SICKLE CELL DISEASE AND GENE THERAPIES:

A TWO-PART WEBINAR SERIES

In recognition of 2025 National Sickle Cell Awareness Month (NSCAM), the US. Department of Health and Human Services (HHS) Office of Minority Health (OMH), in partnership with the Office for Human Research Protections (OHRP), hosted a two-part Gene Therapy Learning Series. The series explored ethical, clinical, and policy considerations for emerging gene therapies for sickle cell disease (SCD) and highlighted trust, access, affordability, and patient perspectives, including lived experiences from Sickle Cell Warriors and their caregivers.

This document provides a brief overview of key messages from the learning series and connects readers to trusted federal resources that support informed decisions about gene therapy and SCD care.

Theme	Key Messages and Resources
Gene Therapy Access for SCD	Approved by the FDA in December 2023[1], gene therapies for SCD offer a new curative option. For the nearly 50% of individuals with SCD covered by Medicaid[2], the CMS Cell and Gene Therapy (CGT) Access Model[3] helps reduce financial barriers and support access. Tools like Maya's Care Journey[4] can also help patients navigate the path to care.
Risks, Benefits, and Ethical Oversight	The decision to pursue gene therapy for SCD is a personal one, and risks and benefits should be made in partnership with a trusted provider. Strong ethical safeguards – such as those outlined in the Belmont Report[5] – are ways in which patients are protected while advancing gene therapy as a viable solution for those with SCD. Resources such as the Cure Sickle Cell Initiative[6], the Democratizing Education for Sickle Cell Disease Gene Therapy Project[7], NIH Clinical Research Trials and You[8], and the About Research Participation[9] initiative support informed, patient-centered decision-making about gene therapies, including clinical trial participation.
Building Trust Through Partnership	Providers can support informed choices about SCD therapeutics with transparency, patience, and respect for patient autonomy. Community-based organizations (CBOs) serve as trusted messengers, expanding awareness through culturally relevant education, helping families navigate needs such as travel and lodging, and strengthening trust between health systems and communities most affected by SCD. Federal programs, including the Health Resources & Services Administration's (HRSA) Sickle Cell Disease Treatment Demonstration Program (TDP) and the Sickle Cell Disease Newborn Screening Follow-up Program (FP), support these efforts by funding SCD Partners[10] across the United States.
Coverage and Affordability	Gene therapy is costly, making state-federal collaboration essential to manage costs and support long-term care. CMS' CGT Access Model[3] helps to improve affordability and access by allowing the federal government to negotiate outcomes-based agreements with manufacturers on behalf of state Medicaid agencies. Curative treatment does not automatically affect coverage, as coverage decisions depend on multiple medical and financial factors, and vary by state. Beyond the CGT Access Model[3] public and private coverage policies for SCD gene therapies continue to evolve as access expands and sustainable approaches to payment are being developed.

Comprehensive Whole-Person Care and Psychosocial Support	While gene therapy can greatly improve pain, organ function, and daily life, individuals may still experience challenges related to identity, mental health, and survivor's guilt. These experiences highlight the need for coordinated, comprehensive care that supports the whole person before, during, and after gene therapy. The National Human Genome Research Institute, through the Democratizing Education for Sickle Cell Disease Gene Therapy Project[7], offers mental health-focused educational materials, such as the Your Mental Health and Gene Therapy[11].

For more information, please visit HHS OMH's Sickle Cell Disease landing page at https://minorityhealth.hhs.gov/sickle-cell-disease[12].

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3. Centers for Medicare & Medicaid Services. (2026, January 20). *Cell and gene therapy access model*. https://www.cms.gov/priorities/innovation/innovation-models/cgt
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12. U.S. Department of Health & Human Services, Office of Minority Health. (2025, September). *Sickle cell disease*. https://minorityhealth.hhs.gov/sickle-cell-disease

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