

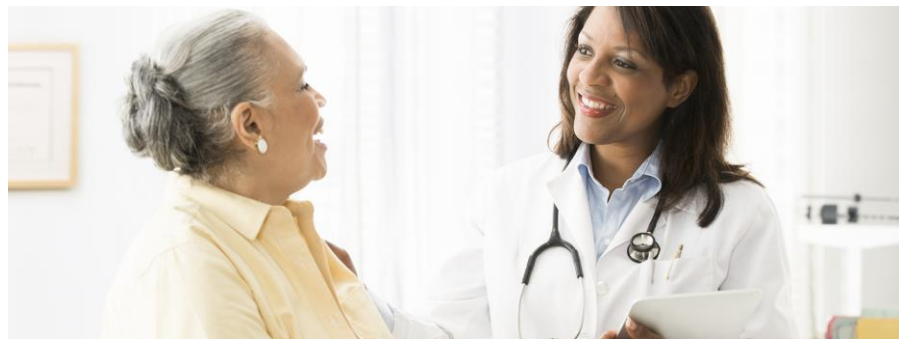
Executive summary: Vizient sickle cell disease state strategy

Guiding health systems through the clinical, financial and operational impact of next-generation therapies

Strategic implications for health systems

- **Evaluate patient populations:** Use clinical data to forecast eligibility and prepare care pathways.
- **Engage early with manufacturers:** Certification as a treatment center requires significant lead time and negotiation.
- **Partner with payers:** Explore innovative contracting models, including outcomes-based agreements (OBAs).
- **Plan operational readiness:** Build infrastructure for cell collection, monitoring and biosafety handling.
- **Consider equity:** Ensure Medicaid and underserved patients have equitable access, given demographic concentration.
- **Leverage Vizient's Advanced Therapy Solutions:** Vizient offers resources and expertise to help members assess emerging therapies, anticipate financial impact and prepare for evolving policy and payer landscapes. By connecting with Vizient's national network, health systems can strengthen readiness and expand patient access to these transformative treatments.

Learn more at vizientinc.com/what-we-do/pharmacy/cell-and-gene-therapy



Burden of Sickle Cell Disease (SCD)

Sickle Cell Disease (SCD) is the most common inherited blood disorder, affecting approximately 120,000 people in the U.S. and more than 500,000 newborns globally each year. The disease disproportionately impacts non-Hispanic Black and African American populations (90% of U.S. cases), with the highest prevalence in Southern states such as Florida, New York, Texas and Georgia.

Life expectancy remains significantly reduced at approximately 54 years. Recurrent vaso-occlusive crises (VOCs) drive high morbidity, frequent acute care utilization and lifetime healthcare costs exceeding \$3.8M per patient.

Transformative innovation: Gene therapy for SCD

The FDA has approved two landmark gene therapies—Casgevy and Lyfgenia—that represent one-time, potentially curative treatments.

Casgevy (Vertex/CRISPR Therapeutics): CRISPR/Cas9 increases fetal hemoglobin (HbF) to reduce sickling; 97% VOC-free for 12 months in Phase 3 trials. List price: ~\$2.2M.

Lyfgenia (bluebird bio): Lentiviral vector delivers a functional anti-sickling hemoglobin gene; 88% achieved complete VOC resolution in Phase 1|2 trials. List price: ~\$3.1M; carries a boxed warning for secondary malignancy.

Both therapies require myeloablative conditioning, prolonged hospitalization, intensive care coordination and 15 years of mandated follow-up.

Vizient CDB data reveals approximately 19,000 **patients in the U.S. may be eligible for sickle cell gene therapy**—highlighting both the opportunity and the challenge for health systems.

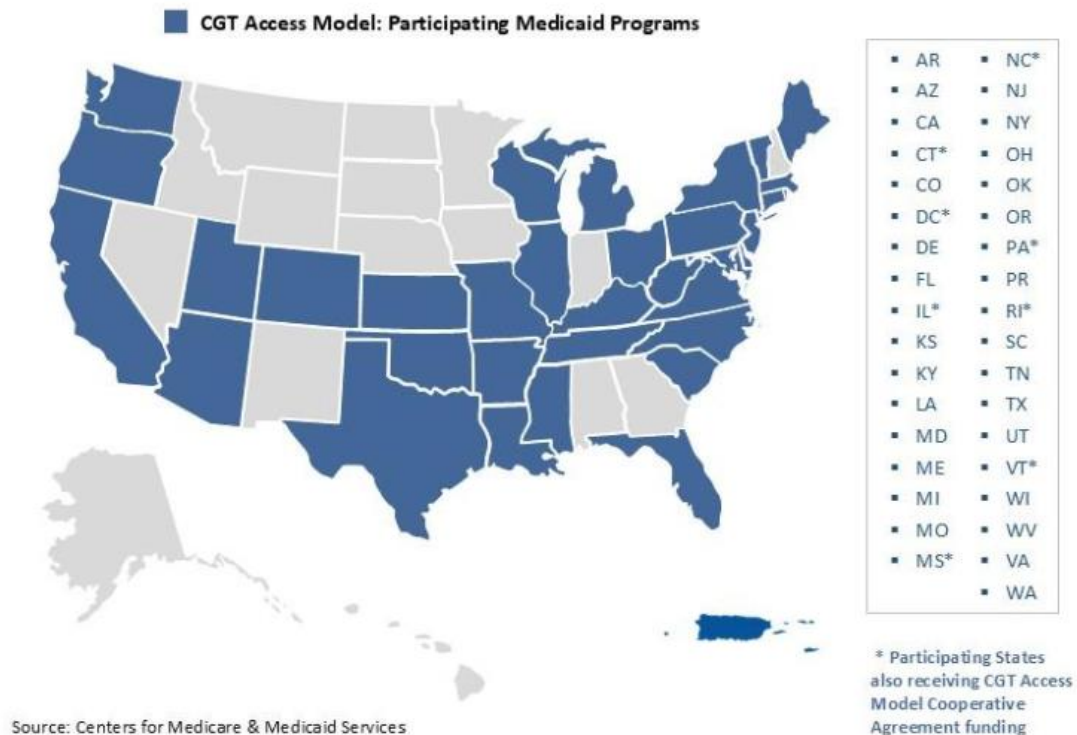
Access and delivery considerations

Delivering gene therapy for SCD requires extensive coordination across specialized centers, payers and care teams—making access as much an operational challenge as a scientific breakthrough.

- **Treatment centers:** Only offered at certified Authorized/Qualified Treatment Centers (ATCs/QTCs); 73 U.S. centers are certified as of 2025.
- **Operational complexity:** Includes fertility counseling, stem cell mobilization/collection, manufacturing lead times (10–26 weeks) and specialized biosafety handling.
- **Infrastructure needs:** Institutions must secure agreements with manufacturers and establish biosafety protocols for genetically modified products.
- **Patient access barriers:** Geographic distribution, long treatment timelines and extended follow-up requirements may delay access, especially in underserved regions.

Payer and policy landscape and future modeling

Payer coverage is variable. The Center for Medicare & Medicaid Services (CMS) has launched the Cell and Gene Therapy Access Model, a landmark outcomes-based framework to expand equitable access while managing financial risk. Medicaid is estimated to cover over half of SCD patients in the U.S. Vizient data and Sg2 modeling forecast evolving care dynamics: modest short-term increases in inpatient utilization, earlier adoption and reduced admissions among pediatric patients, and longer-term declines in inpatient discharges as gene therapies reshape the treatment paradigm.



Source: <https://www.cms.gov/cell-and-gene-therapy-cgt-access-model>



To learn more, contact
pharmacyquestions@vizientinc.com

As the nation's largest member-driven healthcare performance improvement company, Vizient provides solutions and services that empower healthcare providers to deliver high-value care by aligning cost, quality and market performance. With analytics, consulting and a robust sourcing portfolio, we help healthcare organizations improve patient outcomes and lower costs.