advocacy

Clinical trial participants see their involvement as a way to contribute to progress for future generations. Several panelists said they participate not only for themselves but for the next generation living with SCD. By sharing their stories, joining clinical trials, or helping others navigate the system, they contribute to change that benefits the entire community. Participation is framed as a responsibility and as a form of advocacy, underscoring the importance of community engagement in advancing treatments for SCD.

Hope for new therapies drives participation

With limited approved therapies for SCD, participants are motivated by the possibility of new treatments. Panelists spoke openly about wanting options beyond what is currently available and wanting to contribute to the future of sickle cell treatment. Even when participation involves risk or uncertainty, the possibility of better health outcomes fuels engagement.

> *Sickle cell disease shows up in many different ways and with many challenges… realizing how long hydroxyurea has been around and for a while that was the only FDA approved medication for SCD, it was impressed upon me to be a part of the process, and to see where new therapies, new drugs can take us.*

POLICY IMPLICATIONS ON THERAPEUTIC DEVELOPMENT

Speakers

Sara Davis
Vice President, Head of Patient Advocacy & Policy, *Fulcrum Therapeutics*

Annie Kennedy
Chief of Policy, Advocacy, and Patient Engagement, *EveryLife Foundation for Rare Disease*

Josh Trent
CEO, *Leavitt Partners*

Ryann Hill, MPH
Founder & CEO, *Indigo Hill Strategies (Moderator)*

Session Highlights

- The policy environment is more dynamic than ever, due to powerful patient advocacy
- Data-driven policy work can dispel fears about the cost of emerging therapies
- Persistent policy barriers still limit equitable access

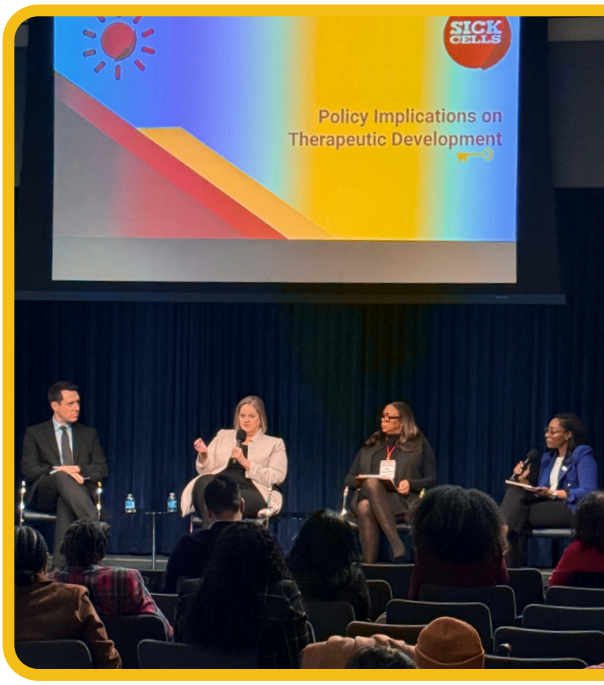
Overview

This session explored how policy, advocacy, and regulatory decisions influence therapeutic development, access, and innovation. Panelists agreed that the **policy environment for rare diseases is more dynamic and favorable than ever, largely because of sustained engagement from patient communities**.

- Rare disease is now central in national conversations, something that was not true even five years ago. This shift is a direct result of sustained pressure and engagement from patient communities.
- Advocates are no longer peripheral; they are fundamentally shaping therapeutic design, regulatory expectations, and access pathways. Advocacy organizations reinforce this through coordinated messaging, coalition-building, and sustained outreach on Capitol Hill.
- The pharmaceutical industry, advocacy groups, policymakers, and community members are more aligned than ever. There is a growing shared accountability for ensuring that new therapies not just enter the market, but actually reach people living with SCD.

Panelists shared that **data-driven policy work can help dispel payer fears about the cost of emerging therapies.**

- New analyses by the EveryLife Foundation show that the fiscal impact of rare disease treatments on Medicaid is minimal, and that future innovation will account for less than 1% of the overall rare disease cost burden.
- Rare diseases already impose high costs, and not investing in effective therapies is the real inefficiency.
- Payers, legislators, and agency leaders often want to help but lack information. Advocates should assume good intent, bring strong data, and build consistent relationships.



The panel emphasized that progress depends on collaboration across sectors and strong relationships with policymakers. **Despite advancements in science, hurdles to access remain.**

- Prior authorization remains overused, and is especially harmful for patients who change insurers or move. Companies must engage payers and regulators early so new therapies do not arrive with built-in barriers.
- More than half of SCD patients rely on Medicaid, but preferred drug lists and Medicaid variability across states create inequitable access.
- The average Medicaid director's tenure is just 18 months. Many policymakers simply lack disease knowledge or context, underscoring the need for clear, persistent education.

HOLISTIC CARE: EXPANDING ON NORMATIVE CARE

Presenter

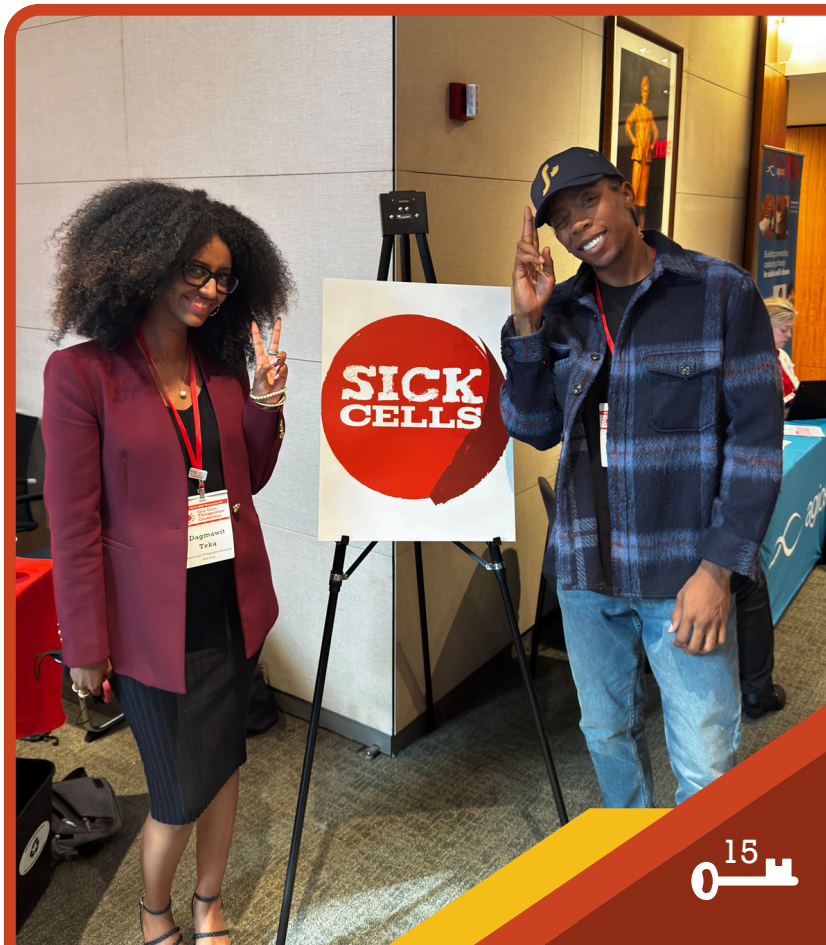
Tabatha McGee
CEO, *Sickle Cell*
Foundation of Georgia

Overview

In a video presentation, Tabatha McGee, CEO of the Sickle Cell Foundation of Georgia, provided an overview of additional care options available to individuals living with SCD. She introduced the Sickle Cell Sanctuary, the first holistic wellness center in the United States designed for individuals living with SCD. Here are her key takeaways:

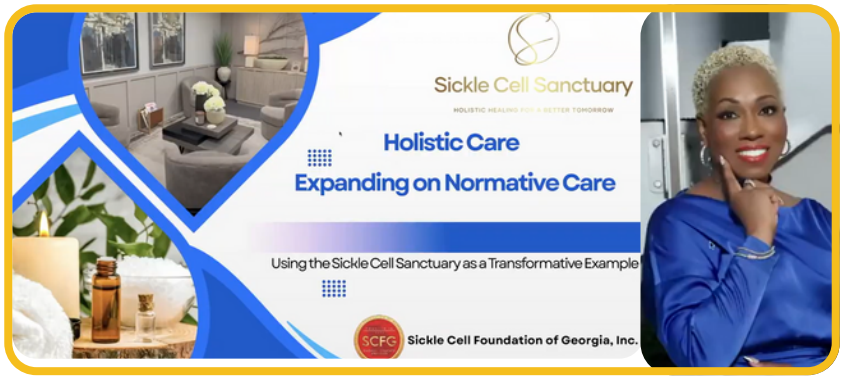
Normative care is not complete care

Traditional health care gives patients the basics like clinical visits, diagnostics and lab work, medication management, acute pain treatment, and emergency care. However, patients continue to carry emotional stress, depression and anxiety, financial hardship, trauma from repeated medical experiences, isolation and a lack of support, and limited access to wellness resources. Normative care manages SCD; it does not address what it means to live with it. True care must extend beyond labs and prescriptions to include the emotional, psychological, and social realities patients face every day.



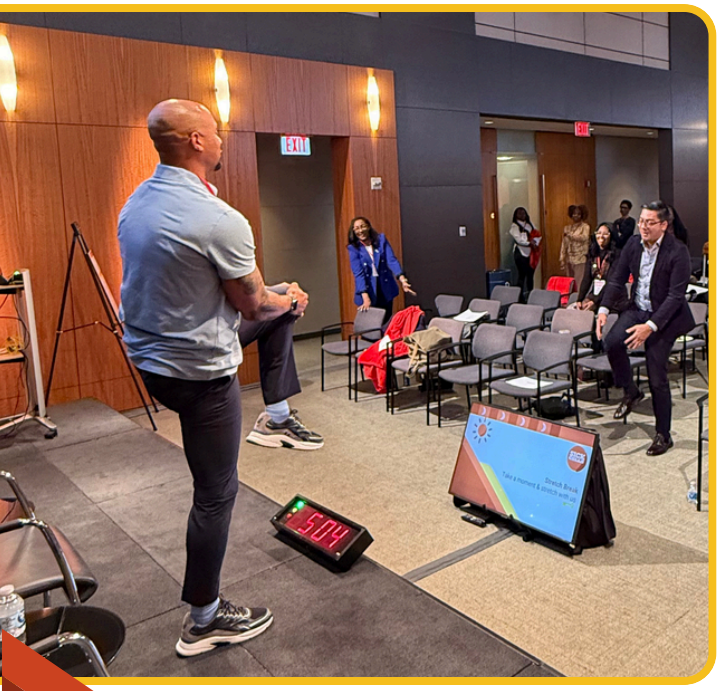
Surviving is not living

Patients are telling us clearly: "I am surviving, but I'm not living." These are not medical complaints – they are human complaints. Holistic care acknowledges that pain, stress, and trauma are deeply interconnected, and that emotional and psychological suffering can escalate physical crises. When patients feel unseen, unsupported, or treated as a diagnosis rather than a person, even the best medical care falls short.



The Sanctuary is a model for holistic wellness

The Sickle Cell Sanctuary provides a comprehensive approach to wellness that complements traditional medical care. The model demonstrates that when supportive services such as mindfulness, mental health care, nutrition, movement therapy, and peer support are woven into care, people do not just feel better; they start living better. Hope, dignity, identity, and resilience return. This model can be integrated into hospitals, qualified health centers, infusion centers, community organizations, school and youth programs.



Holistic care is health equity

Holistic care is essential health care, not a luxury. When stress increases, pain increases; when tension rises, crises rise. Holistic care interrupts that cycle, especially for a community that has long lacked access to the wraparound services other conditions routinely receive. While expanding this nationally requires funding, reimbursement, insurance coverage, and system integration, the Sanctuary proves that it works. Patients at the Sanctuary experience lower pain frequency, better sleep, reduced hospital visits, improved emotional resilience, higher quality of life, and increased hope. Holistic care ensures every person has access not only to treatment, but to peace, stability, and the possibility of living fully.

GETTING INVOLVED: OVERCOMING BARRIERS TO CLINICAL TRIALS

Speakers

Andy Ellner, MD
Co-Founder & CEO, *Quilt Health*

Kim Smith-Whitley, MD
Chief Medical Officer, Real World Evidence, Patient Advocacy & External Collaboration Lead, *Pfizer*

Wally Smith, MD
Director, VCU Adult Sickle Cell Program & Florence Neal Cooper Smith Professor of SCD, *Virginia Commonwealth University*

Maia Laing, MBA
Chief Policy Officer, *Sick Cells*
(Moderator)

Session Highlights

- Expert-guided, patient-centered care is unevenly accessible
- Clinical trial information remains hard to find, and misinformation fills the gap
- Community health workers are a powerful bridge
- Co-creation with patients strengthens clinical trials

Overview

The conference concluded with a session offering a practical, solution-oriented look at how to participate in a clinical trial. The session aimed to demystify clinical trials and address common barriers to getting started. Panelists emphasized that accessing clinical trials and advancing care for people with SCD requires expert-guided, patient-centered support, yet **most patients remain disconnected from specialists due to geographic, structural, and insurance-related fragmentation.**

- Many individuals with SCD do not live near experts, and systems are still built around doctors rather than patients. Bridging geographic gaps remains a major priority.

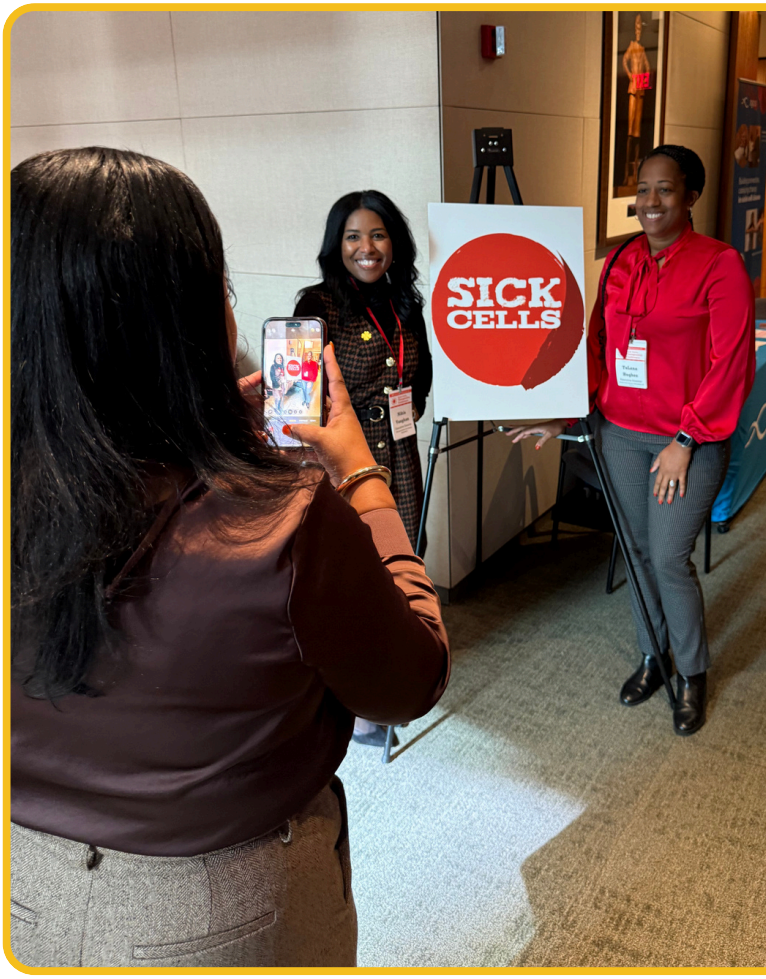
- Because SCD is highly complex, connecting patients with true experts is essential for accurate information, appropriate care, and informed evaluation of clinical trial options.
- Transitions from pediatric to adult care, lack of insurance continuity, and siloed medical data slow progress and leave patients unsupported. Without unified medical records, it is difficult to rapidly match patients to appropriate trials.

The session panelists acknowledged that finding clinical trials remains challenging due to confusing websites and inconsistent information.

- Patients face a flood of online misinformation about therapies and trials. Reliable information sources are difficult to evaluate, and clinicaltrials.gov is challenging for many to navigate. Social media and community networks can be more accessible, but patients need guidance to assess quality and credibility.
- Technology can unlock faster access to appropriate trials and more holistic, compassionate care, but only if systems are redesigned around patient needs rather than institutional convenience.

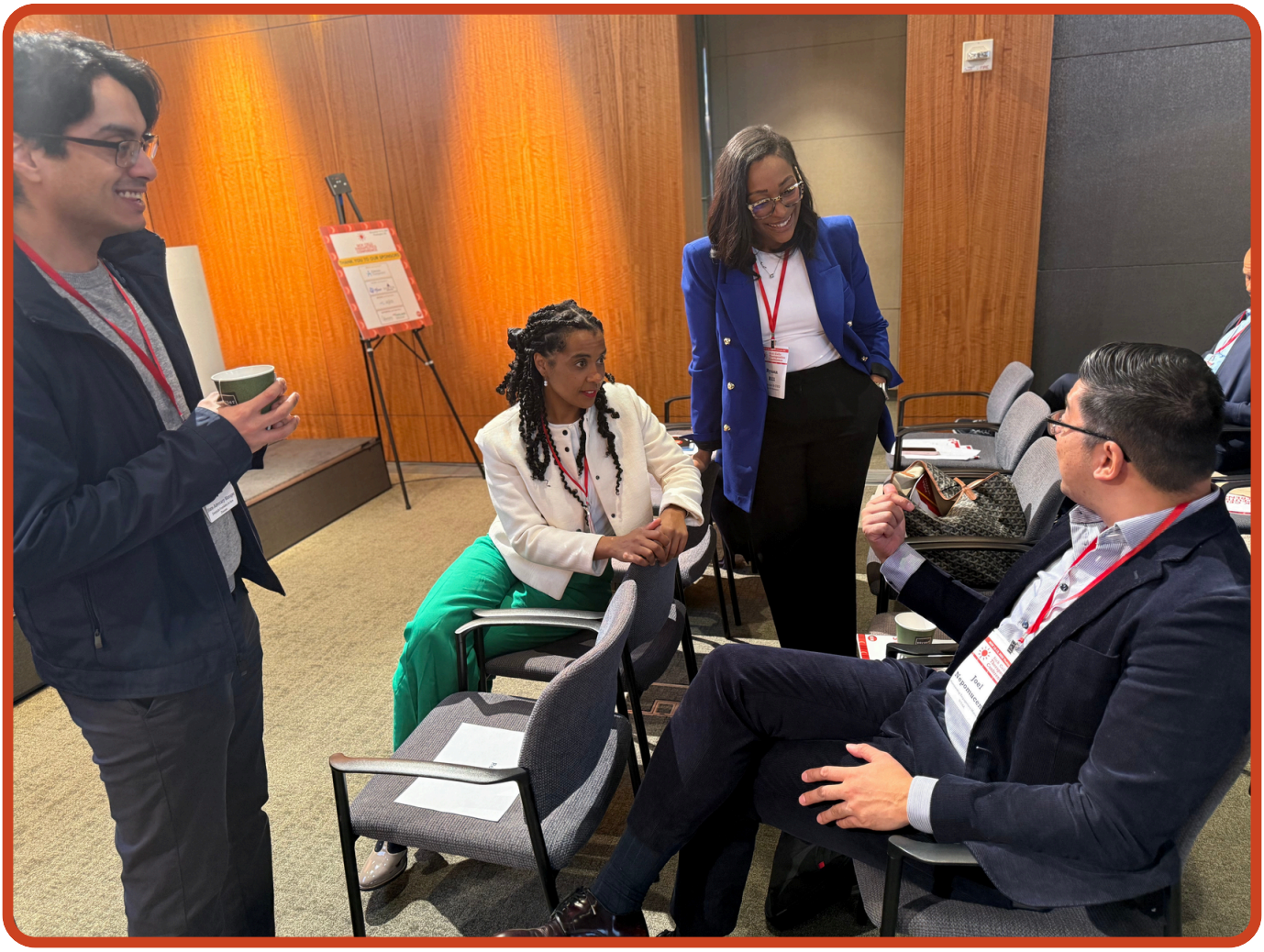
Panelists highlighted the critical role of community health workers in bridging communication gaps, countering misinformation, and helping patients interpret complex medical information.

- Patients frequently leave appointments unsure of what was said, and community health workers can help translate medical language, field questions, and guide navigation across fragmented systems.
- Patients benefit from framing trial decisions around what they want out of them, such as pain relief, higher energy, or willingness to try something more experimental. Community health workers can partner with providers and patients to help individuals with SCD connect realistic clinical options with their personal priorities.
- Peer support, shared experiences, plain-language summaries, and culturally aligned communication are central to clinical trial awareness and self-advocacy.



Panelists emphasized the need to pair high quality, respectful, and compassionate care with high quality, respectful, and compassionate research. To achieve this, all clinical trial stages should be patient centered.

- Patients should help design outcome measures, shape research priorities, and even lead publications. Plain-language summaries and inclusive research teams help ensure that studies reflect what actually matters to individuals living with sickle cell disease.
- Technology must be built around the patient experience, not clinical or institutional convenience. Digital tools alone will not close care gaps, but they can connect patients to experts, support real-time data entry when patients see value, enable simplified trial matching, and help centralize medical records.
- Respectful, compassionate communication is essential to building trust, especially when discussing complex or high-stakes decisions such as enrolling in a clinical trial. Trust in the clinical team is a prerequisite for participation and long-term engagement; patients need assurance that if they become ill during a trial, they will receive responsive and competent care.



SPONSORS

GOLD LEVEL

Fulcrum Therapeutics

Sheinei Alan (M.D., PhD, Director, Inova Adult Sickle Cell Program & Assistant Professor, UVA School of Medicine Inova Campus), Wally Smith (M.D. Director, VCU Adult Sickle Cell Program), and Florence Neal Cooper Smith (Professor of SCD at VCU) provided educational information about Fulcrum's SCD Pioneer study. Learn more at pioneerscdstudy.com

SILVER LEVEL

Pfizer

Aleisha Richards, PharmD, Field Medical Director, provided an overview of Pfizer's SCD pipeline compounds. Learn more at TogetherForRare.com

VERTEX

Shantá Robertson, Patient Educator, offered educational information about Vertex's gene therapy approach for SCD. Learn more at VertexEducators.com and casgevy.com

BRONZE LEVEL

agios

Janie Davis, Director, Global Patient Advocacy, provided educational information about Agios' RISE UP clinical trial. Learn more at agios.com

SUPPORTER LEVEL

Beam THERAPEUTICS

Learn more at beamtx.com

TERUMO BLOOD AND CELL TECHNOLOGIES

Learn more at teruobct.com

APPENDIX

2025 SICK CELLS THERAPEUTICS CONFERENCE AGENDA

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AGENDA

WEDNESDAY, NOVEMBER 12, 2025

03:00 PM - 05:00 PM	**Open Reception** Exhibit booths available for viewing

THURSDAY, NOVEMBER 13, 2025

08:00 AM - 09:30 AM	**Registration** Exhibit booths available for viewing
09:30 AM - 09:40 AM	**Welcome Remarks** **Speaker: Jamie Dornfeld**
09:40 AM - 10:10 AM	**Keynote** **Speaker: Ashley Valentine**
10:15 AM - 11:00 AM	**Therapeutics Landscape & Clinical Trial Overview** **Speakers: Mike Callaghan, MD; Andrew Campbell, MD; Norris Turner, PharmD, PhD; Maia Laing (Moderator)**
11:00 AM - 11:15 AM	**Break**
11:15 AM - 11:30 AM	**Vertex Gene Therapy Overview for Sickle Cell Disease** **Speaker: Shantá Robertson**
11:35 AM - 11:50 AM	**Pfizer Rare Disease-Sickle Cell Disease Pipeline** **Speaker: Aleisha Richards, PharmD**
11:55 AM - 12:25 PM	**Clinical Trial Education with a Patient Educator** **Speakers: Brenda Martin, CPNP-PC, Doris Polanco, Melissa Akinlawon (Moderator)**

AGENDA

12:25 PM - 01:35 PM	**Lunch**
01:35 PM - 02:05 PM	**Community Experience with Clinical Trials** **Speakers: André Harris, Antuan Sartin, Jess Sims, Adrienne Shapiro (Moderator)**
02:10 PM - 02:30 PM	**Exploring New Research in Sickle Cell Disease: Updates from the Pioneer Study** **Speakers: Sheinei Alan, MD, PhD; Wally Smith, MD**
02:30 PM - 02:45 PM	**Break**
02:45 PM - 03:15 PM	**Policy Implications on Therapeutic Development** **Speakers: Sara Davis, Annie Kennedy, Josh Trent, Ryann Hill (Moderator)**
03:20 PM - 03:30 PM	**RISE UP Sickle Cell Disease Clinical Program: Clinical Trials Designed with Patients, for Patients** **Speaker: Janie Davis**
03:35 PM - 03:55 PM	**Holistic Care: Expanding on Normative Care** **Speaker: Tabatha McGee**
04:00 PM - 04:30 PM	**Getting Involved: Overcoming Barriers to Clinical Trials** **Speaker: Andy Ellner, MD; Kim Smith-Whitley, MD; Wally Smith, MD; Maia Laing (Moderator)**
04:30 PM - 04:45 PM	**Closing Remarks**

THANK YOU