SICK CELLS THERAPEUTICS CONFERENCE

"Therapeutics Unlocked"

2025 PROGRAM BOOK

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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 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, policymakers, and industry leaders about the real-world impact of SCD.

We are known for creating inclusive spaces where diverse stakeholders can engage in open dialogue, share knowledge, and collaborate to advance equitable access to care and innovation. Sick Cells continues to serve as a trusted convener and educator across the healthcare ecosystem, ensuring that the patient voice remains at the center of therapeutic development, policy discussions, and systems-level progress.

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www.sickcells.org

engage@sickcells.org

OVERVIEW

Clinical trials and access to care remain ongoing challenges for those living with SCD. At the same time, clinical trial education and awareness have declined 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 is designed to bridge the gap between patients, caregivers, clinicians, researchers, and industry partners. The conference provides an opportunity to hear directly from clinical trial participants as they reflect 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 will gain knowledge and tools to support informed decision-making, strengthen confidence in the treatment development process, and deepen understanding of how innovation is advancing care in SCD.

This conference will be hosted in Washington, DC on November 12-13, 2025.

KEY TAKEAWAYS

During the conference, **tell us about your Key Takeaways** from the event!

Scan the QR code or go to https://forms.gle/NFcguPNXvzR6Sgo48 to submit your Key Takeaways.

THERAPEUTICS UNLOCKED: HANIF'S CLINICAL TRIAL JOURNEY, A BLOG

Sick Cells met with Hanif Mouehla, a Harvard pre-med student and passionate sickle cell advocate, who has courageously shared his story to increase awareness of clinical trials and the promise of cell and gene therapies. Hanif reflects on his experience undergoing a clinical trial at just eight years old, his road to recovery, and his commitment to advancing a future where every Sickle Cell Warrior has access to life-saving treatment.

The Decision That Saved His Life

At eight years old, Hanif experienced a case of acute chest syndrome (ACS) that caused his lungs to collapse. He was put in a medically induced coma for six weeks. During that time, his parents were running out of options and hope, until a pediatric hematology-oncology specialist approached them with a clinical trial: haploidentical allogeneic stem cell transplant. For Hanif's parents, the trial offered hope.

"My parents at that time, they were desperate," Hanif shared. "They were desperate because they had seen their son nearly die more times than any parent should, so they said, 'We need to do this to save Hanif.'"

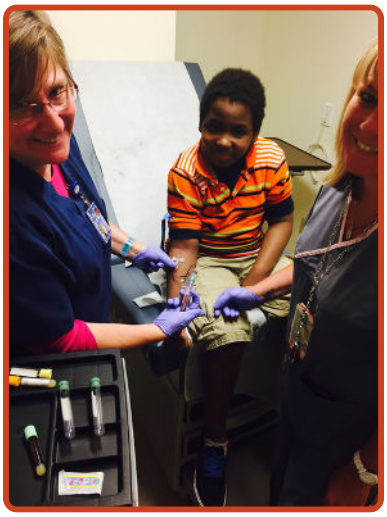
Hanif and his mother

Life before treatment was filled with pain crises, long hospital stays, and frequent absences from school. Beyond the physical challenges, Hanif faced social isolation in environments where few people understood sickle cell disease. This clinical trial represented the turning point his family needed.

Hanif's three siblings were tested for donor compatibility, but none were a match. His medical team then explored a half-match donor option, and ultimately, his mother was identified as the donor. That discovery marked the beginning of a two-year treatment and recovery journey.

Hanif's New Chapter

Hanif remembers his transplant day clearly: September 15, 2014, a date he calls "day zero." After months of transfusions and chemotherapy, the actual procedure lasted only about ten minutes, a surprisingly quick moment following years of pain and months of preparation. While the procedure was brief, the meaning of the day was profound - it marked the beginning of a new chapter, one defined by healing, resilience, and hope.



Hanif with treatment team

young Hanif

A Pioneer and Voice for the Community

Hanif was the seventh patient to participate in this trial. Today, more than sixty Warriors have followed in his footsteps. "I felt like a pioneer for this type of treatment," he said.

But Hanif's journey did not end with his recovery. He has spent over a decade sharing his story and uplifting others navigating sickle cell disease. "There is no right or wrong way to advocate," he says. "I use my story to motivate fellow Warriors. Representation matters, especially for a disorder that deserves much more attention and awareness."

Hanif hopes to one day become a hematologist/oncologist, specializing in sickle cell disease to create a space where patients feel seen, supported, and understood. "I want to say to patients, 'I was in your position. Look at me now, I'm giving back.'"

Hanif's story is a testament to courage, scientific innovation, and the power of community. His story reminds us why expanding access to clinical trials and equitable therapeutic options is essential, and why the future of sickle cell treatment must be shaped alongside those who have lived it.

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)

This session will provide an overview of the current therapeutics pipeline, explain what clinical trials are, outline the phases of clinical trials, and address topics such as accelerated approvals, withdrawals, trial purpose, safety, and a preview of ongoing clinical trials.

11:00 AM - 11:15 AM

Break

11:15 AM - 11:30 AM

Vertex Gene Therapy Overview for Sickle Cell Disease

Speaker: Shantá Robertson

This presentation will offer educational information about Vertex's gene therapy approach for sickle cell disease.

AGENDA

11:35 AM - 11:50 AM	**Pfizer Rare Disease-Sickle Cell Disease Pipeline** **Speaker: Aleisha Richards, PharmD** This presentation will provide an overview of Pfizer's sickle cell disease pipeline compounds.
11:55 AM - 12:25 PM	**Clinical Trial Education with a Patient Educator** **Speakers: Brenda Martin, CPNP-PC, Doris Polanco, Melissa Akinlawon (Moderator)** This discussion will explore barriers to clinical trial participation, provide clear explanations of how patients can get involved, and highlight why participation is meaningful for individuals and the SCD community.
12:25 PM - 01:35 PM	**Lunch** Exhibit booths available for viewing
01:35 PM - 02:05 PM	**Community Experience with Clinical Trials** **Speakers: André Harris, Antuan Sartin, Jess Sims, Adrienne Shapiro (Moderator)** In conversation with community members, this panel will feature firsthand experiences from individuals who have participated in clinical trials along with insight from experts who support trial navigation. Participants will share what the recruitment process looks like, what it is like to be part of a study, what support is available during participation, and what happens at the conclusion of a trial.
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** This presentation will provide educational information about Fulcrum's sickle cell disease study.

AGENDA

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)** This session will provide an overview of the policy and regulatory environment and examine how these systems influence therapeutic development, access, and innovation.
03:20 PM - 03:30 PM	**RISE UP Sickle Cell Disease Clinical Program: Clinical Trials Designed with Patients, for Patients** **Speaker: Janie Davis** This presentation will provide educational information about Agios' clinical trials.
03:35 PM - 03:55 PM	**Holistic Care: Expanding on Normative Care** **Speaker: Tabatha McGee** This presentation will provide an overview of additional care options available to individuals living with sickle cell disease.
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)** This panel will offer a practical, solution-oriented look at how to participate in a clinical trial. Panelists will explain how to find active trials, what the typical enrollment process involves, and how to engage your primary care provider. This session aims to demystify clinical trials and address common barriers to getting started.
04:30 PM - 04:45 PM	**Closing Remarks**

DID YOU KNOW?

A clinical trial is a research study that tests new ways to prevent, detect, or treat disease. These studies may evaluate new drugs, new combinations of existing drugs, medical devices, surgical approaches, or new ways to use existing treatments.

To enroll in a clinical trial, a person can search for a trial they qualify for and speak with their healthcare provider about participation. Individuals may also contact a study site directly to seek more information about enrollment.

Information about active sickle cell clinical trials can be found at clinicaltrials.gov. You can also reach to your local sickle cell community-based organization (CBO) for guidance on trials currently recruiting in your local area.

Not all clinical trials focus on medications. Some trials test other aspects of care, such as strategies to improve quality of life for people living with chronic illnesses.

Clinical trials progress through IV phases, each designed to answer different questions:

- Phase I: Determines whether a medical approach (e.g., drug, diagnostic test, device) is safe, identifies side effects, and establishes appropriate dosing.
- Phase II: Examines whether the medical approach works and continues monitoring for side effects to help design larger Phase III trial.
- Phase III: Confirms whether the medical approach works, compares it to existing treatments, and continues safety monitoring.
- Phase IV: Occurs after a treatment or medical approach is approved for use and monitors long-term effectiveness, side effects, and real-world outcomes.

SPEAKERS

Ashley Valentine, MREs - KEYNOTE

CEO & Co-Founder, Sick Cells

Ashley Valentine, CEO and Co-Founder of Sick Cells, turned her family's experience with SCD into a life of advocacy. With a Master's in Research Methods, she worked in research and policy, helping secure CMS support to reduce ER disparities. Since 2017, she has led Sick Cells, including successfully leading the SCD community in its first-ever ICER review, building coalitions to advocate for federal legislation to be signed into law, testifying at the FDA about the importance of drug development, and more.

Melissa Akinlawon, MA

Senior Director of Community Programs, Sick Cells

Melissa Akinlawon is a communications strategist focused on equity in public health. With 12+ years shaping prevention, chronic disease, and community campaigns, her past work includes NIH Heart Health Education and CDC's Stop HIV Together, plus social media leadership for a primary care provider serving the senior/Medicare population. An award-winning public health leader, she is pursuing a DrPH at George Washington University, focusing on transforming public health storytelling in media to influence and amplify healthy lifestyle behaviors.

Mike Callaghan, MD

Senior Medical Director, Agios Pharmaceuticals

Dr. Callaghan is a Pediatric Hematologist/Oncologist and Senior Medical Director at Agios Pharmaceuticals, working in clinical development on studies of new therapeutics for people with sickle cell disease. He was formerly the Director of the Comprehensive Sickle Cell Center at Children's Hospital of Michigan in Detroit, where he lives and continues to see patients in clinic as a volunteer.

Andrew Campbell, MD

Director of the Comprehensive SCD Program, Children's National Hospital

Dr. Andrew Campbell leads one of the nation's largest sickle cell programs at Children's National, caring for nearly 1,400 patients each year. Inspired early in training, he focuses on closing care gaps through research, advocacy, and global collaboration. His team helped deliver the nation's first pediatric gene therapy treatment after FDA approval in 2023. Dr. Campbell advances inclusive studies and pushes policy to bring better, safer, and more accessible care to patients worldwide.

Janie Davis, MBA

Director, Global Patient Advocacy, Agios Pharmaceuticals

Janie Young Davis brings 25+ years in biotech and pharma and holds an MBA and B.S. in Marketing from Purdue. She has led commercialization and consulting for rare and ultra-rare diseases, with expertise in patient advocacy, brand strategy and tactical execution, new product launches, lifecycle management, managing healthcare professional and patient audiences, sales and storytelling. Inspired by family with sickle cell, she partners with Warriors worldwide to learn about their challenges, obstacles, and achievements globally.

Sara Davis

Vice President, Head of Patient Advocacy & Policy, Fulcrum Therapeutics

Sara Davis is a seasoned advocacy and government relations executive with 20+ years of experience in biotech and pharma. At Fulcrum, she leads efforts to integrate patient perspectives into strategy, development, and policy, with a focus on rare diseases. Prior, Sara held senior advocacy roles at Editas Medicine, CRISPR, and Novartis, where she advanced global engagement and legislative strategies across therapeutic areas. Recognized for her leadership in health equity, coalition building, and Medicaid policy, Sara is dedicated to ensuring that innovation in medicine is guided by and accessible to the patients it is designed to serve.

Jamie Dornfeld, MBA

Chief of Staff and Operations, Sick Cells

Jamie Dornfeld has nearly 20 years in healthcare and nonprofit operations. A mission-driven leader, she builds systems that work for people with complex medical, behavioral, and social challenges. Jamie brings a deep understanding of integrated care models, multi-site operations, and cross-sector collaboration. A strong voice for health equity, she has led efforts to expand access and strengthen services. Jamie holds an MBA from the University of Chicago Booth School of Business.

Andy Ellner, MD

Co-Founder & CEO, Quilt Health

Andy Ellner is a primary care doctor and entrepreneur working to expand access to high-quality care. He is co-founder and CEO of Quilt Health, accelerating access to transformative care for people with complex conditions like SCD. He previously co-founded and led Firefly Health, a virtual-first advanced primary care company with health plan products nationwide. Earlier, he spent a decade on the Harvard Medical School faculty and helped launch the HMS Center for Primary Care and the Program in Global Primary Care and Social Change.

SPEAKERS

André Harris, MSW

Director of Legislative Affairs, Sickle Cell Association of Houston

André Harris is a sickle cell survivor, advocate, and researcher. At the Sickle Cell Association of Houston, he guides the legislative and policy agenda. He serves in several positions with various stakeholders in the sickle cell, rare disease, and HIV/AIDS community. He is currently a fifth-year PhD student in Social Work at the University of Houston where he previously received his Master of Social Work (MSW) with a concentration in Political Social Work. He is a proud member of Phi Beta Sigma Fraternity, where he serves as the National Sickle Cell Director.

Ryann Hill, MPH

Founder & CEO, Indigo Hill Strategies

Ryann Hill is a public health advocate, policy strategist, and Founder & CEO of Indigo Hill Strategies, a DC-based firm focused on healthcare, aging, and disability policy. A Chicago native, she advances equitable policies for marginalized communities. She previously advised U.S. Senator Tammy Duckworth on expanding care access and strengthening disability rights. Ryann holds a Bachelor of Science in Health Policy from Carnegie Mellon University and a Master of Public Health from The George Washington University and is an active member of Alpha Kappa Alpha Sorority.

Annie Kennedy

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

Annie is a veteran leader in rare disease, joining the EveryLife Foundation in 2018. She led the Cost of Delayed Diagnosis study, the National Economic Burden of Rare Disease study, the ICD Code Roadmap, and the Guide to Patient Involvement in Therapy Development. Previously at PPMD and MDA, she advanced major policy, newborn screening, and access efforts. She is a sought-after advisor to patient-centered organizations and initiatives across the nonprofit and government sectors and participates in many advisory committees across the rare community.

Maia Laing, MBA

Chief Policy Officer, Sick Cells

Maia is a healthcare leader and advocate living with SCD. With 25+ years across HHS, UnitedHealth Group, and Optum, she has advanced health equity, access, and value-based care. Her work spans policy, strategy, and data-driven solutions that support people with chronic conditions. Maia holds an MBA from Simmons and continues to champion patient voices and better care for all.

Brenda Martin, RN, MSN, CPNP

Pediatric Nurse Practitioner and Research Coordinator

Brenda Martin is a Pediatric Nurse Practitioner and Research Coordinator dedicated to advancing care for children with SCD. She earned her MS in Nursing from Columbia University, lead and supported NIH and industry studies, and champions research that improves everyday care. Brenda has co-authored numerous publications and received awards for excellence in nursing practice and research.

Tabatha McGee

CEO, Sickle Cell Foundation of Georgia

Visionary leader and advocate for sickle cell warriors, Tabatha McGee is CEO of the Sickle Cell Foundation of Georgia. A former 82nd Airborne paratrooper and tech founder, she turned personal trials—including a heart attack and stroke—into purpose. She opened the nation's first holistic Sickle Cell Wellness Center, securing $2.8 million in groundbreaking grants, and launched Unveiling Sickle Cell: Beyond the Pain. Her mission is to expand services, inspire hope, and empower families.

Doris Polanco

SCD Community Member

Doris lives with sickle cell disease and has experience participating in clinical trials.

Aleisha Richards, PharmD

Field Medical Director, Pfizer

Aleisha Richards has a strong focus on SCD. A pharmacist by training, Aleisha began working in the sickle cell space in early 2021 at Global Blood Therapeutics and has continued her commitment to advancing care at Pfizer. With a passion for improving patient outcomes and supporting healthcare professionals, Aleisha brings deep expertise and real-world insights to the field. She resides just outside Lexington, Kentucky.

SPEAKERS

Shantá Robertson, MA

Patient Educator, Vertex Pharmaceuticals

Shantá Robertson is a Patient Educator at Vertex Pharmaceuticals and a longtime advocate for the sickle cell community. For over 20 years, she has led education and support efforts, co-founding the Maryland Sickle Cell Disease Association and spearheaded the creation of SCD guidelines for Maryland public schools. She has 30+ years in healthcare, including roles in pharma and at the American Society of Hematology. Shantá holds an MA in Organizational Communications.

Antuan Sartin

SCD Community Member

Antuan Sartin is a father, caregiver, and advocate from Louisville. After losing his aunt and uncle to sickle cell, his daughter Kali's diagnosis deepened his mission. Since 2014, his family has worked with the Sickle Cell Association of Kentuckiana, finding support and sharing resources. As a full-time parent, Antuan speaks on care, resilience, and community, helping families face sickle cell together.

Adrienne Shapiro

Founder & Science Administrator, Axis Advocates

Adrienne is the founder of Axis Advocacy and a fifth-generation mother to a child with SCD. A 2018 Stem Cell and Regenerative Medicine Action Inspiration Award honoree, she champions stem cell research and humane care. She has supported Dr. Don Kohn's work at UCLA, advocates for CIRM funding, and serves with Americans for Cures. Adrienne advises the FDA, ASH, and NIH, and works with scientists, caregivers, and patients to improve lives until a cure is found.

Jessica Sims

Senior Manager of Public Affairs, Johnson & Johnson

Jess is a Strategic Communications and Public Affairs leader with experience across government, nonprofits, labor, private sector, and healthcare. She leads public affairs and policy engagement at Johnson & Johnson. Previously at the American Federation of Teachers, she advanced women's rights and childhood nutrition. Previously, she advised the federal government and supported global health at the U.S. Department of State. A former NBC News producer, she holds a BA in Broadcast Journalism and Political Science.

Wally Smith, MD

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

Wally R. Smith, MD is the Florence Neal Cooper Smith Professor of Sickle Cell Disease at VCU and Vice-Chair for Research, General Internal Medicine. A former Scientific Director of the Center on Health Disparities at VCU, he is a leading implementation scientist and focuses on clinical and health services research in SCD. He has 100+ publications, served as an investigator in 50+ grants and contracts, and helped advance two lead compounds for SCD at VCU.

Kim Smith-Whitley, MD

Chief Medical Officer, Real World Evidence, Patient Advocacy & External Collaboration Lead, Pfizer

Dr. Kim Smith-Whitley joined Pfizer after the acquisition of Global Blood Therapeutics (GBT) where she served as EVP, Head of Research and Development. Prior to joining GBT in May 2021, Dr. Smith-Whitley, Professor Emeritus of Pediatrics at the Perelman School of Medicine, University of Pennsylvania, had been the Clinical Director of Hematology and Director of the Comprehensive Sickle Cell Center at Children's Hospital of Philadelphia (CHOP) where her research and clinical work for almost 30 years had centered on SCD.

Josh Trent

CEO, Leavitt Partners

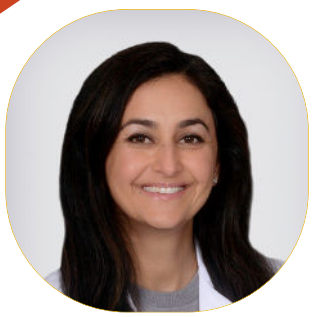
Josh Trent is CEO of Leavitt Partners, a bipartisan health policy and government affairs firm in Washington, DC. He previously served as Chief Health Counsel to the Committee on Energy and Commerce of the U.S. House of Representatives, shaping Medicaid, Medicare, and CHIP in bipartisan laws. He also worked in the White House and HHS under President George W. Bush and advised a U.S. Senator. Josh has served on multiple boards and advisory committees.

Norris Turner, PharmD, PhD

President & CEO, Turner Healthcare Quality Consulting, Inc.

Dr. Norris Turner is a clinical pharmacist and pharmaceutical scientist with 20 years in biopharma at Pfizer, Purdue Pharma, and Johnson & Johnson. He led work in clinical development, medical affairs, and market access, and completed a two-year health policy program. He has deep experience across payers, providers, pharmacy, and government, including CMS, DHA, and ONC. As founder of THCQ Consulting, he helps biopharma deliver real value to customers and patients.

SPEAKERS



Sheinei Alan, MD, PhD

Director of Clinical Research in SCD, Inova Schar Adult Sickle Cell Center

Dr. Sheinei Alan earned her MD and PhD from Virginia Commonwealth University, followed by an internal medicine residency at Georgetown University. She leads care for 250+ adults and as principal investigator for multiple cutting-edge clinical trials in SCD, she leads both industry-sponsored studies of novel therapeutic approaches and an internally funded investigation into cardiac complications in this patient population. She also helps set national care guidelines through the National Alliance of Sickle Cell Centers.

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HONORING MY BROTHER'S COMMITMENT TO INNOVATION, A BLOG

In 2016, Marqus and I found an NIH study for sickle cell disease and leg ulcers. We came across the study through my carpool friend who worked at the National Institutes of Health (NIH). They remembered that I had a brother with sickle cell and brought me information as we exited the metro.

Marqus and Ashley traveling from FDA

I called Marqus to explain the protocol and he jumped at the opportunity. By this point, Marqus' leg ulcers had been open for three years. He was getting them debrided frequently, skin grafts, combination creams, and repeat infections. The open skin got so bad at some points that it was nearly to the bone. The pain was so intense that it would prevent him from walking. We would try anything to contribute to science to not only help Marqus, but to also prevent others from ever having to go through this type of pain.

Part of the study protocol included a wash-out period. That meant that Marqus needed to go off his medications to manage the leg ulcers so that the sample could be fresh. In the end, that led to Marqus' developing an infection and being hospitalized. He could not enroll in the study. What upset us both the most was that he wouldn't be included in science nor could we try a new form of treatment that could potentially help the leg ulcers. He recovered though and we continued to look for more trials and studies.

Francesca, Marqus' mother, caring for his leg ulcer

Marqus' first dose of a new medication

New Treatments Provided Hope: With every new therapy that was approved, Marqus was first in line to access the treatment. We would track the trials via conferences, webinars, and engaging with the companies. When he got his first prescriptions, he wouldn't fill them immediately. He followed our at-home protocol, of course created by our nurse mom. The steps went like this:

- Read entire packet insert online to understand the risk and benefits
- Start new drug on a Tuesday, during work hours, never after work or on weekends, just in case we had to call his provider
- Fill prescription
- Take first dose when someone else was home

When the new sickle cell treatments got approved - 2017 and 2019 - Marqus even framed the prescriptions to remember the moment. In his lifetime, he never waited for sickle-cell specific treatments to be developed. To our family, new treatments, clinical trials, innovation, however you want to call it, were all steps in the solutions, mitigating the complications of sickle cell disease and ensuring a better future for the next generation. For most of our lives, there hadn't been any investment or progress focused on treating sickle cell disease, rather than just managing the complications.

My Hero: Marqus passed away June 20, 2020, in the height of the COVID-19 pandemic. His funeral was restricted to just Illinois residents and had to abide by a laundry list of rules outlined to slow the spread of the dangerous virus. Even in his passing, he still signed up to donate his organs to donor banks and committed himself to science. He awed me even in that moment.

Young Marqus holding Ashley

A Glimmer of Hope: During the first pandemic winter, I sat at my parents' home most days and watched the death toll on the side of the screen increase. I watch the virus run rampant through the sickle cell community, getting calls weekly about another sickle cell warrior, family member, family friend, or colleague that were taken by the virus.

Then in 2021 clinical trials started for covid vaccinations. Those vaccinations offered a glimmer of hope that we could get out of the pandemic and save lives. I thought, what would Marqus do? He would be part of the solution. He would be part of innovation. I enrolled into the Novavax PREVENT-19 trial.

Ashley taking a covid test during clinical trial

My First Clinical Trial: To find the trial, I searched clinicaltrials.gov. I saw openings for two Novavax trial sites in and around Chicago. I called the first site to inquire about enrollment. I chose that site because it was suburban, had a parking lot, and was more isolated from people (think COVID times…Ahh! People!) The study coordinator told me I wasn't qualified, which I knew to be untrue. I knew that the COVID trials were enrolling participants that matched the United States population and that generally finding trials creates barriers to minority populations.

After getting denied from the first trial site, I called the second trial site, which became my second home. The trial coordinator ran me through eligibility and enrolled me into the trial. They sent me my enrollment package which I reviewed with a few nurse friends who worked on clinical trials. The entire enrollment screening took two days and then I had my first appointment.

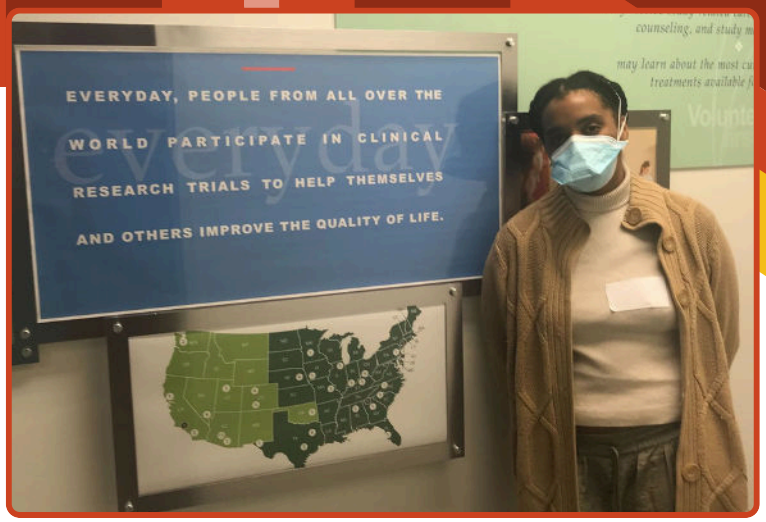
Trial Day: The trial was a double blind, placebo trial and eventually had a crossover study. That meant, the first two doses of vaccination had a cohort that got saline instead of the drug. After the initial data was collected, due to the severity of COVID-19 and need for vaccination, all participants received another two doses. The participants who received the study drug got saline and the participants who received saline got the study drug.

Ashley participating in clinical trial

The visits took 3 hours each. I was always impressed at the number of people who looked like me at the study. It debunked any and every myth that minorities do not enroll in clinical trials. We were all there. Some days I asked other participants why they enrolled. One participant told me that they wanted extra income and were out of work. Another participant told me that they were curious about trial participation. A third participant told me that they wanted to hurry up and get a vaccine and this trial was the fastest way to do that.

I had a total of 5 visits and virtual follow up for a year. During each visit, they would do the following:

- Review the study protocol
- Review of our e-diaries
- Conduct a doctor's appointment
- Test us for covid
- Test women for pregnancy
- Draw many tubes of blood
- Issue study drug



Quote on wall at clinical trial site 'Everyday, people from all over the world participate in clinical research trials to help themselves and others improve the quality of life.'

After we received the study drug, we had to wait a half hour to ensure we did not have a severe reaction. I got excited for each appointment. I felt like I was being part of the solution. I was actively doing something to make others' lives better. I also was protecting my family, protecting myself, and honoring my brother's commitment to innovation.

A Family Affair: That feeling was not mutual however. My parents were nervous. Given that we had just lost Marqus less than a year prior and the ongoing pandemic, my participation in a clinical trial was nerve wracking. Regardless, nerves aside, my parents understood my desire to participate.

Marqus and Ashley, 2019 Red Cross Blood Service Heroes

I suspect that I received the study drug for the first two shots because I had side effects. One side effect was extreme fatigue and a fever. Whenever I awoke from sleep, I found a parent hovering over to check that I was breathing. This was a reminder to me that clinical trials and the decision to enroll in them is a family affair. My dad and sometimes both parents drove me to the appointments. They waited in the car to avoid entering the facility. They cared for me during the side effects and even reminded me of side effects when it came time to complete my e-diary. Together, we got me through that trial.

In the end, the Novavax vaccine was approved under emergency authorization in 2021 and received full approval in 2023. Since then, I have enrolled in natural history studies. I donate blood and help others understand the clinical trial process outside of my work at Sick Cells. As Marqus said, innovation is the future. *We all must be part of the solution.*

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Fulcrum Therapeutics

Who We Are

Fulcrum Therapeutics was founded in 2015 in Cambridge, Massachusetts, US, with the aim of developing disease modifying treatments that result in meaningful outcomes for patients, caregivers, families, and medical teams

We are a group of committed and passionate professionals working in a culture of trust and transparency to enable our employees to do their best work

We take great pride in being purposeful patient partners who do this work not just for patients but with patients

Our Community Engagement Mission

Cultivate relationships with the patient, caregiver, and advocacy community founded on trust and respect

Develop an active presence in patient communities and professional societies

Partner with the patient community to support disease state education, access, and advocacy activities

Incorporate the patient voice in Fulcrum's work and represent Fulcrum in the patient community

Small Molecule Pipeline Across Multiple Rare Diseases

Indication	Asset / Mechanism of Action	Preclinical	Phase 1	Phase 2	Phase 3
Clinical Programs					
Sickle Cell Disease	**Pociredir** (HbF Induction)				
Discovery Programs					
DBA & Other BMFS					
Novel HbF Inducers					
Fibrotic Disorders					
Undisclosed Program					

DBA: Diamond Blackfan Anemia; BMFS: Bone Marrow Failure Syndromes

VERTEX PATIENT EDUCATORS™
EDUCATE • CONNECT • EMPOWER

MEET THE VERTEX PATIENT EDUCATORS

OUR COMMITMENT

Vertex Patient Educators are committed to providing education about sickle cell disease (SCD) to help empower people living with this condition to have meaningful conversations with their healthcare providers.

Visit our booth to:

- **Connect with** the Patient Educators and other members of your community
- **Learn more about** sickle cell disease and gene therapy
- **Get helpful tips** for talking to your healthcare provider

Vertex Patient Educators are not able to provide medical advice. A patient's healthcare team is always the primary source for information related to their health and treatment.

FIND US AT

Sick Cells Therapeutics Conference
KFF Barbara Jordan Conference Center
1330 G St NW Washington, DC 20005
November 13, 2025

Visit our booth and learn more about what we do at VertexEducators.com.

Follow us:
@vertexeducators

ADVANCES IN SICKLE CELL

- In 2017, the FDA approved L-glutaminen (Endari) for patients 5 years and older to reduce sickle cell-related complications. This was the first approved drug for sickle cell disease in almost 20 years.
- In 2019, the FDA approved crizulizumab and voxoletor for sickle cell disease, marking a major milestone for the community.
- In 2023, the FDA approved two milestone treatments, exagamglogene autotemcel (Casgevy) and lovotibeglogene autotemcel (Lyfgenia), representing the first cell-based gene therapies for the treatment of sickle cell disease in patients 12 years and older.

Every patient story shapes our science.

Every rare disease community has a voice, and we're listening. Because by truly understanding, we're able to develop and deliver innovative medicines that have the potential to transform lives.

Connect with us at www.agios.com

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The person shown is a real patient who has been compensated for their time.

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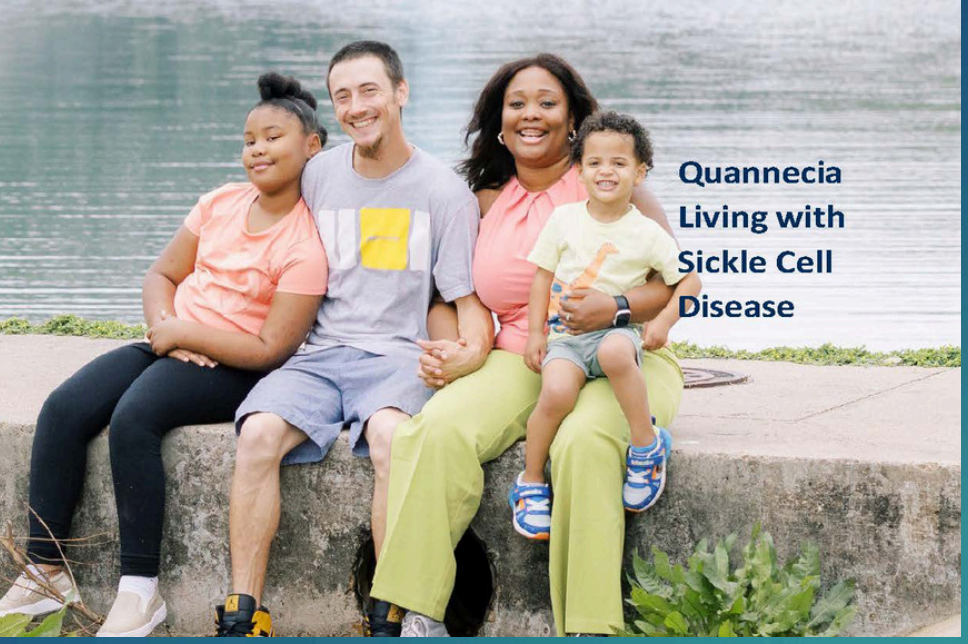
Fueled by Connections to Transform Rare Diseases™



Breaking new ground to advance science and potentially change lives with investigational precision genetic medicines.

Beam Therapeutics is proud to support the Sick Cells Therapeutics Conference.

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With your gift, Sick Cells can continue to provide impactful programming, including the 2025 Sick Cells Therapeutics Conference, and continue elevating the voices of the SCD Community. All funds received directly support our mission and the work needed to advance it.

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