

Innovations and Advances in Sickle Cell Disease Gene Therapies

U.S. Department of Health and Human Services
Office of Minority Health

September 25, 2025, 2:00-3:30 PM EST

***This event is being recorded and will be available on the
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This convening is supported by the U.S. Department of Health and Human Services (HHS) Office of Minority Health (OMH).

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Run-of-Show and Moderator

- Welcome (*Moderator, CDR Matthew Johns*)
- Opening Remarks (*CAPT Mahyar Mofidi-OMH Director*)
- Cell and Gene Therapy (CGT) Access Model (*CMS/CMMI*)
- State Perspective (*South Carolina*)
- Impact of Gene Therapy on Quality of Life (*SCD Patient Advocate*)
- Q&A
- Closing Remarks (*CAPT Mahyar Mofidi-OMH Director*)

We want to hear from you!



Email us at MinorityHealthInfo@hhs.gov or scan our QR code and let us know what part of today's session you found most useful and if you have ideas for how we can improve future events.



CAPT Mahyar Mofidi

Director, HHS Office of Minority Health





Mr. Abraham Sutton

Director, Center for Medicaid and Medicare Innovation



Cell and Gene Therapy (CGT) Access Model Overview

Center for Medicare and Medicaid Innovation
September 2025

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:



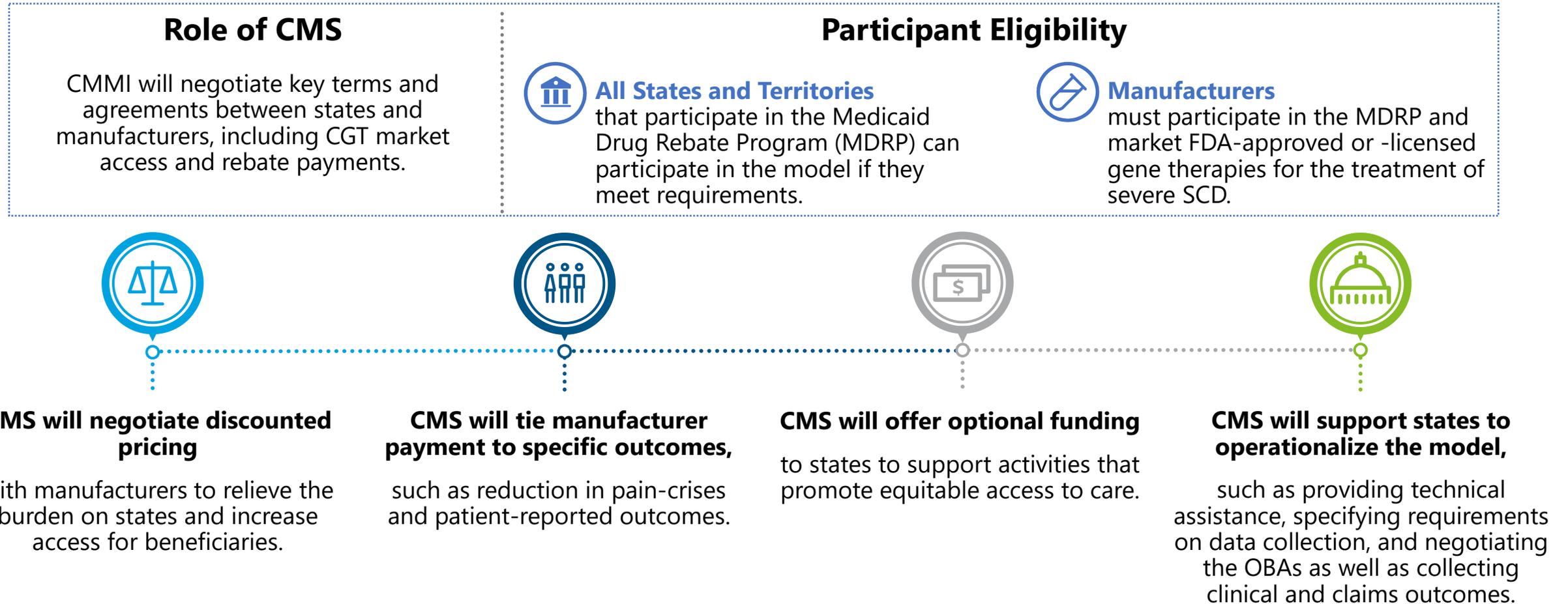
Negotiate with manufacturers on behalf of states for outcomes-based agreements which tie payment to specific outcomes.



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

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.



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

CGT Model | Manufacturers will deliver fertility preservation services at no out-of-pocket cost to eligible beneficiaries

Eligible beneficiary

- Documented diagnosis of SCD
- Has been prescribed gene therapy product, consistent with the label
- Has Medicaid or CHIP
- Enrolled in Medicaid fee-for-service or Medicaid managed care in a Model Participating State
- Has not started myeloablative conditioning

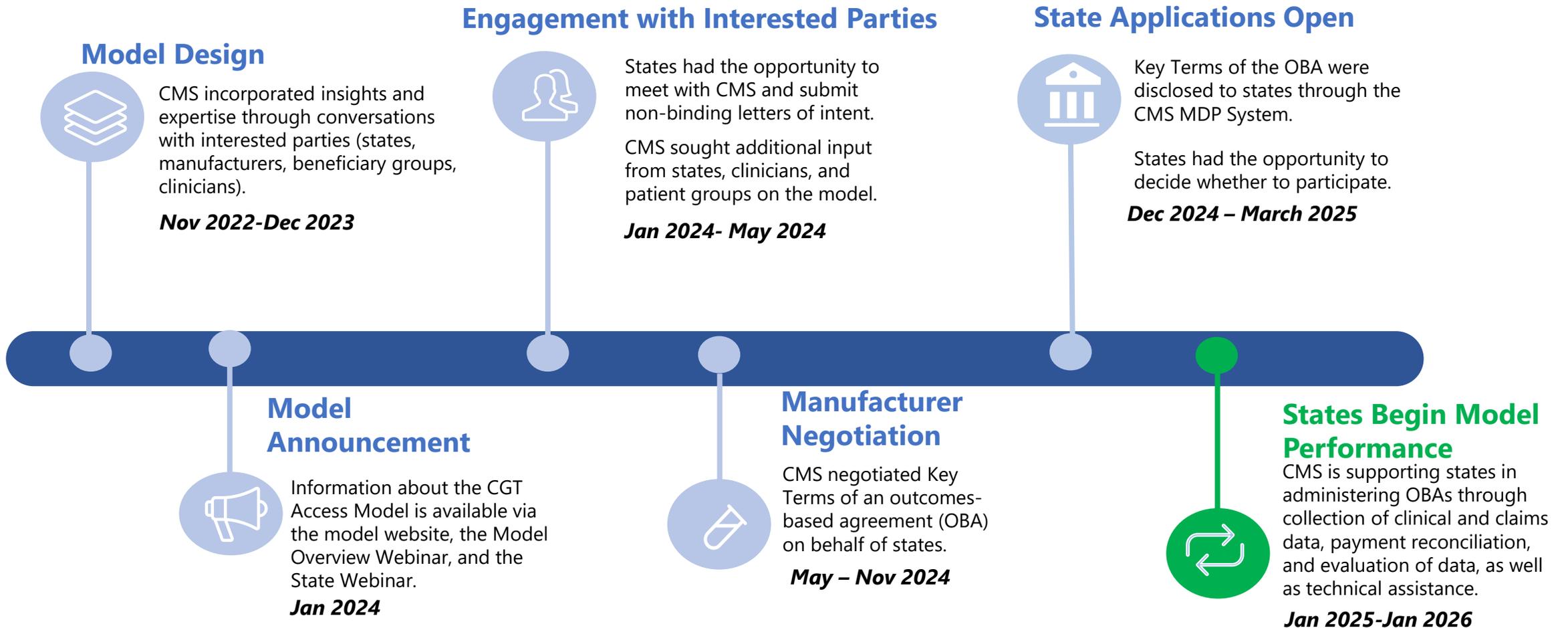
Services

- Harvesting, freezing, and storage of oocytes
- Collecting, freezing, and storing spermatozoa
- Extracting, freezing, and storing testicular tissue
- Associated consultations, counseling, testing, imaging, bloodwork, medications, procedures, and practitioner services

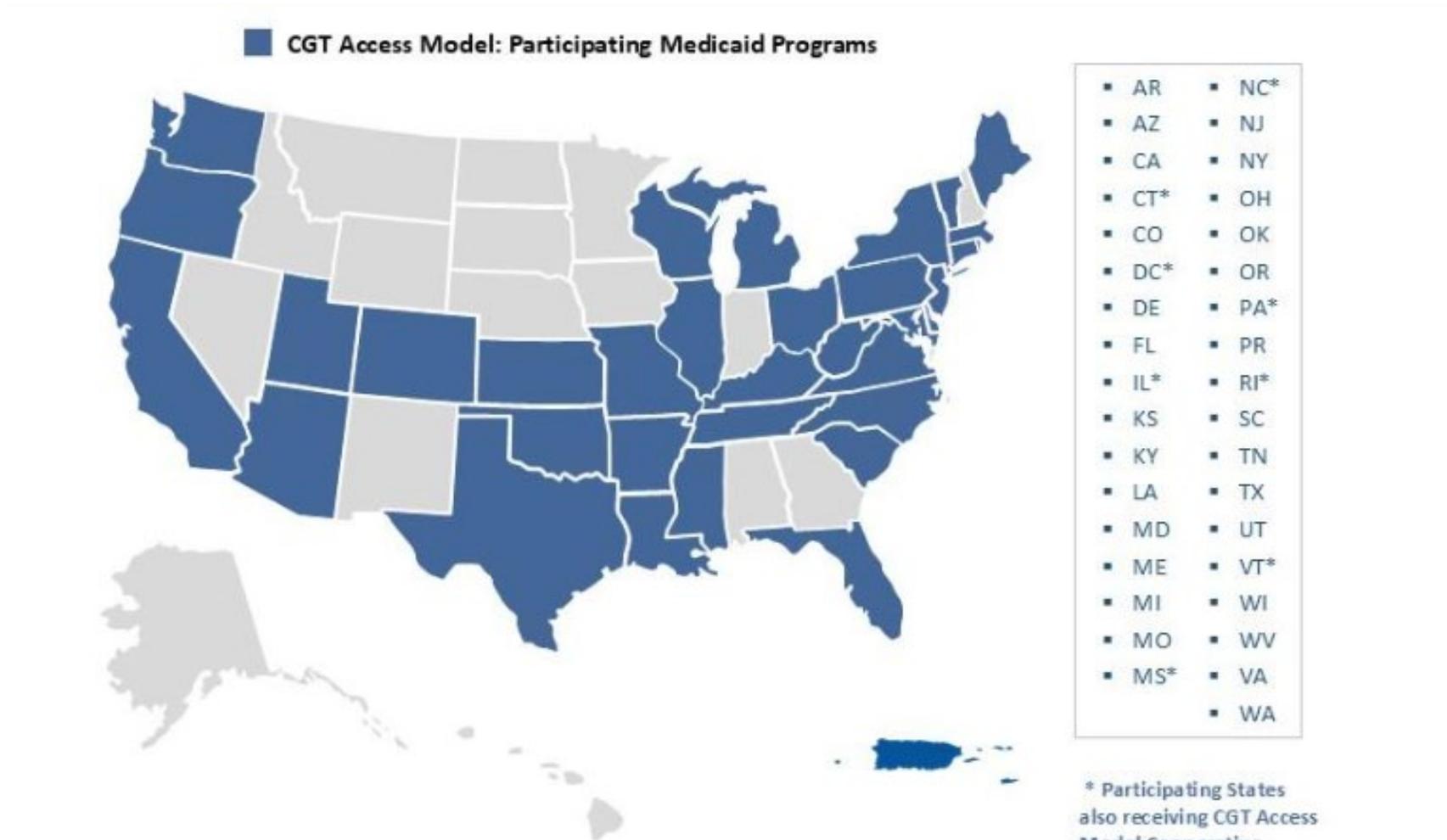
Other manufacturer requirements

- Both manufacturers will pay for storage of reproductive material for fifteen years
- Qualifying lodging, meals, and travel associated expenses will be covered by the manufacturers for beneficiaries who are traveling long distances to receive this care.

CGT Model | Model Timeline



CGT Model | 33 States Plus D.C. and Puerto Rico are Participating in the Model



Source: Centers for Medicare & Medicaid Services

* Participating States also receiving CGT Access Model Cooperative Agreement funding

CGT Model | Recent and upcoming milestones

March-Jun 2025

- States applications processed; States Agreements signed
 - Independent review panel reviewed optional funding applications and determined allocation of state awards
 - Ongoing technical assistance (TA) and cooperative agreement support
-

August 2025

- All Cooperative Agreements awarded
-

January 2026

- All participating states will have gone live; TA and cooperative agreement support to states continue
-

Ongoing

- Monitoring of CGT Pipeline to determine future conditions for the Model

Share your ideas of future conditions or other directions for the CGT Access Model with the CGT Access Model Team at CGTModel@cms.hhs.gov using the subject line “Future Model Considerations.”

Model Resources

The CGT Access Model team has a host of resources to support interested states. To see the latest resources, visit the model's website at

<https://www.cms.gov/priorities/innovation/innovation-models/cgt>.

Cell and Gene Therapy (CGT) Access Model State Request for Applications (RFA) Factsheet

The CGT Access Model will test whether a CMS-led approach to developing and administering outcomes-based agreements (OBAs) for cell and gene therapies (CGTs) improves Medicaid beneficiaries' health outcomes, broadens access to innovative treatment, and reduces health care expenditures.

Goals for States

- 1 Reduce the burden of negotiating and implementing OBAs for gene therapies.
- 2 Facilitate the adoption of OBAs.
- 3 Facilitate savings to states due to greater productivity rebates, and long-term reductions in health care expenditures.

STATE PARTICIPATION REQUIREMENTS

States will be required to implement the following requirements during the model:

Operational Requirements

State participants must implement requirements to support the model, including:

- Legal Authority: Have the authority to implement the Model, including CMS approval of a State Plan Amendment (SPA).
- Standardized Access Policy: Establish a standardized Model Drug access policy consistent with CMS-manufacturer negotiated Key Terms.
- Model Drug Consent: Care Model Drug out of an inpatient payment bundle, if necessary, and make payment for the Model Drug until that relates under the RFA apply.
- Provider Reimbursement Requirements: Agree to follow Model-specific requirements related to registry participation and claims submission.
- Permissible State Expenses: Avoid covering any costs paid for by a manufacturer (e.g., for certain facility presentation services) as state expenses.
- Managed Care Alignment: Ensure that applicable Medicaid managed care plan policies align with Model requirements.

Agreements with Manufacturers

State participants must sign agreements with participating manufacturers, including:

- Value Based Purchasing (VBP) Supplemental Incentive Agreement (SIA): Exclude a VBP SIA with a participating manufacturer that reflects the Key Terms.
- Optional VBP Agreement for Separate CHIP Beneficiaries: If applicable, exclude a VBP agreement for separate CHIP beneficiaries with a participating manufacturer that reflects the separate CHIP Key Terms.

Access to Care

To help ensure beneficiaries' access to care under the Model, states are required to:

- Ensure that applicable Medicaid managed care plan policies align with Model requirements.
- Necessary transportation and related travel expenses to Model beneficiaries and their caregivers, as applicable.
- States will submit Medicaid claims data through the Transformed Medicaid Statistical Information System (TMSIS) and will be expected to meet TMSIS Outcomes Based Agreement.
- Each state participant must submit documentation and performance reports to CMS on Model implementation and performance.

Cell and Gene Therapy (CGT) Access Model Notice of Funding Opportunity (NOFO) Factsheet

The Notice of Funding Opportunity (NOFO) announces the opportunity to apply for Cooperative Agreement funding to support state participation in the CGT Access Model.

NOFO DETAILS

FUNDING AMOUNT
up to **\$9.55M** for each state, over the duration of the model.

Cooperative Agreement funding is intended to support state model implementation activities and to support states that take steps to improve equitable access to gene therapy and promote multi-disciplinary, comprehensive care in conjunction with the model test.

Cooperative Agreements offer grants to states that involve substantial interaction between the Federal awarding agency and the non-Federal entity carrying out activities under the award. However, many of the same rules, regulations, and policies that apply to grants also apply to cooperative agreements.

FUNDING STRUCTURE

Two types of funding will be available under the Cooperative Agreement:

- Implementation Funding** for model activities that involve staff/contractor time and infrastructure costs.
- Milestone Funding** for successful completion of research project.

NOFO APPLICATION PROCESS AND TIMELINE

CMS requires electronic submission of applications on [grants.gov](https://www.grants.gov) by February 28, 2025, 11:59 pm ET. The anticipated award date will be July 1, 2025.

To be considered, an applicant must apply to both the CGT Access Model State Request for Applications (RFA) and the NOFO. Both applications are due by February 28, 2025.

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.

Model Goals

- Improve Beneficiary Access
- Improve Health Outcomes
- Reduce Health Care Utilization and Expenditures

CGT ACCESS MODEL PARTICIPANTS

STATES

All states and territories that participate in the Medicaid Drug Rebate program can participate in the model if they meet requirements.

MANUFACTURERS

Manufacturers will be able to apply to the model by agreeing to a Manufacturers who participate in the MDDB and market U.S. Food & Administration (FDA)-approved or -cleared gene therapies for the CGT Access Model. Negotiations between manufacturers are scheduled to take place between May - November.

PROVIDERS

Providers will not be participants in the model.

MODEL POPULATION

The model population is beneficiaries for whom the primary payer and Medicaid expansion Children's Health Plan (CHIP) beneficiaries ("Title XIX beneficiaries") and Medicaid managed care.

Manufacturers and states will have the option to include two beneficiary groups ("Title XIX beneficiaries") alongside Title XIX de minimis CHIP beneficiaries in the model.

Beneficiaries must receive an FDA-approved CGT for SCD or SCN, and be a resident of a participating state or a covered county (CC) or CHIP that participates in the model.

CGT requires electronic submission of applications on [grants.gov](https://www.grants.gov) by February 28, 2025, 11:59 pm ET. The anticipated award date will be July 1, 2025.

State RFA Resources

The [State RFA](#) is on the model webpage. Read through the [CGT State RFA Factsheet](#) and the [CGT State RFA Frequently Asked Questions](#) to learn more about applying to participate in the model.

NOFO Resources

The [NOFO](#) is on Grants.gov. Read through the [CGT NOFO Factsheet](#) and the [CGT NOFO Frequently Asked Questions](#) on the model website to learn more about applying for model funding.

Other Model Resources

Read through the [CGT Model Overview Factsheet](#), the [CGT Model Infographic](#), and the [Patient Care Journey Visual](#) to learn more about the CGT Access Model and the patient care journey for SCD gene therapy. See the latest [Press Release](#) announcing manufacturer participation in the model.

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.

THANK YOU!



Director Eunice Medina Dr. Kevin Wessinger

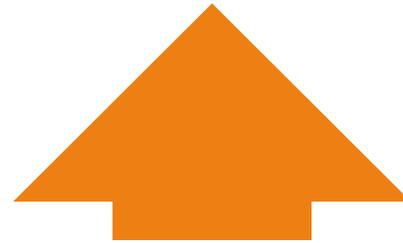
South Carolina Department of Health and Human Services



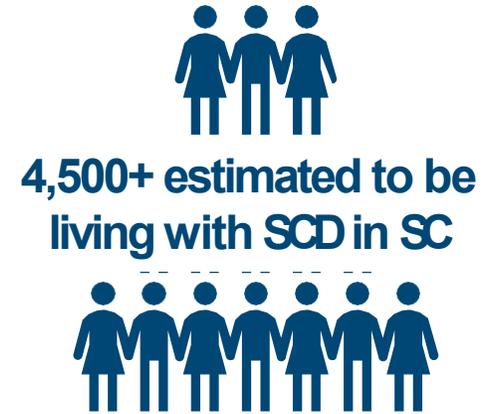
South Carolina's Experience with Gene Therapy Access for Sickle Cell Disease (SCD)

Expanding Access, Lessons Learned, and Policy Perspective

Why this matters in South Carolina?



High cost creates access and sustainability challenges



- Approx. 400 SC Medicaid members may qualify for gene therapy
- Medical University of South Carolina = only authorized treatment center (Capacity 6-10/year)
- Disproportionate impact on African American communities

Steps taken to expand access



Early engagement with MUSC and manufacturers



Required State Plan Amendment submitted in 2024



Negotiated State-Specific Supplemental Rebate Agreements (SRAs)



Utilized High Cost/No Experience (HCNE) drug list for MCO coverage



Carved out hospital payments from DRG to ensure adequate reimbursement



SC Experience: Impact

- First treatments underway at MUSC
- Two (2) SC Medicaid members have completed infusion
- Three (3) additional members in treatment journey
- The Center for Medicare and Medicaid Innovation's (CMMI) Cellular and Gene Therapy (CGT) Model participation effective 6/1/2025

Lessons Learned



Early preparation and proactive negotiations critical



Strong collaboration with MUSC and Manufacturers



Peer learning from Missouri's early adoption



Close partnership with CMMI accelerated readiness

Challenges Encountered

- Capacity limitations at treatment centers
- Optimistic implementation timelines
- Managing high upfront fiscal impact
- Ensuring comprehensive coverage (transportation, fertility preservation, etc.)

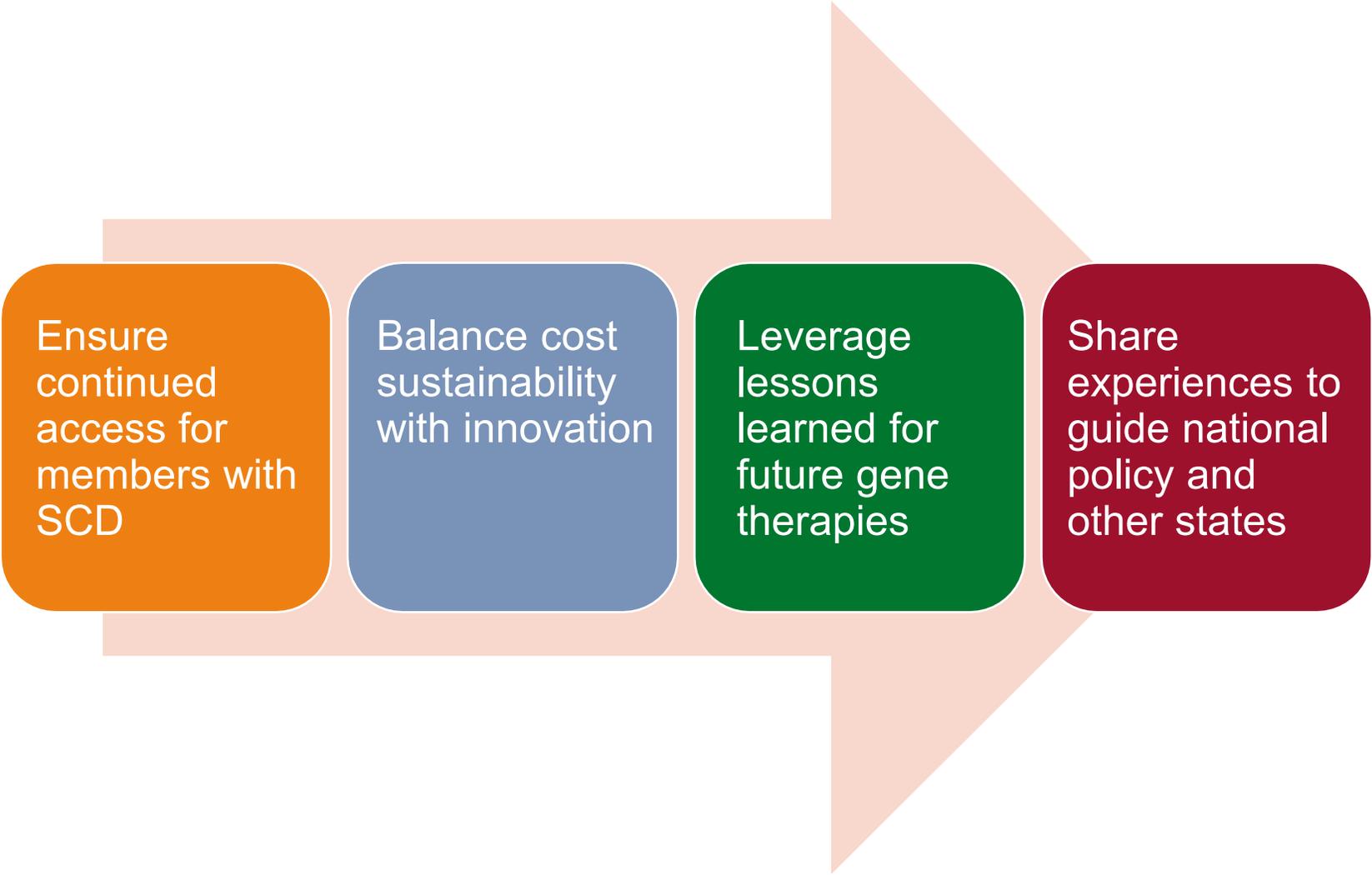
State Perspective: CMMI Access Model

- Participation optional, SC joined early (second state approved)
- Value-Based Purchasing with supplemental rebate options
- Outcome-Based Agreements (refund if therapy fails)
- Standardized payments ensure equity in access

State Perspective: Policy Outlook

- Pending federal legislation may shape sustainability
- Need for predictable financing mechanisms for high-cost therapies
- Strong alignment with Medicaid's mission to expand equitable access
- South Carolina positioned as a leader and early adopter

South Carolina's Path Forward







Mr. Jimi Olaghere

Sickle Cell Disease Patient Advocate



Q&A



CAPT Mahyar Mofidi

Director, HHS Office of Minority Health



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