


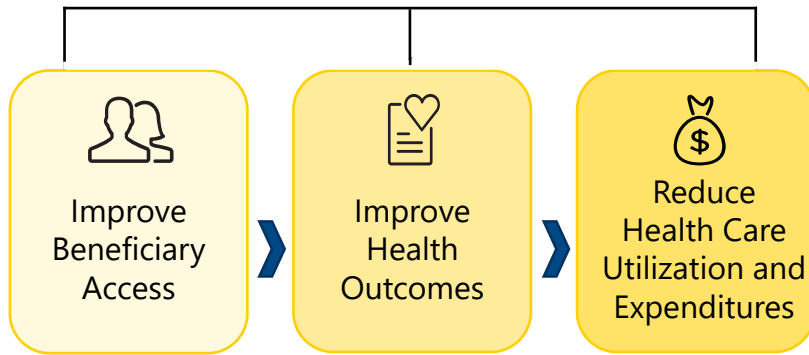
Cell and Gene Therapy (CGT) Access Model Overview Factsheet

CGT ACCESS MODEL PURPOSE

The Cell and Gene Therapy (CGT) Access Model seeks to test whether a CMS-led approach to negotiating and administering outcomes-based agreements (OBAs) for cell and gene therapies, in the context of a comprehensive strategy for addressing a range of barriers to equitable access to cell and gene therapies, will improve access and health outcomes for people with Medicaid, and reduce health care costs.



 **Cell and Gene Therapies (CGTs)** are a growing class of transformative, one-time medicines designed to treat previously intractable diseases.

Model Goals



Sickle Cell Disease (SCD)

The model will focus initially on CGTs for SCD, a genetic blood disorder that affects 100,000+ people in the U.S., the majority of whom are Black Americans. People with SCD have:

-  An average lifespan more than 20 years shorter than average life expectancy in the U.S.
-  Excruciating pain episodes, which can cause multiple hospitalizations.

CGT ACCESS MODEL PARTICIPANTS



States

All states and territories that participate in the Medicaid Drug Rebate Program (MDRP) can participate in the model if they meet requirements.

States will be able to express their intent to participate by submitting a Letter of Intent (LOI) by April 2024. States may then apply to the model by responding to a Request for Applications (RFA) by February 2025. After states sign an agreement with CMS, states may begin participation in the model between January 2025 and January 2026.



MANUFACTURERS

Manufacturers will be able to apply to the model by responding to a RFA by May 2024. Manufacturers who participate in the MDRP and market U.S. Food & Drug Administration (FDA)-approved or -licensed gene therapies for the treatment of severe SCD are also eligible to participate in the model. Negotiations between CMS and manufacturers are scheduled to take place between May - November 2024.



PROVIDERS

Providers will not be participants in the model.

MODEL POPULATION



The model population is beneficiaries for whom Medicaid is the primary payer and Medicaid expansion Children's Health Insurance Program (CHIP) beneficiaries ("Title XIX beneficiaries") in fee-for-service and Medicaid managed care.

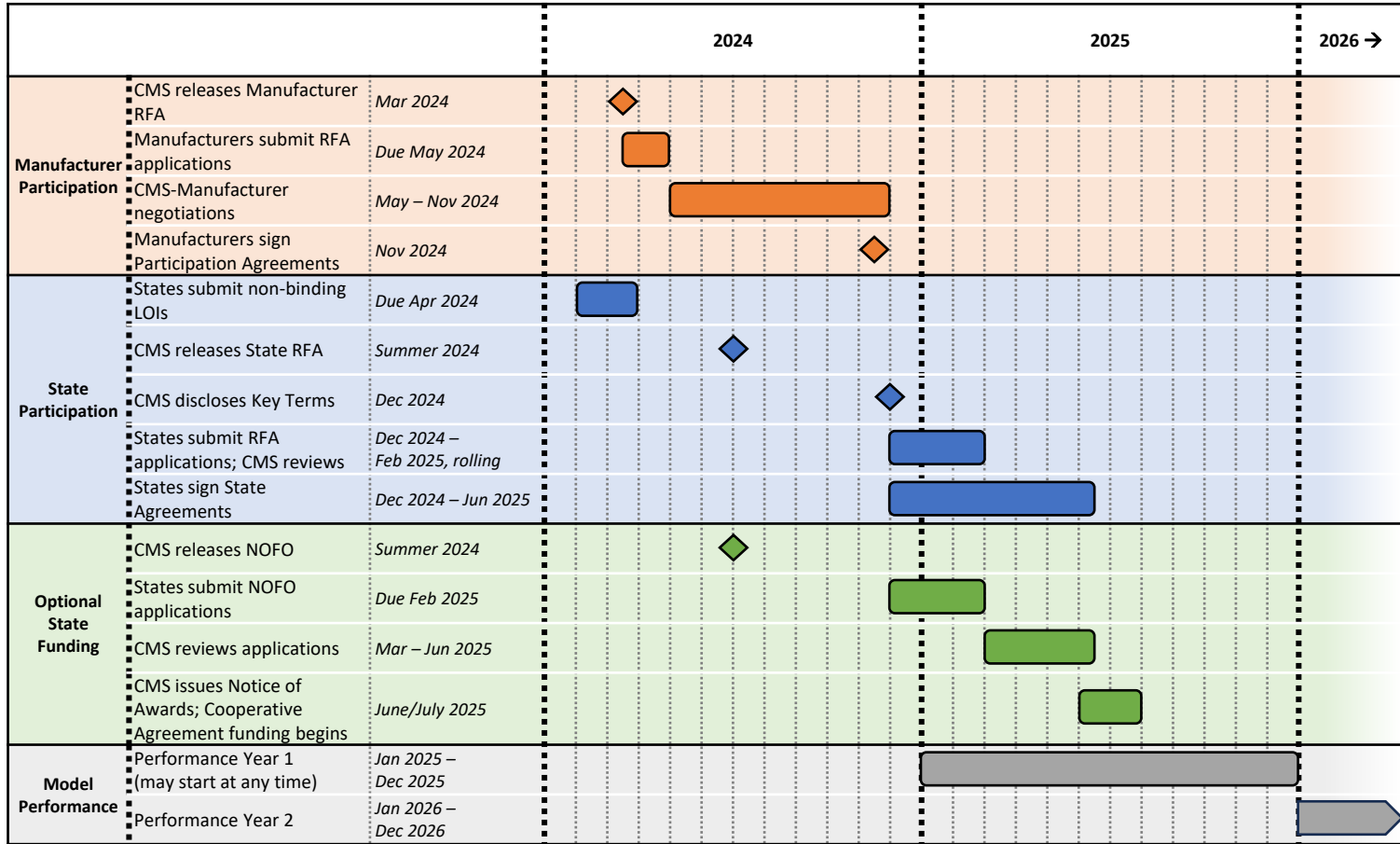
Manufacturers and states will have the option to include separate CHIP beneficiaries ("Title XXI beneficiaries") alongside Title XIX beneficiaries.

Beneficiaries must receive an FDA-approved CGT for SCD that is covered and paid for by either (1) a participating state as a covered outpatient drug, or (2) a CHIP that participates in the model.

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APPLICATION PROCESS AND TIMELINE

The CGT Access Model will operate for up to approximately 11 years, depending on the OBA term for each state. States may apply to the Notice of Funding Opportunity (NOFO) to receive optional funding under the model.



Legend ● Manufacturer activities ● State activities ● Funding timeline ● Model performance timeline

HEALTH EQUITY & IMPACT ON BENEFICIARIES

The CGT Access Model aims to support beneficiaries and address health equity, in alignment with the [CMS Framework for Health Equity](#) in three ways:

COST

Lower the cost of CGTs and enable more people with Medicaid to access potentially transformative treatment.

HEALTH DISPARITIES

Increase access to potentially transformative therapies for all individuals with SCD, including groups who have experienced historic disparities associated with this disease.

BENEFICIARY IMPACT

- Increased access to transformative therapies for SCD
- Reduced burden of SCD for beneficiaries
- Improved quality of life, including the ability to achieve major life goals related to education, work, and family life
- Easier navigation of care due to streamlined authorization process

ACCESS BARRIERS

Offer states funding for activities that reduce access barriers for people with Medicaid. Require manufacturers to cover certain fertility preservation services, because the care journey for SCD CGT typically results in infertility. Lack of access to fertility preservation services presents a significant access barrier to individuals considering CGT.

MODEL RELATIONSHIPS

The CGT Access Model seeks to test whether a CMS-led approach to negotiating and administering OBAs for cell and gene therapies, in the context of a comprehensive strategy for addressing a range of barriers to equitable access to cell and gene therapies, will improve access and health outcomes for people with Medicaid, and reduce health care costs.



CMS AND MANUFACTURERS

CMS will negotiate key terms for an OBA with manufacturers. Manufacturers will in turn make the negotiated OBA available to participating states. Throughout the model, manufacturers will submit patient-level sales data to CMS to cross-check against claims data of patients who receive CGT.



CMS AND STATES

CMS and states will have an arrangement wherein:

1. States will provide data to CMS. CMS will use submitted claims data in the Transformed Medicaid Statistical Information System for model operations and analysis.
2. CMS will provide states with optional funding to support activities that promote equitable access to care.
3. States will be responsible for the cost of the cell and gene therapy, but at a discounted price tied to specific outcomes, as negotiated by CMS.



STATES AND MANUFACTURERS

The contract between states and manufacturers, with key terms as negotiated by CMS on behalf of states, will be structured as a supplemental rebate agreement. States and manufacturers will have the option to include separate CHIP programs that will be subject to different considerations.

Within this agreement, manufacturers will be obligated to provide states with supplemental rebates that reflect model-negotiated terms (i.e., pricing, access standards, outcomes). In turn, states will be obligated to implement an agreed-upon standard access policy.

Model Contact Information and Resources

[CMS Blog Article for Sickle Cell Disease Month](#)
[CMS Sickle Cell Disease Action Plan](#)