SUMMARY REPORT

Sickle Cell Disease and Gene Therapies: A Two-Part Webinar Series

U.S. Department of Health & Human Services (HHS)
Office of Minority Health (OMH)
Office for Human Research Protections (OHRP)

TABLE OF CONTENTS

EXECUTIVE SUMMARY

National Sickle Cell Awareness Month 2025
September 1 - 30, 2025

U.S. DEPARTMENT OF HEALTH & HUMAN SERVICES, OFFICE OF MINORITY HEALTH (OMH) IN PARTNERSHIP WITH THE OFFICE FOR HUMAN RESEARCH PROTECTIONS (OHRP)

Sickle cell disease (SCD) is a serious inherited blood disorder that disproportionately affects Black and African American communities and is associated with significant morbidity, reduced life expectancy, and substantial health system and societal costs. Individuals living with SCD often experience chronic pain, organ damage, and frequent hospitalizations, while also encountering longstanding gaps in access to care, research participation, and emerging therapies. Although recent scientific advances, particularly in gene-based treatment, have created new opportunities to improve outcomes, disparities in awareness, infrastructure, and access continue to limit their reach.[1]

National Sickle Cell Awareness Month (NSCAM), designated by Congress in 1983, serves as an annual opportunity to elevate public understanding of SCD and support improvements in care and treatment.[2] In recognition of NSCAM, the U.S. Department of Health & Human Services (HHS) Office of Minority Health (OMH) led a coordinated set of 2025 activities designed to raise awareness, promote access to innovation, and strengthen collaboration across federal agencies, states, health systems, and community partners.

Activities:

A key component of these efforts was OMH's two-part Gene Therapies Learning Series, hosted in partnership with OHRP, which examined ethical, clinical, and policy considerations related to emerging gene therapies for SCD.

Webinar #1, *Trust, Clinical Trials, and Ethical Pathways in Gene Therapy and Sickle Cell Disease*, hosted by OHRP, focused on the foundational role of trust and ethics in advancing research and care. Presenters discussed historical and contemporary drivers of mistrust in medical research, particularly among communities disproportionately affected by SCD. Patient advocates and community representatives contributed perspectives on barriers to clinical trial participation, emphasizing the importance of transparent communication, culturally responsive engagement, and meaningful informed consent. The session underscored that sustained patient and community involvement is essential to improving trial participation, strengthening ethical research practices, and supporting responsible translation of scientific advances.

Webinar #2, *Innovations and Advances in Sickle Cell Disease Treatments and Therapies*, examined the practical considerations associated with access to and implementation of gene therapies. Federal presenters outlined the Centers for Medicare & Medicaid Services (CMS) Cell and Gene Therapy (CGT) Access Model, a national initiative designed to support state Medicaid programs in addressing the high cost and administrative

complexity of cell and gene therapies through coordinated support and outcomes-based approaches.[3] State partners shared early implementation experiences, highlighting the importance of federal-state coordination, provider readiness, payment design, and partnerships with academic medical centers and community-based organizations.

Webinar #2 also included patient testimony describing lived experience with gene therapy. This perspective highlighted both the potential clinical benefits of advanced therapies and the importance of comprehensive supports, including long-term monitoring, mental health services, and clear communication throughout the treatment process.

Across both sessions, a consistent theme emerged: improving outcomes in SCD care requires more than scientific innovation alone. Effective progress depends on coordinated policy, ethical research practices, sustainable financing, clinical infrastructure, and community-driven engagement that centers patient perspectives. As HHS continues to advance gene therapy access and strengthen systems of care, OMH remains committed to fostering cross-sector collaboration and supporting implementation across the SCD care continuum.

WEBINAR #1: TRUST, CLINICAL TRIALS, AND TRANSFORMATIVE THERAPIES: ETHICAL PATHWAYS IN GENE THERAPY AND SICKLE CELL DISEASE

Thursday, September 18, 2025
1:00 PM – 2:30 PM ET

Agenda

Segment
Welcome Moderator: Jonathan Green, MD, MBA *Director, Office of Human Subjects Research Protections* *National Institutes of Health*
Opening Remarks Speaker: CAPT David Wong, MD *Senior Advisor, Office for Human Research Protections* *Office of the Assistant Secretary for Health*
Housekeeping Moderator: Jonathan Green, MD, MBA *National Institutes of Health*
Presentation #1: Patient/Caregiver Perspective on Gene Therapy Speaker: Antuan Sartin *Sickle Cell Disease Advocate* *Louisville, KY*
Presentation #2: Responsibilities for The Ethical Conduct of SCD Research Speaker: Wally Smith, MD *Florence Neal Cooper Smith Professor of Sickle Cell Disease* *Vice-Chair for Research, Division of General Internal Medicine* *Virginia Commonwealth University (VCU) Health, Richmond, VA*
Panel Discussion | Question & Answer Moderator: Jonathan Green, MD, MBA *National Institutes of Health* Panelist: Lakshmana Krishnamurti, MD *Professor of Pediatrics, Chief of the Section of Pediatric Hematology/Oncology/Bone Marrow Transplantation* *Yale School of Medicine, New Haven, CT* Panelist: Megha Kaushal, MD, MSc *Branch Chief, Division of Clinical Evaluation, Hematology Office of Therapeutic Products, Center for Biologics Evaluation and Research* *U.S. Food & Drug Administration* Panelist: Antuan Sartin *Sickle Cell Disease Advocate* Panelist: Wally Smith, MD *VCU Health*

Segment
Closing Remarks Speaker: Natalie Klein, PhD *Acting Director, Office for Human Research Protections* *Office of the Assistant Secretary for Health*

Summary of Webinar #1: Trust, Clinical Trials, and Transformative Therapies: Ethical Pathways in Gene Therapy and Sickle Cell Disease (hosted by OHRP)

BACKGROUND AND PURPOSE

Sickle cell disease (SCD) is a prevalent inherited blood disorder in the United States and is associated with significant clinical, social, and health system impacts. Individuals living with SCD commonly experience recurrent pain episodes, progressive organ damage, frequent use of acute care services, and reduced life expectancy. As scientific advances accelerate the development of disease-modifying and gene-based therapies, attention to ethical research practices, participant protections, and community engagement remains essential to supporting responsible innovation.

Webinar #1 of the National Sickle Cell Awareness Month (NSCAM) 2025 Gene Therapy Learning Series, *Trust, Clinical Trials, and Transformative Therapies: Ethical Pathways in Gene Therapy and Sickle Cell Disease*, convened federal leaders, clinicians, researchers, patient advocates, and caregivers to examine ethical considerations related to gene therapy research in SCD. As the first session in a two-part series, the webinar focused on research ethics, trust, and transparency as foundational elements for clinical trials and future therapeutic implementation.

The session occurred during a period of increasing research activity in SCD gene therapies. As clinical trials expand and therapies move through regulatory review, addressing issues related to informed consent, long-term follow-up, and research oversight is critical. Webinar #1 highlighted the importance of incorporating community perspectives and maintaining consistent human subjects' protections as part of the research process.

The webinar was intended for a broad audience, including individuals with SCD and their families, caregivers, clinicians, researchers, policymakers, human research protection professionals, and community-based organizations. Of the 965 individuals that registered for the meeting, 576 (60%) attended. Its structure combined patient and caregiver perspectives with expert presentations and a facilitated panel discussion to present multiple viewpoints on ethical research practices.

WELCOME

Jonathan Green, MD, MBA

Director, Office of Human Subjects Research Protections
National Institutes of Health

Dr. Jonathan Green, Director of the Office of Human Subjects Research Protections at the National Institutes of Health (NIH), opened the session by introducing the webinar's focus on trust, clinical trials, and ethical pathways in gene therapy for SCD. Drawing on his clinical experience as a pulmonary and critical care physician, he reflected on longstanding challenges in sickle cell care, noting that while potentially curative approaches such as stem cell transplantation and gene editing have advanced, many individuals with SCD continue to experience limited access to these therapies and reduced life expectancy. Dr. Green emphasized that the webinar would bring together a range of perspectives, including individuals with lived experience, clinician-investigators, and regulatory representatives, to examine ethical considerations, research protections, and the responsible development of transformative therapies. He concluded by outlining the structure of the session and introducing the opening remarks speaker.

OPENING REMARKS

CAPT David Wong, MD

Senior Advisor, Office for Human Research Protections
Office of the Assistant Secretary for Health

CAPT David Wong, representing the U.S. Department of Health & Human Services (HHS) Office for Human Research Protections (OHRP), opened the session by reaffirming HHS's commitment to improving outcomes for individuals living with SCD and their families. He noted that, historically, the sickle cell community has not received attention commensurate with the disease's burden and highlighted recent federal efforts to address this gap. CAPT Wong referenced milestones achieved across multiple administrations, including the development of the first National Academies consensus report and strategic blueprint for SCD, the establishment of a federal interagency workgroup involving 11 HHS agencies, and the convening of national summits and roundtables during previous Sickle Cell Awareness Months. He outlined ongoing contributions across HHS, including Health Resources & Services Administration (HRSA) supported comprehensive care programs, NIH-funded research and clinical trials, and the Cure Sickle Cell Initiative, which collectively contributed to the U.S. Food & Drug Administration's (FDA) landmark approval of two gene therapies for SCD in December 2023, including the first therapy utilizing CRISPR-based genome editing. CAPT Wong explained that Webinar #1 marked the first session in a two-part series led by OHRP and emphasized its focus on ethical considerations, participant protections, and trust in SCD gene therapy research, using the recently approved therapies as a case study.

PRESENTATION 1: PATIENT AND CAREGIVER PERSPECTIVE ON GENE THERAPY

Antuan Sartin
Sickle Cell Disease Advocate

Antuan Sartin, a parent and caregiver of a child with SCD, described his extended family's long-standing experience with SCD and the progression of his 12-year-old daughter's condition over time. He reported that his daughter was diagnosed at birth in 2013 and has received ongoing specialty care, including routine monitoring, preventive medications in early childhood, and hydroxyurea, which initially helped reduce pain crises. He noted that her health later declined, including recurrent pain crises, acute chest syndrome, frequent hospitalizations, and complications such as splenic enlargement, a hip replacement, and chronic transfusion needs.

Mr. Sartin described a severe episode in 2024 involving hemolytic anemia, prolonged hospitalization including intensive care, and subsequent stabilization with a specialty medication, which he attributed to improved outcomes. He stated that after consultation with her care team, the family explored curative options, including bone marrow transplant and gene therapy; he reported that transplant was not feasible due to lack of a donor match. He described significant administrative and clinical requirements to pursue gene therapy, including extensive testing and insurance authorization challenges, and indicated that Medicaid approval for gene therapy was obtained in 2025 but the family elected to pause pursuit of gene therapy temporarily due to his daughter's improved clinical condition.

During the Q&A, Mr. Sartin indicated that clinical trials had not been directly offered to his family and that he would need more information to consider trial participation. He emphasized the importance of caregiver advocacy, patient and family education, and sustained communication with clinicians when evaluating complex treatment decisions.

PRESENTATION 2: RESPONSIBILITIES FOR THE ETHICAL CONDUCT OF SCD RESEARCH

Wally Smith, MD
Florence Neal Cooper Smith Professor of Sickle Cell Disease
Vice-Chair for Research, Division of General Internal Medicine
VCU Health

Dr. Wally Smith discussed the ethical responsibilities of clinicians and institutions involved in SCD research and treatment, with a focus on balancing risks and benefits in clinical decision-making. He emphasized that institutions conducting research - including universities, pharmaceutical companies, and health systems - have a primary obligation to protect human subjects by ensuring scientifically sound study designs, minimizing risk, and clearly communicating potential risks and benefits to patients, families, and care teams. Transparent informed consent and adherence to established research protections were identified as foundational to responsible research practice.

Dr. Smith outlined how risk–benefit considerations vary across the life course for individuals with SCD. He noted that while pediatric outcomes have improved substantially due to newborn screening, preventive interventions, and early treatment, adults with SCD continue to experience higher rates of organ damage and mortality. These differences influence treatment decisions, including tolerance for higher-risk interventions such as gene therapy or transplant. He explained that younger patients with severe disease manifestations may reasonably consider therapies with greater upfront risk due to the potential for long-term benefit, while older patients with established organ damage may face different tradeoffs.

He further described how clinical trials and gene therapy eligibility criteria are developed, including age thresholds, disease severity indicators, and exclusion criteria, to ensure that anticipated benefits are reasonable in relation to risks. Dr. Smith noted that a substantial proportion of patients who initially express interest in gene therapy or transplant ultimately do not proceed, due to clinical ineligibility, insurance barriers, or personal decisions after receiving detailed information about risks and benefits.

In the discussion on trust, Dr. Smith stated that addressing historical and contemporary sources of mistrust directly is an essential part of engaging patients in care and research. He described an approach that includes acknowledging past ethical violations, recognizing ongoing challenges in pain management and access to care, and prioritizing effective pain control as a foundation for building clinician-patient relationships. He emphasized that trust is reinforced through consistent communication, responsiveness to patient needs, and shared decision-making, allowing individuals and families to make informed choices aligned with their goals and circumstances.

PANEL DISCUSSION AND QUESTION & ANSWER SESSION

Jonathan Green, MD, MBA
Moderator

Panelists:

- Lakshmana Krishnamurti, MD - Yale School of Medicine
- Megha Kaushal, MD, MSc - U.S. Food and Drug Administration
- Antuan Sartin - Sickle Cell Disease Advocate
- Wally Smith, MD - VCU Health

The panel discussion expanded the webinar's focus on trust, access, and ethical oversight by bringing together clinical, regulatory, and patient perspectives on gene therapy for SCD. New perspectives were provided by additional panelists, Dr. Lakshmanan Krishnamurti (Yale School of Medicine) and Dr. Megha Kaushal (FDA). The discussion addressed how individuals and families learn about gene therapy, how risks and benefits are communicated, and how systems can better support informed decision-making.

Panelists emphasized that many individuals with SCD are not routinely informed about gene therapy or other transformative treatment options, particularly those receiving care primarily through emergency departments or outside of specialized centers. Dr. Krishnamurti described the extensive, multi-visit counseling process required to support informed decision-making, noting that discussions often span several hours across multiple appointments and must account for patients' medical, social, and economic circumstances. He noted that only a subset of patients who seek consultation ultimately proceed with treatment, reflecting both eligibility criteria and individual preferences.

In response to questions about affordability and access, panelists highlighted recent federal and state efforts to support coverage for cell and gene therapies, including Medicaid participation in multi-state access models. They noted that while payment mechanisms and care coordination frameworks are increasingly in place, continued outreach is needed to ensure that patients, families, and frontline clinicians are aware of available options and referral pathways.

From the regulatory perspective, Dr. Kaushal described the FDA's approach to reviewing gene therapies, emphasizing the importance of demonstrating meaningful clinical benefit - such as reductions in vaso-occlusive crises - alongside rigorous safety evaluation. She noted that long-term follow-up, including monitoring for delayed adverse effects, is a standard component of approval and post-market oversight for these therapies.

The discussion also addressed the role of institutional review boards (IRBs) and informed consent in balancing patient urgency with research protections. Dr. Smith explained that gene therapy trials require extended follow-up periods, often up to 15 years, to assess long-term safety and outcomes. Panelists emphasized that IRBs, informed consent processes, and ongoing patient engagement are central safeguards in the research enterprise.

Audience questions highlighted persistent gaps in awareness about eligibility and treatment centers. Panelists noted that relatively few clinicians have the specialized expertise needed to assess candidacy for gene therapy and encouraged referrals to designated treatment centers and national organizations that maintain updated information. They also discussed the importance of framing conversations around potential benefit, rather than eligibility alone, to support shared decision-making.

Finally, panelists addressed the psychological and social dimensions of transformative therapies. They noted that while reductions in pain and hospitalizations can significantly improve quality of life, patients may continue to experience anxiety, depression, or challenges related to identity and life transitions following treatment. The discussion underscored the need for multidisciplinary care models that include mental health and social support services before, during, and after gene therapy.

The session concluded with agreement that improving outcomes for individuals with SCD will require coordinated efforts across clinical care, research, regulation, and community engagement, alongside sustained attention to education, trust-building, and long-term patient support.

CLOSING REMARKS

Natalie Klein, PhD

Acting Director, Office for Human Research Protections
Office of the Assistant Secretary for Health

In her closing remarks, Dr. Klein thanked the speakers and participants for a substantive and informative discussion and acknowledged the range of expertise represented, including the perspectives of patients, caregivers, clinicians, and researchers. She emphasized that these varied forms of expertise collectively contribute to trustworthy clinical research.

Dr. Klein reiterated the mission of OHRP to safeguard the rights, safety, and welfare of research participants, noting that this responsibility is shared across researchers, institutions, and the broader community. She recognized attendees as active contributors to this shared mission.

She also encouraged participants to continue their engagement by attending Part 2 of the webinar series, hosted by the HHS Office of Minority Health.

WEBINAR #2: INNOVATIONS AND ADVANCES IN SICKLE CELL DISEASE GENE THERAPIES

Thursday, September 25, 2025
2:00 PM – 3:30 PM ET

Agenda

Segment
Welcome Moderator: CDR Matthew Johns *Sickle Cell Disease Team Lead, Office of Minority Health* *U.S. Department of Health & Human Services*
Opening Remarks Speaker: CAPT Mahyar Mofidi *Director, Office of Minority Health* *U.S. Department of Health & Human Services*
Cell and Gene Therapy (CGT) Access Model Overview Speaker: Abraham Sutton Director, Center for Medicare and Medicaid Innovation Centers for Medicare & Medicaid Services
South Carolina's Experience with Gene Therapy Access for Sickle Cell Disease (SCD): Expanding Access, Lessons Learned, and Policy Perspective Speaker: Eunice Medina *Director* *South Carolina Department of Health and Human Services* Speaker: Kevin Wessinger, MD *Chief Medical Officer, Medical Director* *South Carolina Department of Health and Human Services*
Sickle Cell Disease Patient Advocate Presentation/Video: Living with SCD after Gene Therapy Speaker: Jimi Olaghere Sickle Cell Warrior

Segment
Question & Answer Moderator: LCDR Oluwabukola "Bukky" Akinsiku *Senior Advisor, Office of Minority Health* *U.S. Department of Health & Human Services* Panelist: Eunice Medina *South Carolina Department of Health and Human Services* Panelist: Kevin Wessinger, MD *South Carolina Department of Health and Human Services* Panelist: Caroline Horrow *CMS Cell and Gene Therapy (CGT) Access Model Co-Lead, Center for Medicare and Medicaid Innovation* *Centers for Medicare & Medicaid Services*
Closing Remarks Moderator: CDR Matthew Johns U.S. Department of Health & Human Services

Summary: Innovations and Advances in Sickle Cell Disease Gene Therapies (hosted by HHS OMH)

BACKGROUND AND PURPOSE

Sickle cell disease (SCD) disproportionately affects Black and African American communities and has historically received limited public health attention relative to its burden. Individuals living with SCD experience significant pain, chronic health complications, and reduced life expectancy. While recent therapeutic innovations offer hope for longer, healthier lives, the uneven distribution of knowledge, access, and resources across states, health systems, and socioeconomic groups continue to shape the patient experience.

Webinar #2 of the National Sickle Cell Awareness Month (NSCAM) 2025 series, *Innovations and Advances in Sickle Cell Disease Gene Therapies*, brought together federal partners, state leaders, clinicians, patient advocates, and individuals living with SCD to explore the rapidly evolving landscape of gene therapy and its implications for access. As part of HHS's coordinated recognition of NSCAM, this session built on the themes of ethics and trust introduced in Webinar #1 and shifted the discussion toward the structural, clinical, and human dimensions of gene therapy implementation.

The session took place at a pivotal moment in SCD care. Only two years had passed since the landmark U.S. Food & Drug Administration (FDA) approval of the first gene therapies for SCD in December 2023[4], and states were entering the early phases of implementing the Centers for Medicare & Medicaid Services (CMS) Cell and Gene Therapy (CGT) Access Model, an ambitious federal effort to support availability of these transformative treatments. Against that backdrop, Webinar #2 served as a national platform to illuminate not only the science behind newer therapies but the policies, partnerships, and lived experiences that shape how innovations reach the communities most affected.

The webinar was designed for a broad audience of stakeholders, including individuals with SCD and their families, researchers, clinicians, policymakers, patient advocates, and community-based organizations. Of the 882 individuals who registered for the meeting, 381 (43%) participated. The meeting's structure - federal presentation, state perspective, and person with lived experience - reflected the U.S. Department of Health & Human Services (HHS) Office of Minority Health's (OMH) commitment to integrating systems-level insight with lived experience.

WELCOME

CDR Matthew Johns

Sickle Cell Disease Team Lead, Office of Minority Health
U.S. Department of Health and Human Services

CDR Matthew Johns served as moderator and guided attendees through the structure, goals, and engagement opportunities of the session. His remarks framed the day's conversation as part of a broader mission to use SCD Awareness Month as a catalyst for education and empowerment across the SCD community. He reminded attendees that the webinar was being recorded and would be archived as part of a continuing HHS effort to make SCD educational resources widely accessible across communities and time zones.

OPENING REMARKS

CAPT Mahyar Mofidi

Director, Office of Minority Health
U.S. Department of Health and Human Services

CAPT Mahyar Mofidi opened the webinar by expressing his deep commitment, on behalf of HHS, to improving the lives of individuals living with SCD and their families. He underscored that nearly 100,000 people in the United States are affected by SCD and highlighted the promise of newly emerging, innovative tools to treat and manage the complex challenges patients face.

CAPT Mofidi emphasized the important partnership between HHS and CMS's Center for Medicare & Medicaid Innovation (CMMI), noting that participants would soon hear about national efforts to expand access to breakthrough SCD treatment. He framed the webinar as the second in a two-part series dedicated to advancing understanding of SCD and gene therapies, led by the Office of Minority Health (OMH), to strengthen connections among patients, caregivers, advocates, and community health workers.

CAPT Mofidi affirmed that collaboration across HHS agencies - including CMS, the Health Resources & Services Administration (HRSA), National Institutes of Health (NIH), U.S. Centers for Disease Control and Prevention (CDC), and Administration for Strategic Preparedness & Response (ASPR) - continues to drive progress in SCD efforts and that OMH is committed to building on longstanding relationships with community organizations. Looking ahead, CAPT Mofidi highlighted the significance of newly approved gene therapies and expressed confidence that continued partnership and dialogue can help transform the trajectory of SCD nationwide. He closed by thanking attendees and passing the program back to the moderator.

PRESENTATION 1: THE CMS CELL AND GENE THERAPY ACCESS MODEL

Abraham Sutton

Director, Center for Medicare and Medicaid Innovation (CMMI)
Centers for Medicare & Medicaid Services (CMS)

CMS Innovation Center Director Abraham Sutton opened by framing the CGT Access Model for SCD to turn the promise of highly effective but extremely expensive gene therapies (often ~$2M per patient) into real, meaningful access for Medicaid beneficiaries. He described how CMS centrally negotiates outcome-based agreements and discounts with manufacturers, then offers states a standardized package - single pricing structure, unified coverage criteria, and prior authorization requirements - within a voluntary model that also provides states with technical assistance, data support, and optional implementation funding. Director Sutton outlined three core goals of the Model: hold manufacturers accountable for real-world outcomes, improve beneficiary access to innovative therapies, and generate long-term savings through discounted pricing and reduced health expenditures.

Director Sutton highlighted that 33 states, D.C., and Puerto Rico have joined the Model, with agreements in place with both approved sickle cell gene therapy manufacturers, and services already underway. A key feature of the Model is required, no-out-of-pocket fertility preservation for eligible patients, enabled through fraud-and-abuse safe harbors, given the risk of treatment-related sterilization in a young population. Director Sutton emphasized the model's broader significance as a template for future cell and gene therapies across conditions, aligning interests of manufacturers, states, patients, and taxpayers, He also recognized the collaboration of HHS, states, treatment centers, manufacturers, and sickle cell warriors in bringing the model from concept to implementation.

PRESENTATION 2: STATE EXPERIENCE - SOUTH CAROLINA'S GENE THERAPY ACCESS JOURNEY

Eunice Medina
Director
South Carolina Department of Health and Human Services

Kevin Wessinger, MD
Chief Medical Officer, Medical Director
South Carolina Department of Health and Human Services

South Carolina Medicaid Director, Eunice Medina, highlighted the significant impact of the CGT Model on the state's ability to expand treatment access for SCD. Since 2021, growing interest from state leadership prompted South Carolina to examine its Medicaid population, identifying around 400 beneficiaries eligible for gene therapy. South Carolina began early outreach to gene therapy manufacturers and while the state initially explored its own agreements, CMS's CGT Model offered far greater opportunity and scale. Director Medina emphasized the critical role of the Medical University of South Carolina (MUSC) as the state's treatment center and described steps to ensure sustainable financing, including developing a separate payment approach, submitting a State Plan Amendment, and using a high-cost/no-experience drug list to support managed care plans. Director Medina concluded by introducing Chief Medical Officer Dr. Kevin Wessinger to discuss implementation progress.

Dr. Kevin Wessinger, Chief Medical Officer for South Carolina Medicaid, described how the state's early groundwork positioned it to move quickly once the CGT Access Model launched. He noted that South Carolina began planning and negotiating with MUSC and manufacturers even before sickle cell gene therapies received FDA approval in December 2023, which gave the state a head start in meeting model requirements and securing early approval to participate. Since joining the CGT Model on June 1, 2025, MUSC has initiated gene therapy treatment for Medicaid enrollees, with two members already completing infusion and three more in the treatment pipeline, using products from both participating manufacturers.

Dr. Wessinger emphasized that this progress has required close, ongoing collaboration with MUSC, CMS, and peer states such as Missouri, as well as careful attention to capacity constraints and financing. With an estimated 400 Medicaid beneficiaries potentially eligible for gene therapy and current capacity of only 6-10 treatments per year at MUSC, he underscored the need to plan for both clinical and fiscal impact. He highlighted the importance of comprehensive coverage (including fertility preservation and transportation), the use of a high-cost/no-experience drug list to protect managed care plans while guaranteeing payment to treatment centers, and the critical role of

CMS-negotiated outcomes-based agreements in making the model workable for a small state team. Looking ahead, Dr. Wessinger stated that South Carolina views participation in the CGT Model as essential, is proud to be an early adopter, and intends to leverage lessons learned to support future gene therapies and inform national policy.

PRESENTATION 3: LIVED EXPERIENCE - A PATIENT'S GENE THERAPY JOURNEY

Mr. Jimi Olaghere
Sickle Cell Warrior

Sickle Cell Warrior Jimi Olaghere shared his experience accessing gene therapy. After years of severe complications and repeated near-death episodes, he proactively researched gene therapy, set up alerts for emerging information, and eventually learned about the first CRISPR trial participant, Victoria Gray. He contacted the trial's principal investigator, completed a screening call, and soon traveled to Nashville for extensive testing. Within a month, he was accepted into the trial.

Mr. Olaghere began treatment in January 2020, describing a four-stage process: (1) a stabilization period with frequent transfusions; (2) multiple stem-cell collection sessions; (3) chemotherapy-based conditioning to clear his bone marrow; and (4) infusion of his own CRISPR-edited cells, which engrafted within about two weeks without the risks associated with donor transplants.

After treatment, Mr. Olaghere's chronic sickle cell pain disappeared, and he experienced a profound improvement in physical functioning - enabling him to exercise, climb Mount Kilimanjaro, and pursue additional mountain-climbing challenges to raise awareness for SCD. He also reflected on the emotional transition that follows such transformative therapy, noting the need for mental health support as patients adjust to life and identity beyond decades of illness.

QUESTION AND ANSWER SESSION

LCDR Oluwabukola "Bukky" Akinsiku
Senior Advisor, Office of Minority Health
U.S. Department of Health and Human Services

Panelists:

- Eunice Medina - South Carolina Department of Health and Human Services
- Kevin Wessinger, MD - South Carolina Department of Health and Human Services
- Caroline Horrow - CGT Access Model Co-Lead, CMS CMMI

During the Question & Answer (Q&A) discussion, moderated by LCDR Oluwabukola Akinsiku, several clear themes emerged about what it takes to make sickle cell gene therapy accessible and supportive for patients.

Community partnerships were emphasized as essential. CMMI described how community-based organizations (CBOs), advocacy groups, and nonprofits were involved throughout the development of the CGT Access Model. States can use federal cooperative agreement funds to partner with CBOs to expand patient education, reduce logistical barriers such as transportation and lodging, and provide navigation and peer support. Speakers underscored that CBOs remain a trusted bridge to communities most affected by SCD.

Access to treatment centers was another major theme. Because relatively few facilities nationwide can deliver gene therapy, the model requires participating states to ensure Medicaid beneficiaries can reach at least one qualified center, even if it is located out of state. States must publish coverage criteria, maintain points of contact, and proactively plan for out-of-state treatment to minimize delays for patients.

Questions about fertility preservation reflected a high level of concern among attendees. CMMI explained that manufacturers participating in the outcomes-based agreements must cover a defined package of fertility preservation services at no cost to the patients or states, including collection, cryopreservation, and storage. This requirement was designed to protect patients undergoing conditioning regimens that may compromise fertility.

The discussion also touched on state participation and future expansion. Thirty-three states, the District of Columbia, and Puerto Rico have joined the CGT Access Model, and several have received funding to strengthen implementation. While enrollment for the sickle cell component has closed, CMMI noted that future conditions or new therapies could create additional opportunities for participation.

Attendees also raised concerns about coverage and eligibility. South Carolina clarified that receiving gene therapy does not automatically affect Medicaid eligibility, which depends on multiple financial and medical factors. For patients facing insurance denials,

treatment centers may offer additional programs or supports outside standard coverage pathways.

A recurring theme was the psychosocial impact of gene therapy. Drawing on patient stories, including Mr. Olaghere's reflections on identity shifts after treatment, participants asked how social workers can better support patients emotionally. South Carolina indicated that, as more patients undergo therapy, they expect the need for behavioral health services and more holistic, coordinated care to grow.

Finally, South Carolina leaders highlighted the importance of state-federal collaboration as a key lesson. Because gene therapies are complex and expensive, the shared federal framework of the CGT Model has been instrumental in helping states navigate pricing, access, and implementation. They viewed this approach as a promising model for future therapies that will require similarly robust coordination.

Overall, the Q&A reinforced that successful access to gene therapy requires not only scientific innovation, but also strong partnerships, clear processes, and comprehensive support for patients throughout their treatment journey.

CLOSING REMARKS

CDR Matthew Johns

Sickle Cell Disease Team Lead, Office of Minority Health
U.S. Department of Health and Human Services

CDR Johns concluded with reflections on the quality and depth of audience engagement, underscoring the strong interest in the ethical, clinical, and practical dimensions of emerging gene therapies. Participants were encouraged to revisit Part 1 of the webinar series, which explored ethical considerations and challenges in clinical trial recruitment, topics that complement the more implementation-focused discussions of this session.

Looking ahead, CDR Johns noted that the next phase of the series will turn toward comprehensive systems of care, an opportunity to integrate lessons from the gene therapy conversation into a broader strategy for supporting individuals living with SCD. This future focus aligns with ongoing efforts across HHS agencies, including OMH, NIH, FDA, CDC, CMS, and HRSA, to build coordinated, patient-centered approaches that extend beyond clinical innovation alone.

Key takeaways emphasized the need for collaboration across federal, state, and clinical partners, and for careful planning and wraparound supports to ensure that gene therapy advances translate into real-world access. The remarks also reaffirmed a commitment to community-driven approaches in which patients, families, advocates, and clinicians all play central roles in shaping solutions.

The closing message acknowledged the strength and resilience of individuals living with SCD, honoring them as leaders and advocates whose experiences guide the work

ahead. Clinicians and caregivers were likewise recognized for their dedication and partnership. The session ended with gratitude to presenters, partners, and attendees, and a renewed commitment to advancing comprehensive care for the sickle cell community.

BUILDING ON THIS FOUNDATION

The two-part National Sickle Cell Awareness Month (NSCAM) 2025 webinar series highlighted both the opportunities and considerations associated with emerging gene therapies for sickle cell disease (SCD). Across both sessions, speakers emphasized that recent scientific advances represent an important development in SCD care, while also underscoring the need for careful attention to ethical oversight, patient protections, system readiness, and sustained engagement with affected communities.

Webinar #1 focused on ethical pathways in gene therapy, emphasizing the importance of trust, transparency, and informed decision-making in clinical research. Presenters and panelists discussed the respective responsibilities of institutions, investigators, regulators, and clinicians to protect human research participants, clearly communicate risks and potential benefits, and support voluntary participation in clinical trials. The session also highlighted the role of patient and caregiver perspectives in shaping ethical research practices, particularly in the context of historical mistrust and ongoing challenges in access to care.

Building on this foundation, Webinar #2 examined the early implementation of the U.S. Food & Drug Administration (FDA) approved gene therapies and the policy and operational considerations required to support patient access. Federal and state leaders described the Centers for Medicare & Medicaid Services (CMS) Cell and Gene Therapy (CGT) Access Model as a mechanism to address cost, coverage, and administrative complexity for Medicaid beneficiaries. State experiences illustrated the importance of early planning, coordination with treatment centers, and alignment across clinical, financial, and operational systems. Patient perspectives reinforced the need for comprehensive supports, including education, navigation, and psychosocial services, throughout the treatment process.

Taken together, the webinars demonstrate that advancing gene therapy for SCD requires coordinated action across research, regulatory, clinical, and policy domains. Scientific innovation alone is insufficient without complementary efforts to strengthen trust, ensure appropriate protections, and support health system capacity.

Based on the discussions from both sessions, several areas for continued focus emerged:

- **Sustain ethical oversight and transparency:** Continue to reinforce clear communication of risks, benefits, eligibility criteria, and uncertainties in both clinical trials and clinical care, consistent with federal human subjects protections.
- **Support informed decision-making:** Promote access to balanced, understandable information for patients and families to support individualized treatment decisions aligned with clinical circumstances and personal preferences.
- **Strengthen care delivery infrastructure:** Encourage continued collaboration among federal agencies, states, treatment centers, and community-based organizations to address capacity constraints, workforce needs, and care coordination.

- **Expand education and engagement:** Maintain efforts to disseminate educational resources, archive webinar materials, and engage diverse stakeholders, including patients, caregivers, clinicians, and policymakers.
- **Monitor implementation and outcomes:** Continue to assess early experiences with gene therapy implementation, including access, utilization, patient experience, and longer-term outcomes, to inform future policy and program development.
- **Building Comprehensive Systems of Care**: Understand and advance comprehensive systems of care for SCD to promote holistic disease management and improved quality of life.

As gene therapies for SCD continue to evolve, the insights shared during this webinar series provide a framework for aligning innovation with ethical practice, patient protections, and system readiness. Ongoing dialogue and coordination across sectors will remain essential to responsibly translating scientific advances into clinical care.

RESOURCES

A range of federal initiatives and informational platforms support patients, families, clinicians, and policymakers seeking to advance understanding of sickle cell disease (SCD) and expand access to emerging therapies.

Sickle Cell Disease | Office of Minority Health

The HHS OMH SCD landing page serves as a centralized hub for federal resources, educational materials, webinar recordings, and updates related to SCD. It provides ongoing information about federal initiatives aimed at improving health outcomes and strengthening community engagement.

Link: minorityhealth.hhs.gov/sickle-cell-disease

CMS Cell and Gene Therapy (CGT) Access Model Information Page

CMS provides detailed information on the CGT Access Model, including state participation, program requirements, and resources for implementation. The site outlines the model's goals, financing structure, and guidance for participants and other stakeholders.

Link: cms.gov/priorities/innovation/innovation-models/cgt

NIH Cure Sickle Cell Initiative (CureSCi)

The National Institutes of Health leads the Cure Sickle Cell Initiative, a collaborative research effort focused on accelerating the development of genetic therapies and improving long-term outcomes for individuals living with SCD. The website offers scientific updates, clinical trial information, patient education tools, and opportunities to engage with the research community.

Link: curesickle.org

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APPENDIX

MEET THE PRESENTERS

Part 1—Trust, Clinical Trials, and Transformative Therapies: Ethical Pathways in Gene Therapy and Sickle Cell Disease
Thursday, September 18, 2025 | 1:00 PM – 2:30 PM ET

Jonathan M. Green, MD, MBA (Moderator)

Director, Office of Human Subjects Research Protections
National Institutes of Health

Jonathan M. Green, MD, MBA, is Director, Office of Human Subjects Research Protections and Institutional Official for Human Subjects at the National Institutes of Health (NIH). Prior to joining the NIH, Dr. Green was professor of medicine, pathology, and immunology, as well as Associate Dean for Human Studies, and Executive Chair of the institutional review board at Washington University School of Medicine in St. Louis, MO. At Washington University, Dr. Green conducted research on the molecular mechanisms of T cell activation, focusing on the CD28 costimulatory family of receptors. He received his medical degree from Wayne State University in Detroit followed by residency training in internal medicine at Boston City Hospital. He then completed a fellowship in pulmonary and critical care medicine at the University of Michigan Medical Center, and additional post-doctoral training at the University of Chicago. He received an MBA from Washington University Olin School of Business in 2017. He is board certified in internal medicine, pulmonary diseases, and critical care medicine. Dr. Green continues to serve as an attending physician in the Medical Intensive Care Unit and Pulmonary Consult Service at the NIH Clinical Center and has conducted both basic science and clinical research on the regulation of the immune response.

Dr. Green has had a long-standing interest in biomedical ethics. He had been a member of the Barnes Jewish Hospital Ethics Committee since 2000, leading the clinical ethics consultation service from 2001-2005 and serving as Chair of the Ethics Committee from 2005-2009. After joining the Washington University Institutional Review Board in 2008, he assumed the role of committee co-chair in 2009. In 2010, he was appointed Associate Dean of Human Studies and Executive Chair of the IRB at Washington University in St Louis. Dr. Green served on the Secretary's Advisory Committee on Human Research Protections (SACHRP) from 2015-2018, also serving on the Subpart A subcommittee.

Areas of Expertise: Biomedical ethics, human research protections, institutional review boards, pulmonary-critical care medicine

Antuan Sartin
Sickle Cell Disease Advocate

My name is Antuan Sartin, and I am the father of Kali Sartin. I was born in Louisville, Kentucky, and attended Ballard High School in 2001. I subsequently enrolled at Western Kentucky University to pursue higher education. A few years later, I became an employee of the United States Postal Service (USPS) as a city carrier.

Throughout my life, I have been aware of sickle cell anemia, but it did not fully resonate with me until after discovering my daughter's diagnosis. During my childhood, my aunt and uncle were afflicted with sickle cell anemia, and I frequently observed them visiting the hospital. When my mother would mention, "That's your auntie or uncle returning from the hospital," I perceived it as a routine occurrence. However, it was not until November 2008 that I comprehended the severity of the condition. That same year, I tragically lost my aunt due to complications associated with sickle cell anemia, at the age of 36. My uncle subsequently succumbed to sickle cell complications in March 2019, at the age of 51.

In 2014, my family and I were introduced to the Sickle Cell Association of Kentuckiana. This organization provided us with valuable information and support regarding our daughter's condition. We actively participated in some of the events they organized.

Presently, as a full-time parent and caregiver for my 12-year-old daughter, I am committed to making numerous sacrifices to ensure her well-being. Together, we have forged a strong bond and are determined to navigate the challenges of sickle cell anemia.

Areas of Expertise: Advocacy, lived experience as a caregiver of a child with SCD, navigating SCD clinical care and treatment options

Wally R. Smith, MD

Florence Neal Cooper Smith Professor of Sickle Cell Disease
University Distinguished Professor
Vice-Chair for Research
Division of General Internal Medicine
Virginia Commonwealth University School of Medicine

Wally R. Smith, MD, is the Florence Neal Cooper Smith Professor of Sickle Cell Disease and Vice Chair for Research of the Division of General Medicine at Virginia Commonwealth University (VCU). Dr. Smith is Executive Editor of the Journal of Sickle Cell Disease, sponsored by the Foundation for Sickle Cell Disease Research and published by Oxford University Press. He is active in the American Society of Hematology. He has held over 50 grants, but is best known for his NIH-funded Pain in Sickle Cell Epidemiology Study (PiSCES), which led to the first NIH Request for Proposals on the Neurobiology of Pain in SCD, and supported national consensus research definitions of acute and chronic pain in SCD. He was a member of the lnteragency Pain Research Coordinating Committee for DHHS, which published the National Pain Strategy.

Dr. Smith sits on the Multi-disciplinary Working Group advising the NIH's $500 million/ year Helping to End Addiction Long-term (HEAL) Initiative. He was a principal investigator in the Cooperative Study of Sickle Cell Disease, the Multicenter Study of Hydroxyurea in Sickle Cell Disease, the Sickle Cell Disease Clinical Research Network, the Sickle Cell Disease Outcomes Research Network, the Health Resources and Services Administration Sickle Cell Disease Treatment Demonstration Program, and the NIH Basic and Translational Research Program in SCD. Dr. Smith has been associated with the development of two potential remittive agents for SCD at VCU and has been a major contributor to several trials of SCD remittive agents either just approved or nearing FDA approval. His latest multicenter NIH-funded trial was SHIP HU, which is now complete with several publications.

Areas of expertise: SCD clinical trials, pain research and management, epidemiology, emerging therapies, institutional research oversight

Lakshmanan Krishnamurti, MD

Professor of Pediatrics
Chief, Section of Pediatric Hematology/Oncology/Bone Marrow Transplantation
Yale School of Medicine
Yale New Haven Hospital

Dr. Krishnamurti is a pediatric hematologist oncologist with a primary focus interest in clinical and patient reported outcomes research, and clinical trials in SCD. He currently serves as Professor of Pediatrics and the Chief of Pediatric Hematology, Oncology, and Bone Marrow Transplantation at Yale University Hospital. Dr. Krishnamurti's research contributions in SCD span acute and chronic pain, newborn screening, community and international outreach, bone marrow transplantation and gene therapy. His research has been funded by the NIH, CDC, HRSA, PCORI, and several philanthropic foundations. Dr. Krishnamurti's experience in curative therapies for SCD also extends to patient education, patient decision making, implementation of these therapies program and evaluation of short-term and long-term outcomes. Dr. Krishnamurti has been active as a site principal investigator (PI) in multiple clinical trials of gene therapy for SCD and is an author in several peer reviewed publications relevant to the subject.

Areas of expertise: SCD clinical trials, bone marrow transplantation, gene therapies, patient education and decision making

Megha Kaushal, MD, MSc

Branch Chief, Division of Clinical Evaluation Hematology
Office of Therapeutic Products
Center for Biologics Evaluation and Research
Food & Drug Administration

Megha Kaushal, MD, MSc. is a pediatric hematologist/ oncologist who serves as the Branch Chief for Benign Hematology in the Division of Clinical Evaluation Hematology within the Office of Therapeutic Products in the Center for Biologics Evaluation and Research (CBER) at the Food and Drug Administration (FDA).

Dr. Kaushal received her M.D. from Rush University, Chicago and completed her medical training in Pediatrics at Medical College of Georgia and her fellowship in Pediatric Hematology Oncology at Children's National in DC. Her clinical interests include SCD, hemophilia, bone marrow failure syndromes, and hemostasis and thrombosis.

Following fellowship, she joined the Division of Bone Marrow Transplant at Children's National prior to joining the FDA in 2014 as a clinical reviewer. In her role, she has been responsible for the review and regulatory oversight of several benign and malignant hematology Investigational New Drug (IND) and Biologic Licensing Applications (BLAs).

Areas of expertise: Sickle cell disease, clinical review and regulatory oversight of gene therapies and other biologics

Natalie Klein, PhD

Acting Director
Office for Human Research Protections
U.S. Department of Health & Human Services

Natalie Klein, PhD, is the Acting Director of the U.S. Department of Health and Human Services (HHS), Office for Human Research Protections (OHRP), which provides leadership in the protection of the rights, welfare, and well-being of subjects involved in research conducted or supported by HHS.

Dr. Klein joined OHRP as the Director of the Division of Policy and Assurances in 2021 from the U.S. Army Medical Research and Development Command (USAMRDC), where she served as a liaison to the Command's intramural research institutes for human research protections policy and helped provide regulatory oversight for USAMRDC-supported research conducted at over 1600 institutions in 67 countries. She holds a doctorate in Brain and Cognitive Sciences.

Areas of expertise: Human research protections, regulatory oversight, policy development

Part 2—Innovations and Advances in Sickle Cell Disease Gene Therapies
Thursday, September 25, 2025 | 2:00 PM – 3:30 PM ET

CDR Matthew Johns (Moderator)

Sickle Cell Disease Team Lead, Office of Minority Health
U.S. Department of Health and Human Services

Commander (CDR) Matthew Johns, MPH is the Regional Health Administrator for HHS Region 9 and a commissioned officer in the U.S. Public Health Service, serving as the senior federal public health official for the Office of the Assistant Secretary for Health. He also serves as Deputy for the Division of Preventive Medicine and Chief Epidemiologist for the PHS-2 Rapid Deployment Force.

CDR Johns has led and supported domestic and international public health emergency responses, including deployments for Ebola in Liberia, major hurricanes, COVID-19, and other disaster and humanitarian missions. His work spans global health security, emergency preparedness and response, epidemiology, and interagency coordination, including partnerships with FEMA to strengthen data-driven public health operations and community resilience.

Areas of expertise: Emerging infectious disease surveillance, epidemiology and response, and public health capacity building

CAPT Mahyar Mofidi, DMD, PhD

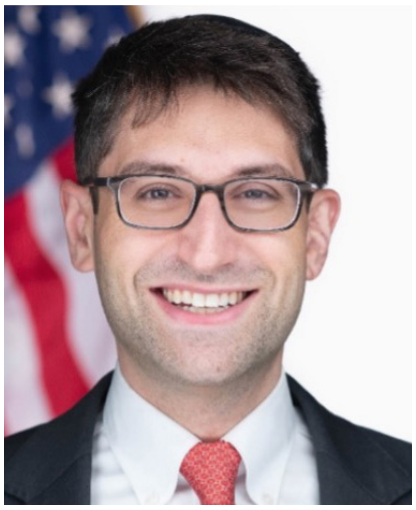
Deputy Assistant Secretary for Minority Health
Director, Office of Minority Health
U.S. Department of Health and Human Services

CAPT Mahyar Mofidi serves as the Deputy Assistant Secretary for Minority Health and Director of the HHS Office of Minority Health, where he leads national efforts to improve the health of racial and ethnic minority populations. He brings more than 25 years of public health experience across urban and rural settings, with a focus on serving underserved communities.

Prior to joining OMH, CAPT Mofidi spent over nine years at the Health Resources and Services Administration's HIV/AIDS Bureau, where he directed community HIV/AIDS programs, oversaw a $300 million portfolio supporting hundreds of community-based organizations, and advanced rural health and oral health initiatives. His earlier career includes academic and research roles at the University of North Carolina, with work focused on access to care for low-income and rural populations.

CAPT Mofidi holds a Doctor of Dental Medicine degree from the University of Louisville and a PhD in Health Behavior from the University of North Carolina.

Areas of expertise: Public health leadership, minority health policy, health equity and access, HIV/AIDS programs, community-based health systems, rural and underserved population health, federal health program administration



Mr. Abe Sutton

Director, Center for Medicare and Medicaid Innovation (CMMI)
Centers for Medicare & Medicaid Services (CMS)

Abe Sutton serves as the Director of the Center for Medicare and Medicaid Innovation and Deputy Administrator for the Centers for Medicare and Medicare Services (CMS). Before assuming this role in January of 2025, he was a Principal at Rubicon Founders where he co-founded two health service companies; Honest Health, which focuses on enabling primary care physicians, and Evergreen Nephrology, which focuses on enabling nephrologists. Sutton focused on health policy with the federal government from 2017 to 2019, serving at the National Economic Council, Domestic Policy Council and Department of Health and Human Services. In these roles, he coordinated health policy across the federal government, with a focus on the shift to paying-for-value within Medicare, increasing choice and competition in health care markets, and updating the federal government's approach to kidney care.

Sutton started his career as a consultant with McKinsey & Company, where he worked with clients in the health sector. He holds a law degree from Harvard Law School and undergraduate degrees in political science, management, and health care management and policy from the Wharton School and the College at the University of Pennsylvania. In 2018, he was named to Forbes 30 Under 30 for Law and Policy.

Areas of expertise: Medicare and Medicaid policy, value-based care innovation, federal health system reform

Ms. Eunice Medina

Director
South Carolina Department of Health and Human Services

On Feb. 27, 2025, Eunice Medina was unanimously confirmed to serve as the Director of the South Carolina Department of Health and Human Services (SCDHHS) by the South Carolina Senate. Ms. Medina was nominated for the role of director on Nov. 6, 2024, by Governor Henry McMaster. Prior to this role, Medina had served as the agency's chief of staff and deputy director of programs since June 2021.

Medina has extensive health care experience including more than 20 years with Medicaid policy and operations. Prior to her current role with SCDHHS, she served as a bureau chief with the Florida Agency for Health Care Administration where she managed Florida's 18 plus Medicaid managed care plans and worked extensively with the state's home and community-based services waiver programs.

Medina is a graduate of Florida State University and of the South Carolina Executive Institute.

Areas of expertise: State Medicaid leadership, Medicaid policy and operations, managed care and home- and community-based services

Dr. Kevin Wessinger, M.D., F.A.A.P.

Medical Director-Chief Medical Officer
South Carolina-Department of Health and Human Services

Dr. Kevin Wessinger is a seasoned healthcare professional serving as the Chief Medical Officer at the South Carolina Department of Health and Human Services. With over three decades of experience in pediatrics and healthcare management, he has held leadership roles that emphasize clinical quality and strategic planning. He is a board-certified physician with a strong background in electronic medical records and practice management. Kevin has also been instrumental in leading pediatric healthcare initiatives through his previous roles, including President of the South Carolina Pediatric Alliance. His dedication to improving health outcomes is evident in his commitment to public speaking and education in the medical field. Kevin's academic foundation includes a Doctor of Medicine from the Medical University of South Carolina and a Bachelor's in Microbiology and Immunology from Clemson University. He is passionate about advancing pediatric healthcare and is known for his collaborative approach to leadership.

Areas of expertise: Medicaid clinical leadership, pediatric healthcare, clinical quality improvement, health system management

Mr. Jimi Olaghere

Sickle Cell Disease Patient Advocate

Drawing from his personal experience of living with Sickle Cell Disease for 35 years, Jimi Olaghere has become a staunch advocate for increased accessibility of gene therapies for SCD patients. His dedication to patient advocacy stems from a deep-rooted belief in the power of equitable healthcare and the fundamental right of every individual to access life-changing treatments.

Olaghere's journey took a transformative turn when he became involved in a groundbreaking clinical trial. His participation in the trial resulted in a significant achievement – a functional cure for Sickle Cell Disease. This monumental breakthrough not only transformed Olaghere's life but also propelled him into the forefront of the conversation surrounding gene therapy and its potential to revolutionize healthcare.

Areas of expertise: Lived experience with sickle cell disease, patient advocacy, clinical trial participation, gene therapy awareness

LCDR Oluwabukola "Bukky" Akinsiku (Q&A Moderator)

Senior Advisor, Office of Minority Health
U.S. Department of Health & Human Services

LCDR Oluwabukola Akinsiku is a United States Public Health Service (USPHS) Commissioned Corps Officer dedicated to improving health outcomes for all populations and recognizes the integral and diverse roles that pharmacists can play in public health. She has had a variety of roles in healthcare including community pharmacist, pharmacy manager, and clinical pharmacist. She is currently a Senior Advisor in the Office of the Director at the Office of Minority Health. LCDR Akinsiku graduated from the University of Maryland Baltimore County with a Bachelor of Science Degree in Biology in 2004, and from Howard University School of Pharmacy with her doctorate in pharmacy in 2009. She is Board-Certified in Advanced Diabetes Management and Pharmacotherapy and completed a Graduate Certificate for Global Health from the Uniformed Services University in 2023.

Areas of expertise: Public health leadership, policy, global and population health, clinical pharmacy and pharmacotherapy, advance diabetes and chronic disease management.

Caroline Horrow

CMS Cell and Gene Therapy (CGT) Access Model Co-Lead,
Center for Medicare and Medicaid Innovation
Centers for Medicare & Medicaid Services

Caroline Horrow is the co-lead of the Cell and Gene Therapy (CGT) Access Model at the Centers for Medicare & Medicaid Services (CMS) Innovation Center. Before joining the Innovation Center, she was a postdoctoral research fellow with the Program On Regulation, Therapeutics And Law (PORTAL) at Brigham & Women's Hospital and Harvard Medical School, where her research focused on the regulation and reimbursement of precision medicine. She previously worked in health law at the National Institutes of Health, the U.S. House of Representatives, the Massachusetts State Senate, and the Center for Health Law & Policy Innovation, as well as bioethics research at Mayo Clinic. Caroline received her JD from Harvard Law School and a BSE in biomedical engineering from Duke University.

Areas of expertise: Cell and gene therapy policy; health law, biomedical engineering, genomic policy, bioethics

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