Cell and Gene Therapy (CGT) Access Model Overview for States

Center for Medicare and Medicaid Innovation
February 8, 2024
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Agenda

1. Welcome and Introductions
2. Model Overview
3. Why States Should Join
4. Potential Outcomes-Based Agreement Structure
5. Ensuring Equitable Access to Sickle Cell Disease Gene Therapy Care
6. State Participation
7. Model Timeline
8. Question & Answer Session
9. Closing & Resources
Welcome and Introductions
Today’s Objectives
This webinar will address the following questions:

**MODEL FEATURES**

- How will the CGT Access Model work?
- What might the CGT Access Model's negotiated deal look like?

**STATE PARTICIPATION & SUPPORT**

- Why should states join the CGT Access Model?
- How else does the CGT Access Model help states ensure access to gene therapy?
- What would states have to do to participate in the CGT Access Model?
- How can states join the CGT Access Model?
Model Overview
The Cell and Gene Therapy (CGT) Access Model is a framework wherein CMS negotiates with manufacturers on behalf of states for outcomes-based agreements (OBAs) for CGTs that cover Medicaid enrollees.

- CMS directly negotiates Key Terms of OBAs with manufacturers
- States decide whether to enter into agreements with manufacturers regarding OBA Key Terms
- CMS supports implementation, reconciliation, and evaluation
State Eligibility

Which states are eligible to participate in the CGT Access Model?

Who Can Apply

All states and territories that participate in the Medicaid Drug Rebate Program (MDRP) are encouraged to apply for the model.

Letter of Intent

States are encouraged to provide input on the model by meeting with the model team and submitting non-binding letters of intent (LOIs).

Model Start

States can apply to the model beginning in December 2024. The model will begin in 2025 with a “rolling start” – states can choose to begin participation from January 1, 2025, to January 1, 2026.
Model Populations

The CGT Access Model will focus on Medicaid beneficiaries with sickle cell disease (SCD) in participating states.

Primary Population

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.*

*The Model includes an option for manufacturers and states to include separate Title XXI CHIP beneficiaries through separate agreements.

Eligible Beneficiaries

Beneficiaries in the model population with sickle cell disease (SCD) who receive a gene therapy made by a participating manufacturer.

Jan 1, 2025

During the “rolling start” period (2025), states may choose to begin with only their fee-for-service members and bring their managed care lives into the agreement as late as January 1, 2026.

Jan 1, 2026
CMS is currently engaging interested stakeholders to gather additional insights.

**Where Are We Now?**

CMS is engaged in the following stages:

- **Executive Order**: CMS developed the CGT Access Model in response to President Biden’s Executive Order 14087, Lowering Prescription Drug Cost for Americans.

- **Model Design**: CMS incorporated insights from conversations with stakeholders (states, manufacturers, beneficiary groups, clinicians).

- **Model Announcement**: Information about the CGT Access Model is available via the model website, the Model Overview Webinar, and this State Webinar.

- **Manufacturer Negotiation**: CMS will negotiate Key Terms of an outcomes-based agreement (OBA) on behalf of states.

- **Engagement with Interested Parties**: CMS seeks additional input from states, clinicians, and patient groups on the model. States can meet with CMS and submit non-binding letters of intent.

- **State Applications**: Key Terms of the OBA will be disclosed to states. States will decide whether to participate and may seek optional funding for model implementation and achievement of milestones.

- **Model Launch**: CMS will support states in administering OBAs through collection of clinical and claims data, payment reconciliation, and evaluation.
Why States Should Join
Challenges with Gene Therapy

The CGT Access Model aims to help states in the following ways:

- **High cost of expensive gene therapies**
- **Clinical uncertainty for newly approved gene therapies**
- **Burden of negotiating and implementing OBAs for gene therapies**
- **Population with high health care utilization that has been historically underserved**

**CMS will be positioned to negotiate greater discounts through pooled, multi-state bargaining**

**OBAs provide the possibility of additional rebates in cases where treatment with gene therapies does not produce expected results**

**CMS will support states in implementing, monitoring, reconciling, and evaluating the financial and clinical outcomes outlined in OBAs**

**Greater access promotes health equity and may drive long-term reductions in health expenditures**
CMS Support for States

States can negotiate supplemental rebates on their own, but through the CGT Access Model, CMS can offer:

1. OBA negotiation
   - Burden of negotiating with manufacturers
   - Greater leverage through pooled, multi-state bargaining, a standardized access policy across states, and manufacturer payment for certain fertility preservation services

2. Favorable pricing and OBA structures
   - Greater negotiation leverage may lead to discounted pricing
   - Broader OBAs that incorporate multiple types of rebates
   - Ability to incorporate multiple types of outcomes through claims data and partnerships with patient registries

3. Support in OBA implementation
   - Technical assistance for model implementation
   - Monitoring, reconciling, and evaluating the financial and clinical outcomes outlined in OBAs

4. Optional funding
   - Support implementation of model requirements
   - Support activities that promote equitable access to care and multi-disciplinary, comprehensive care
Potential Outcomes-Based Agreement (OBA) Structure
Potential Negotiated OBA Key Terms

CMS will negotiate Key Terms of OBAs with manufacturers on behalf of states.

Key Terms may include...

- Rebate structure
- Standardized access policy
- Manufacturer payment for fertility preservation
- CMS support in OBA implementation

Negotiation Timeline:

- May 2024: CMS will negotiate the Key Terms with manufacturers.
- November 2024: Key Terms will be disclosed to states.

States will be responsible for their share of the gene therapy cost, but at a discounted price tied to specific outcomes, as negotiated by CMS.

CMS intends to represent states’ interests in negotiations. States may express their priorities to CMS through a non-binding letter of intent (LOI) and by meeting with the model team.
Potential Rebate Structure

Pricing may reflect both the statutory rebate and the CMS-negotiated rebates, including volume-based and outcome-based rebates.

**Status Quo**
- MDRP Statutory Rebate

All statutory rebates continue to apply in the model (no change).

**Model Supplemental Rebate**

- Guaranteed Rebate
- Volume Rebates
- Patient Outcome Rebates
- Population Outcome Rebates

CMS negotiates additional rebates that would apply via a Supplemental Rebate Agreement.

**Final Rebate**

- MDRP Statutory Rebate
- Guaranteed Rebate
- Volume Rebates
- Patient Outcome Rebates
- Population Outcome Rebates

Under the model, states can benefit from multiple, layered rebates.
Potential Rebate Structure

Rebates may be paid over time, based on the rebate type.
Potential Rebate Timeline

Multiple cohorts may be tracked over the model lifetime.

- Assess Population-Level Threshold & Process Potential Rebate
- Assess Patient-Level Threshold & Process Potential Rebate
- Volume-Based Rebate
- Statutory Rebate
- Guaranteed Rebate

<table>
<thead>
<tr>
<th>Year</th>
<th>2025</th>
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- = Statutory Rebate
- = Guaranteed Rebate
- = Volume-Based Rebate
- = Assess Patient-Level Threshold & Process Potential Rebate
- = Assess Population-Level Threshold & Process Potential Rebate
Ensuring Equitable Access to Sickle Cell Disease (SCD) Gene Therapy Care
Care Journey
The recommended care journey for SCD gene therapy is long, rigorous, and complex.

1. Identification for Gene Therapy (Duration varies)
2. Disease Management by Specialist (Duration varies)
3. Evaluation for Gene Therapy and Education (~30 days)
4. Transition from Disease Management to Preparation for Gene Therapy (60-90 days)
5. Apheresis (3-9 days, inpatient)
6. Stem Cell Modification (40-180 days)
7. Optional Fertility Preservation (Duration varies)
8. Chemotherapy (7-9 days, inpatient)
9. Infusion and Inpatient Stay (30–45 days, inpatient)
10. Follow-Up Care (15 years, outpatient)
The CGT Access Model will provide optional state funding through a Cooperative Agreement. Funding will be available to help states implement the Model, as well as to support states that engage in activities that would increase equitable access to gene therapy and promote multi-disciplinary, comprehensive care for beneficiaries with SCD receiving gene therapy.

<table>
<thead>
<tr>
<th>Topic</th>
<th>Challenges</th>
<th>State Options to Address</th>
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<tbody>
<tr>
<td>Patient Knowledge</td>
<td>• Patient awareness of gene therapy</td>
<td>• Partnering with CBOs and non-profits</td>
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<td></td>
<td>• Patient knowledge of &amp; access to non-emergency medical transportation (NEMT)</td>
<td>• Directly expanding or increasing reimbursement rates for these benefits -- including through the existing optional SCD benefit.</td>
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<tr>
<td>SCD Care</td>
<td>• Access to SCD specialist</td>
<td>More details will be released in a Notice of Funding Opportunity by Summer 2024.</td>
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<tr>
<td></td>
<td>• Access to out-of-state providers</td>
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<tr>
<td>Other Specialty Care</td>
<td>• Access to behavioral health providers</td>
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<tr>
<td></td>
<td>• Access to other specialty care services and providers</td>
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<tr>
<td>Social Needs</td>
<td>• Health-related social needs (HRSNs), including childcare</td>
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<tr>
<td>Care Coordination</td>
<td>• Care coordination / Patient navigation</td>
<td></td>
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<td></td>
<td>• Navigating changes in insurance coverage</td>
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CMS will require manufacturers to pay for a defined scope of fertility preservation services.

- Manufacturers pay for collection, cryopreservation, and storage of reproductive materials in clinical trials
- Meets conditions of CMS-sponsored model safe harbor 42 CFR 1001.952(ii)
- May yield learning to inform state Medicaid agencies’ future decision-making regarding coverage for fertility preservation services in connection with CGT

CMS intends to negotiate an access policy with manufacturers that ensures treatment centers offer appropriate, multi-disciplinary care.

- Behavioral Health Services (mental health, SUD treatment, pain management)
- Case Management
State Participation
### Key Requirements for States

States participating in the CGT Access Model must meet the following requirements:*

<table>
<thead>
<tr>
<th>Requirement</th>
<th>Action</th>
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<tr>
<td>Execute value-based purchasing supplemental rebate agreements (VBP SRAs)</td>
<td>Pursue state plan amendments (SPAs) where appropriate</td>
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<tr>
<td>with manufacturers that reflect the Key Terms negotiated by CMS</td>
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<td>Establish a standardized access policy for included gene therapies</td>
<td>Carve included gene therapies out of any inpatient payment bundle</td>
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<td>Require providers to follow requirements for data reporting and claims submissions</td>
<td>Ensure beneficiaries have access to care with in-state or out-of-state qualified gene therapy provider(s)</td>
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<tr>
<td>Ensure necessary transportation and related travel expenses to beneficiaries (NEMT)</td>
<td>Meet minimum T-MSIS data requirements</td>
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</table>

* As applicable, these requirements apply with respect to a state's beneficiaries enrolled in fee-for-service and Medicaid managed care. As discussed earlier, a state may choose to begin in 2025 with only their fee-for-service members and bring their managed care lives into the agreement as late as January 1, 2026.

*More details will be released in the State Request for Applications by Summer 2024.*
Model Timeline
How to Join

Please keep in mind the important items and dates below:

**Optional: Submit a non-binding LOI**

- Letter of Intent (LOI) template is available now on the model website and has been emailed to states.
- Responses are important to help CMS represent state priorities in negotiation with manufacturers.
- Was due April 1, 2024; extended to April 12, 2024.

**Required: Apply to join the model**

- State Request for Applications will be released by Summer 2024.
- States can apply **December 2024 to February 2025**; CMS will review applications on a rolling basis.
- States may begin participation between January 2025 and January 2026.

**Optional: Apply for model funding**

- Notice of Funding Opportunity (NOFO) will be released by Summer 2024.
- States can apply **December 2024 to February 2025**.
- CMS will issue Notice of Awards (and initial funding will be released) as early as June/July 2025.
# Model Launch Timeline

Please keep in mind the important items and dates below.

<table>
<thead>
<tr>
<th>Manufacturer Participation</th>
<th>2024</th>
<th>2025</th>
<th>2026 →</th>
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<tbody>
<tr>
<td>CMS releases Manufacturer RFA</td>
<td>Mar 2024</td>
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<tr>
<td>Manufacturers submit RFA applications</td>
<td>Due May 2024</td>
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<tr>
<td>CMS-Manufacturer negotiations</td>
<td>May – Nov 2024</td>
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<tr>
<td>Manufacturers sign Participation Agreements</td>
<td>Nov 2024</td>
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<thead>
<tr>
<th>State Participation</th>
<th>2024</th>
<th>2025</th>
<th>2026 →</th>
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<tbody>
<tr>
<td>States submit non-binding LOIs</td>
<td>Due Apr 2024</td>
<td></td>
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<tr>
<td>CMS releases State RFA</td>
<td>Summer 2024</td>
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<tr>
<td>Key Terms are disclosed to States</td>
<td>Dec 2024</td>
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<tr>
<td>States submit RFA applications; CMS reviews</td>
<td>Dec 2024 – Feb 2025, rolling</td>
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<tr>
<td>States sign State Agreements</td>
<td>Dec 2024 – Jun 2025</td>
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<tr>
<th>Optional State Funding</th>
<th>2024</th>
<th>2025</th>
<th>2026 →</th>
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<tbody>
<tr>
<td>CMS releases NOFO</td>
<td>Summer 2024</td>
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<tr>
<td>States submit NOFO applications</td>
<td>Due Feb 2025</td>
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<tr>
<td>CMS reviews applications</td>
<td>Mar – Jun 2025</td>
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<tr>
<td>CMS issues Notice of Awards; Cooperative Agreement funding begins</td>
<td>June/July 2025</td>
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<tr>
<th>Model Performance</th>
<th>2024</th>
<th>2025</th>
<th>2026 →</th>
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<tr>
<td>Performance Year 1 (may start at any time)</td>
<td>Jan 2025 – Dec 2025</td>
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<tr>
<td>Performance Year 2</td>
<td>Jan 2026 – Dec 2026</td>
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### LEGEND

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

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 and Resources
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](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.

**State Letter of Intent (LOI)**
Non-binding LOI was due April 1, 2024; extended to no later than 11:59pm EDT April 12, 2024

**Helpdesk**
If you have questions or would like to meet with the model team, please reach out to us via email at [CGTModel@cms.hhs.gov](mailto:CGTModel@cms.hhs.gov).
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 CGTModel@cms.hhs.gov with subject line CGT Access Model State Announcement Webinar
THANK YOU!