

Cell & Gene Therapy (CGT) Access Model Overview Webinar

Center for Medicare and Medicaid Innovation
February 6, 2024

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Agenda

This webinar provides an introduction to the CGT Access Model. The following topics will be discussed:

1 | Welcome and Introductions

2 | Model Background and Overview

3 | Addressing Health Equity

4 | Model and Contracting Structure

5 | Model and Application Timeline

6 | Question and Answer Session

7 | Closing and Resources

Welcome and Introductions

Today's Presenters



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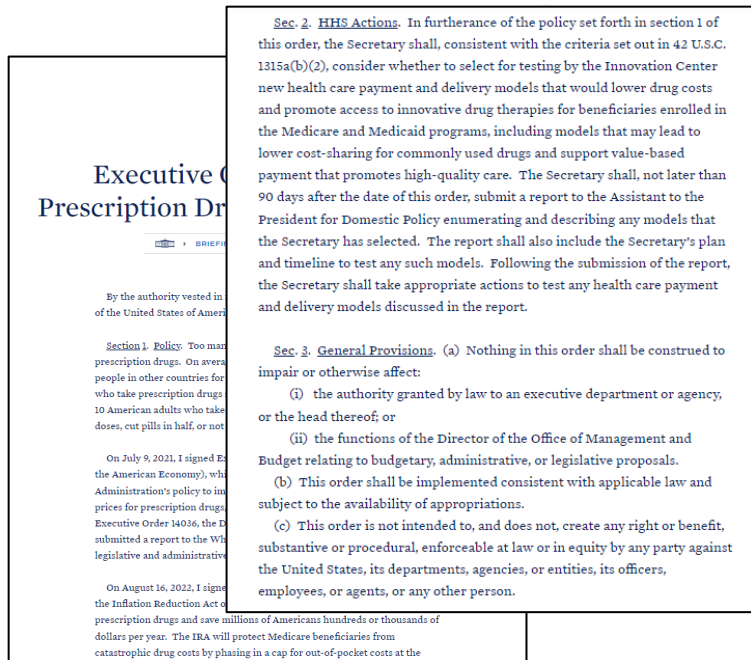
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Model Background and Overview

Model Background

The CGT Access Model aims to reduce healthcare costs by facilitating outcomes-based agreements between manufacturers and states.

The Cell and Gene Therapy (CGT) Access Model was developed in response to **President Biden's Executive Order 14087, Lowering Prescription Drug Costs for Americans** and intends to drive down prescription drug costs, building on the Inflation Reduction Act.



The model is a framework wherein **CMS negotiates with manufacturers on behalf of states** for outcomes-based agreements, or OBAs, for CGTs that cover beneficiaries for whom Medicaid is the primary payer.

MODEL GOALS



Improve Beneficiary Access to Transformative CGT Therapies



Reduce Long-Term Health Care Utilization and Expenditures



Improve Health Outcomes

Overview of Cell & Gene Therapies

CGTs are a rapidly growing class of one-time treatments, many of which are developed to treat rare and severe diseases.

Cell therapy aims to treat diseases by altering sets of cells in the body or by using cells to carry a therapy through the body.

Gene therapy aims to treat diseases by replacing, inactivating, or introducing genes into cells.

Though CGTs hold great potential, they often cost millions of dollars.

To help states and beneficiaries gain access to these treatments, CMS will:



Negotiate with manufacturers on behalf of states for outcomes-based agreements which tie payment to specific outcomes.



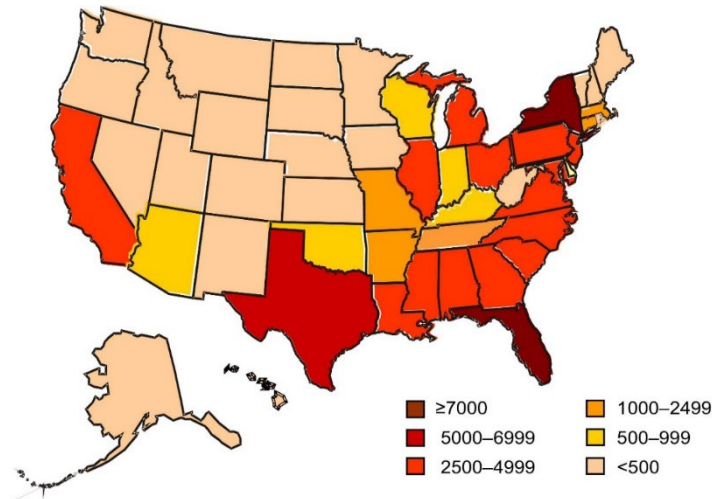
Negotiate for discounted prices and develop a broader strategy to address barriers to equitable care.

Overview of Sickle Cell Disease





CMS is initially focusing the CGT Access Model on gene therapies for sickle cell disease (SCD) to increase access to potentially curative therapies for all individuals with SCD for whom gene therapy may be an appropriate option.

Description

SCD is a genetic blood disorder that affects 100,000+ people in the U.S., the majority of whom are Black Americans. This disease is unevenly spread across the U.S., as shown in the state-by-state patient counts to the right.



Biopsychosocial Challenges

-  The lifelong effects of SCD result in individuals' lifespans being reduced by 20+ years compared to average life expectancy in the U.S.
-  Individuals have excruciating pain episodes leading to multiple hospitalizations per year and the need for prescription pain medication.
-  Frequent pain has broad effects on a patient's life, impacting educational attainment and employment.
-  SCD gives rise to other conditions, such as mental health challenges.

Potential of CGTs

On December 8, 2023, the FDA approved two gene therapies for SCD, Casgevy and Lyfgenia.

Both products hold the promise of dramatically improving the lives of people with SCD by potentially **reducing or fully eliminating the occurrence of severe pain crises.**

Addressing Health Equity

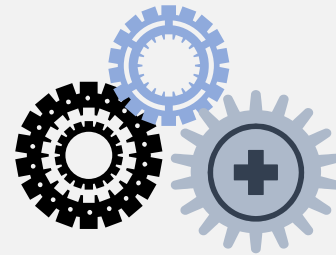
Addressing Health Equity

The CGT Access Model aims to enable states to improve equitable access to included CGTs for all eligible Medicaid beneficiaries, in three key areas.

COST



- Contracts between states and manufacturers reflecting CMS-negotiated key terms will potentially lower the cost of CGTs and enable more Medicaid beneficiaries to access potentially transformative treatment.



- States will be offered **optional funding for activities that reduce access barriers** for people with Medicaid.
- Manufacturers will be required to cover a defined scope of fertility preservation services.
- Test may yield learning to help inform state Medicaid agencies' future decisions regarding coverage for fertility preservation services in connection with CGT.

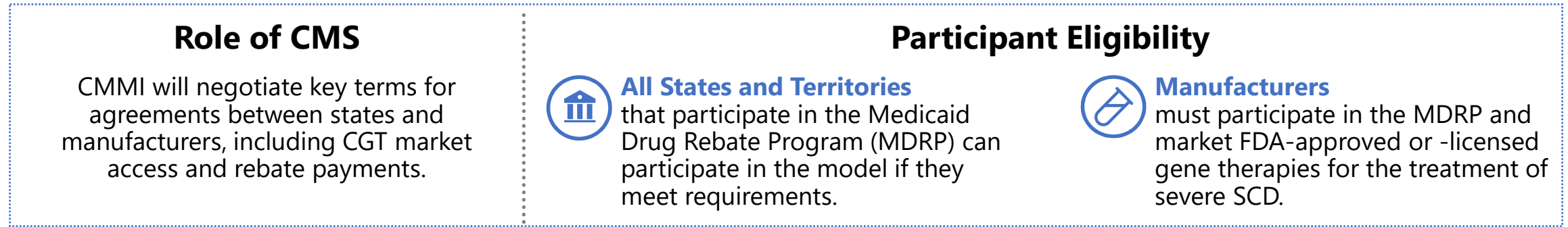


- Racial bias and treatment disparities are present among individuals with SCD, that have limited access to specialized care and treatments.
- By **increasing access to transformative therapies** for SCD, the Model could help address these historic disparities, poor health outcomes, and low life expectancy.

Model and Contracting Structure

Model Structure

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



CMS will negotiate discounted pricing

with manufacturers to relieve the burden on states and increase access for beneficiaries.



CMS will tie manufacturer payment to specific outcomes,

such as reduction in pain-crises and patient-reported outcomes.



CMS will offer optional funding

to states to support implementation and activities that promote equitable access to care.



CMS will support states to operationalize the model,

such as providing technical assistance, specifying requirements on data collection, and collecting clinical and claims outcomes.

Contracting Structure

CMS will facilitate negotiations between states and pharmaceutical manufacturers.

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 would 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 their share of 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.



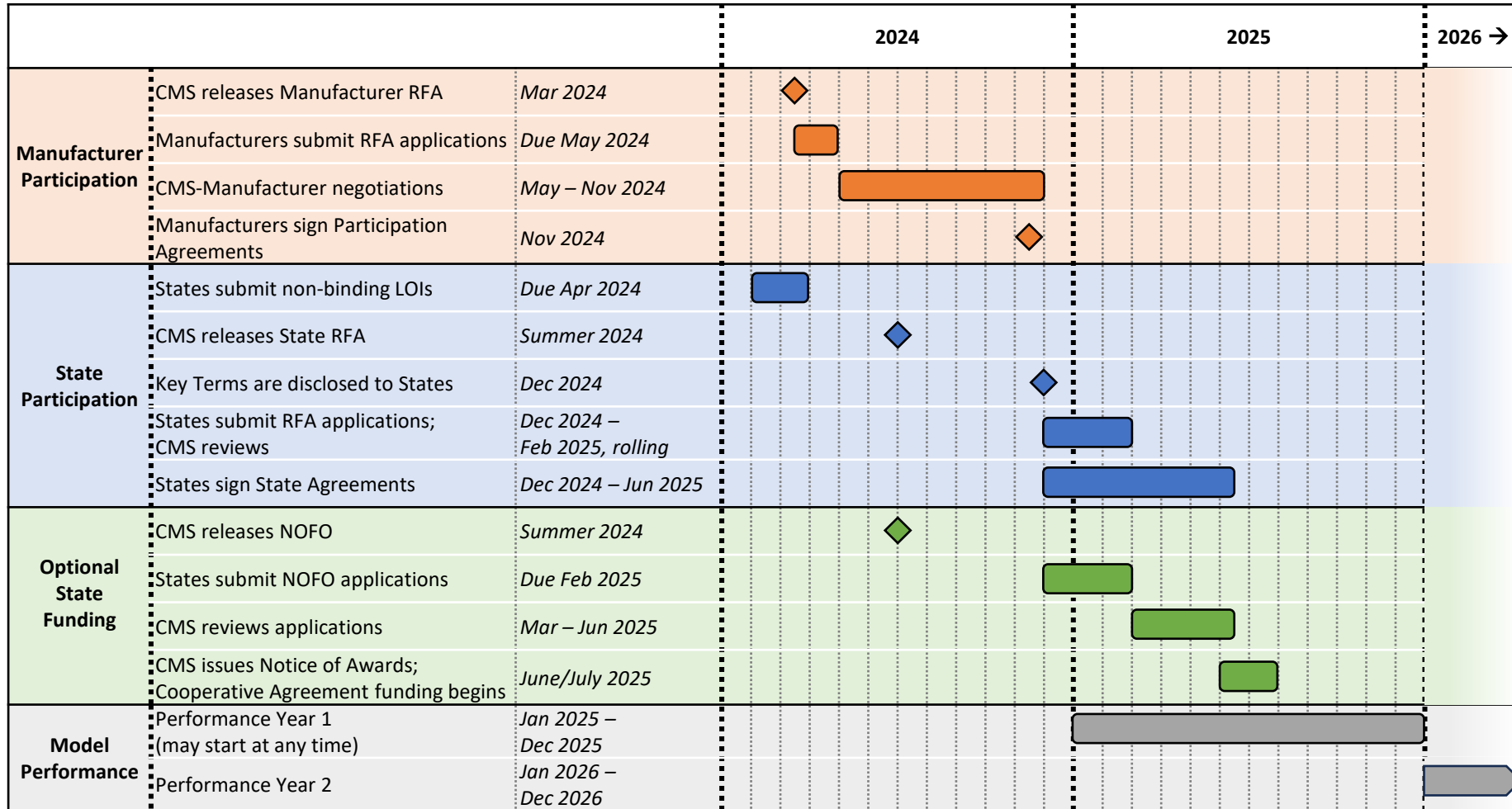
BENEFICIARY IMPACT

- 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

Model and Application Timeline

Model and Application Timeline

The CGT Access Model will operate for up to 11 years, depending on the OBA term for each state.



LEGEND

- Manufacturer activities
- State activities
- Funding timeline
- Model performance timeline

Model Resources

The CGT Access Model team has a host of resources to support interested organizations. To see the latest resources, visit the model's website at <https://www.cms.gov/priorities/innovation/innovation-models/cgt>.

The image shows a factsheet and infographic for the Cell and Gene Therapy (CGT) Access Model. The factsheet is titled "Cell and Gene Therapy (CGT) Access Model Overview Factsheet" and includes sections for "CGT ACCESS MODEL PURPOSE", "Cell and Gene Therapies (CGTs)", "Model Goals", "CGT ACCESS MODEL PARTICIPANTS", and "MODEL POPULATION". The infographic, titled "Cell and Gene Therapy Access Model", includes a "Rolling start for states: January 2025 to January 2026" and "The model goals are to:" section with three bullet points: "Increase access for people with Medicaid", "Improve health outcomes", and "Reduce health care utilization and expenditures". It also features a "Sickle Cell Disease" section with three bullet points: "Affects more than 100,000 people in the U.S. - the majority of whom are Black Americans", "Costs the health care system \$3 billion each year", and "50-60% of people with sickle cell disease are enrolled in Medicaid".



Model Factsheet and Infographic

Read through the [CGT Model Overview Factsheet](#) and the [CGT Model Infographic](#) on the model website to learn more.

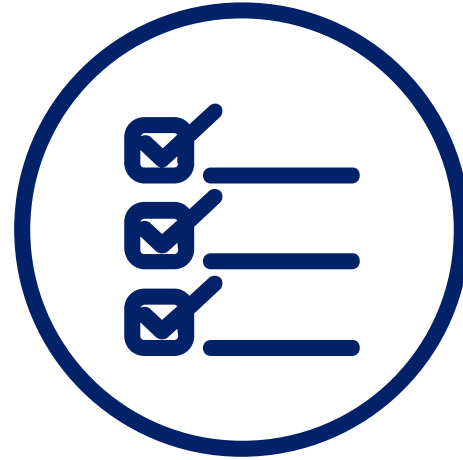


Helpdesk

If you have questions for the model team, please reach out to us via email at to CGTModel@cms.hhs.gov.

Question and Answer Session

Please Complete Our Survey



We appreciate your input!

Please click the link posted in the chat to take our survey.

We would love to learn how to make our events better.

Question & Answer Session



Open Q&A

Please **submit questions via the Q&A pod** to the right of your screen.
Specific questions about your organization can be submitted to
CGTModel@cms.hhs.gov.

Closing & Resources

Thank You for Attending this Webinar



We appreciate your time and interest!

Please take the survey following this webinar so we can learn how to make our events better.

Do you have questions? Email your comments and feedback to to CGTModel@cms.hhs.gov
with subject line ***CGT Access Model Overview Webinar***

THANK YOU!