Cell & Gene Therapy (CGT) Access Model Overview Webinar

Center for Medicare and Medicaid Innovation
February 6, 2024

CMS
CENTERS FOR MEDICARE & MEDICAID SERVICES

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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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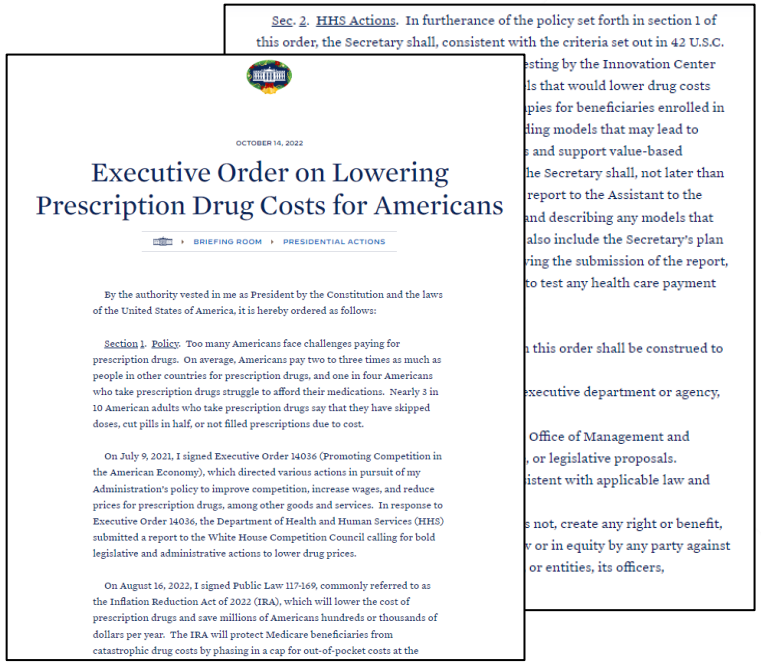
Model Background and Overview

Model Background

The CGT Access Model aims to reduce healthcare costs by creating 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.



OCTOBER 14, 2022

Executive Order on Lowering Prescription Drug Costs for Americans

BRIEFING ROOM › PRESIDENTIAL ACTIONS

By the authority vested in me as President by the Constitution and the laws of the United States of America, it is hereby ordered as follows:

Section 1. Policy. Too many Americans face challenges paying for prescription drugs. On average, Americans pay two to three times as much as people in other countries for prescription drugs, and one in four Americans who take prescription drugs struggle to afford their medications. Nearly 3 in 10 American adults who take prescription drugs say that they have skipped doses, cut pills in half, or not filled prescriptions due to cost.

On July 9, 2021, I signed Executive Order 14036 (Promoting Competition in the American Economy), which directed various actions in pursuit of my Administration's policy to improve competition, increase wages, and reduce prices for prescription drugs, among other goods and services. In response to Executive Order 14036, the Department of Health and Human Services (HHS) submitted a report to the White House Competition Council calling for bold legislative and administrative actions to lower drug prices.

On August 16, 2022, I signed Public Law 117-169, commonly referred to as the Inflation Reduction Act of 2022 (IRA), which will lower the cost of prescription drugs and save millions of Americans hundreds or thousands of dollars per year. The IRA will protect Medicare beneficiaries from catastrophic drug costs by phasing in a cap for out-of-pocket costs at the

Sec. 2. HHS Actions. In furtherance of the policy set forth in section 1 of this order, the Secretary shall, consistent with the criteria set out in 42 U.S.C. esting by the Innovation Center ls that would lower drug costs pies for beneficiaries enrolled in ding models that may lead to s and support value-based he Secretary shall, not later than report to the Assistant to the nd describing any models that also include the Secretary's plan ring the submission of the report, to test any health care payment

this order shall be construed to

executive department or agency,

Office of Management and , or legislative proposals. istent with applicable law and

s not, create any right or benefit, or in equity by any party against or entities, its officers,

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 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.

Fast Stats

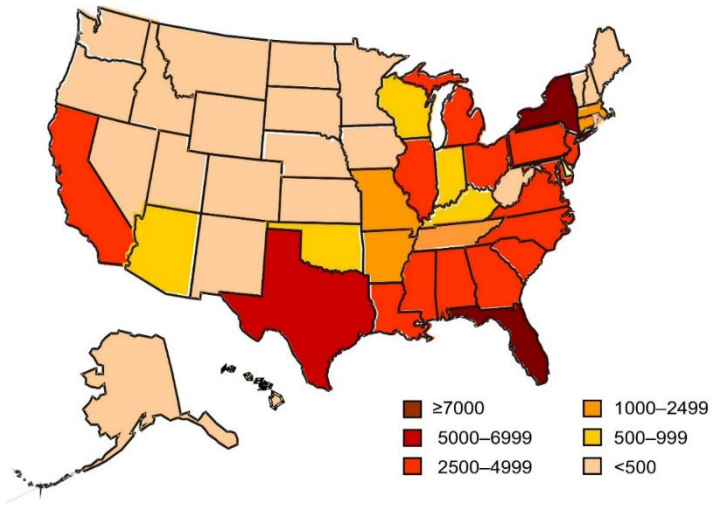
100k+
People affected in the U.S.

~60%
Of people with SCD are enrolled in Medicaid

$2.98B
In costs per year to the U.S. health system *(mostly accrued to Medicaid)*

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

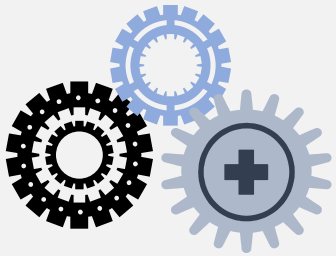
Addressing Health Equity

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

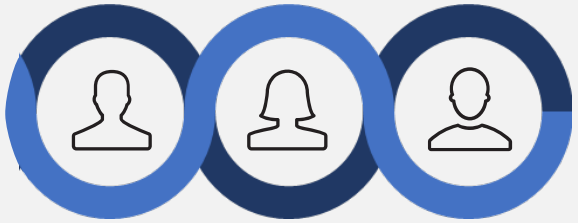
- Contracts between states and manufacturers reflecting CMS-negotiated key terms will potentially lower the cost of CGTs and enable more Medicaid beneficiaries to access potentially transformative treatment.

ACCESS BARRIERS



- 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,** as the care journey for SCD CGT typically results in infertility, which presents a significant access barrier.

HEALTH DISPARITIES



- 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, 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.

Role of CMS

CMMI will negotiate key terms and agreements between states and manufacturers, including CGT market access and rebate payments.

Participant Eligibility

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

Manufacturers must participate in the MDRP and market FDA-approved or -licensed gene therapies for the treatment of severe SCD.

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 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 negotiating the OBAs as well as 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 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 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.

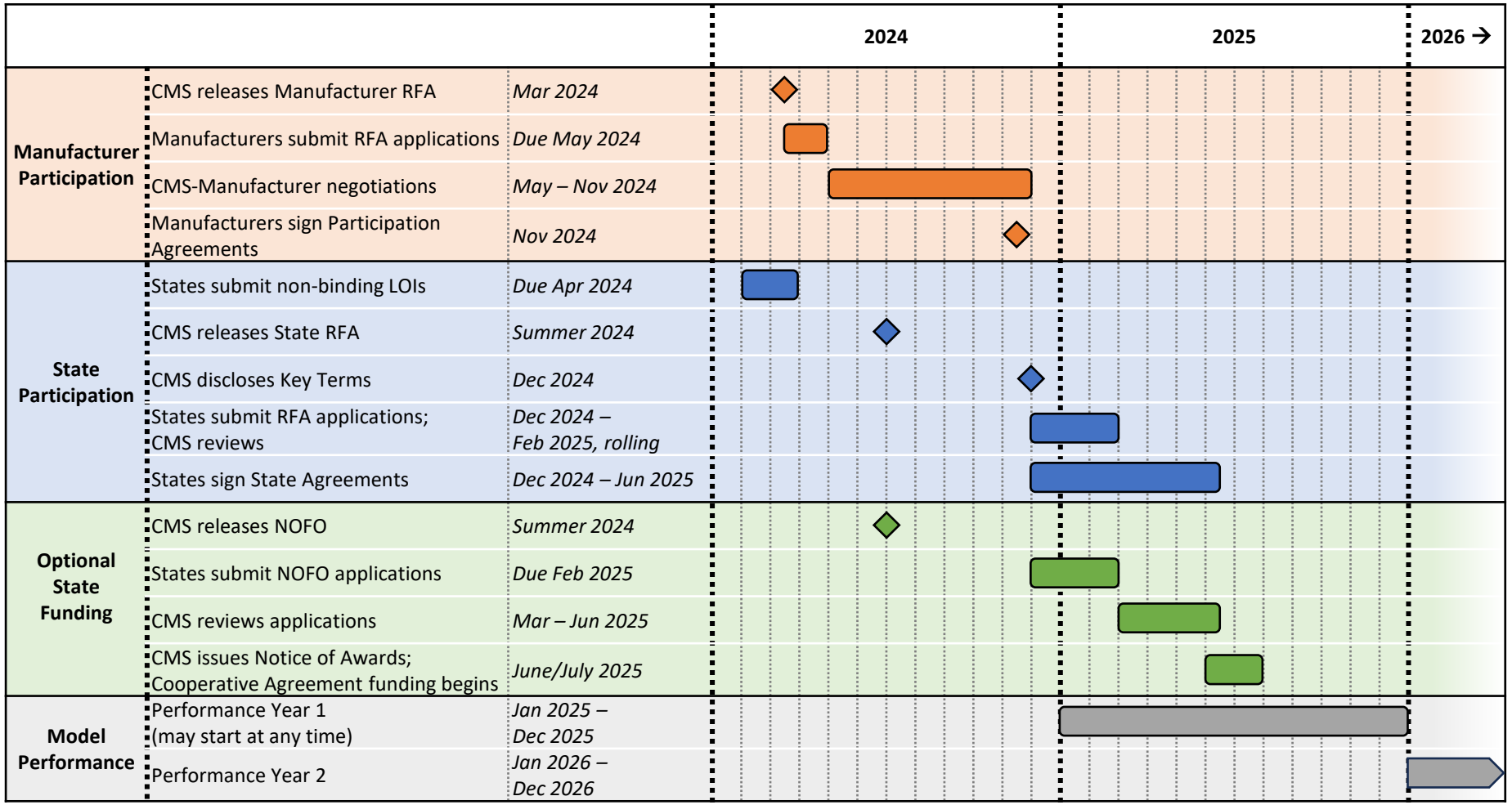
BENEFICIARY IMPACT

- 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.

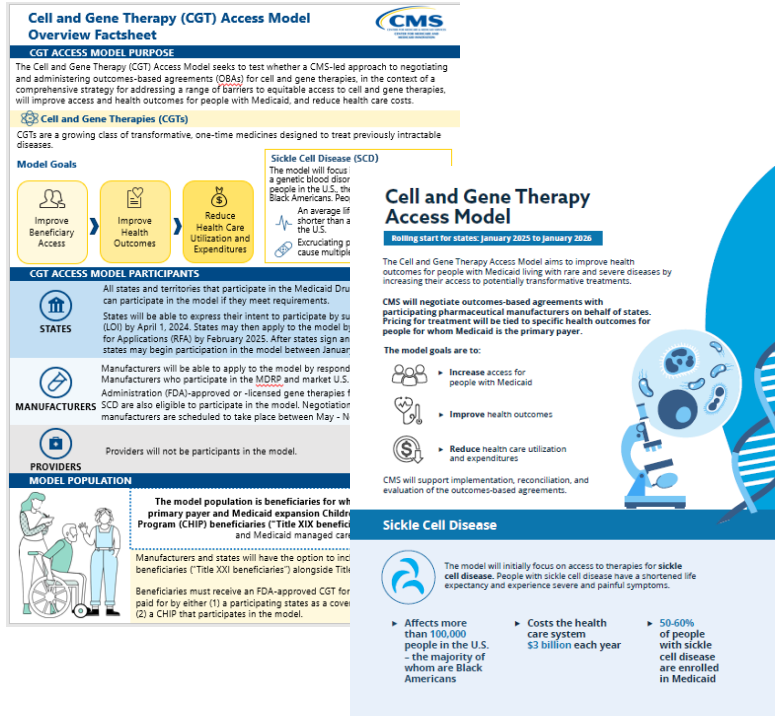


LEGEND

- 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.

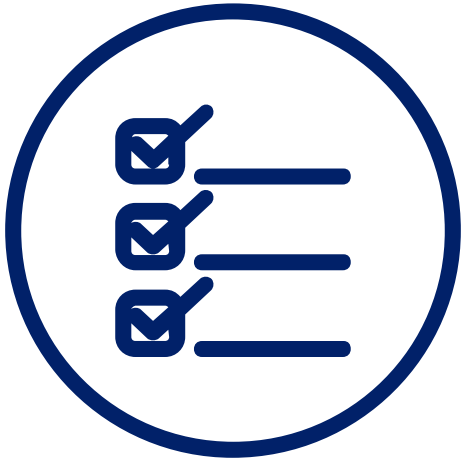


Model Factsheet and Infographic
Read through the CGT Model Overview Factsheet and the CGT Model Infographic on the model website to learn more.

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!