





SICKLE CELL DISEASE AND GENE THERAPIES: A TWO-PART WEBINAR SERIES (SEPTEMBER 2025)

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 in September 2025. 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
<p data-bbox="207 695 418 758">Gene Therapy Access for SCD</p> 	<p data-bbox="475 678 1463 846">Approved by the FDA in December 2023¹, gene therapies for SCD offer a new curative option. For the nearly 50% of individuals with SCD covered by Medicaid², the CMS Cell and Gene Therapy (CGT) Access Model³ helps reduce financial barriers and support access. Tools like Maya's Care Journey⁴ can also help patients navigate the path to care.</p>
<p data-bbox="175 947 443 1010">Risks, Benefits, and Ethical Oversight</p> 	<p data-bbox="475 867 1463 1035">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 Report⁵ – are ways in which patients are protected while advancing gene therapy as a viable solution for those with SCD.</p> <p data-bbox="475 1056 1463 1224">Resources such as the Cure Sickle Cell Initiative⁶, the Democratizing Education for Sickle Cell Disease Gene Therapy Project⁷, NIH Clinical Research Trials and You⁸, and the About Research Participation⁹ initiative support informed, patient-centered decision-making about gene therapies, including clinical trial participation.</p>
<p data-bbox="167 1346 451 1409">Building Trust Through Partnership</p> 	<p data-bbox="475 1241 1463 1451">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.</p> <p data-bbox="475 1472 1463 1640">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 Partners¹⁰ across the United States.</p>
<p data-bbox="215 1724 402 1787">Coverage and Affordability</p> 	<p data-bbox="475 1650 1463 1818">Gene therapy is costly, making state-federal collaboration essential to manage costs and support long-term care. CMS' CGT Access Model³ helps to improve affordability and access by allowing the federal government to negotiate outcomes-based agreements with manufacturers on behalf of state Medicaid agencies.</p> <p data-bbox="475 1839 1463 2007">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 Model³ public and private coverage policies for SCD gene therapies continue to evolve as access expands and sustainable approaches to payment are being developed.</p>

**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>
4. Centers for Medicare & Medicaid Services. (n.d.). *Improved access to gene therapy for sickle cell disease: Maya's care journey* [PDF]. U.S. Department of Health and Human Services. <https://www.cms.gov/files/document/cgt-model-journey-map.pdf>
5. National Commission for the Protection of Human Subjects of Biomedical and Behavioral Research. (1979, April 18). *The Belmont report: Ethical principles and guidelines for the protection of human subjects of research*. U.S. Department of Health and Human Services. <https://www.hhs.gov/ohrp/regulations-and-policy/belmont-report/index.html>
6. National Heart, Lung, and Blood Institute. (n.d.). *Cure sickle cell initiative*. <https://curesickle.org/>
7. National Human Genome Research Institute. (2024, September 12). *Sickle cell disease gene therapy education project*. National Institutes of Health. <https://www.genome.gov/research-at-nhgri/Projects/Democratizing-Education>
8. National Institutes of Health. (2025, June 23). *NIH clinical research trials and you*. U.S. Department of Health & Human Services. <https://www.nih.gov/health-information/nih-clinical-research-trials-you>
9. Office for Human Research Protections. (2023, May 10). *About research participation*. U.S. Department of Health and Human Services. <https://www.hhs.gov/ohrp/education-and-outreach/about-research-participation/index.html>
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11. National Human Genome Research Institute. (2024, December 12). *Your mental health and gene therapy*. National Institutes of Health. <https://www.genome.gov/research-at-nhgri/Projects/Democratizing-Education/your-mental-health-scd-gene-therapy>
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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