

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

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INNOVATIONS AND ADVANCES IN SICKLE CELL DISEASE GENE THERAPIES | SEPTEMBER 25, 2025

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

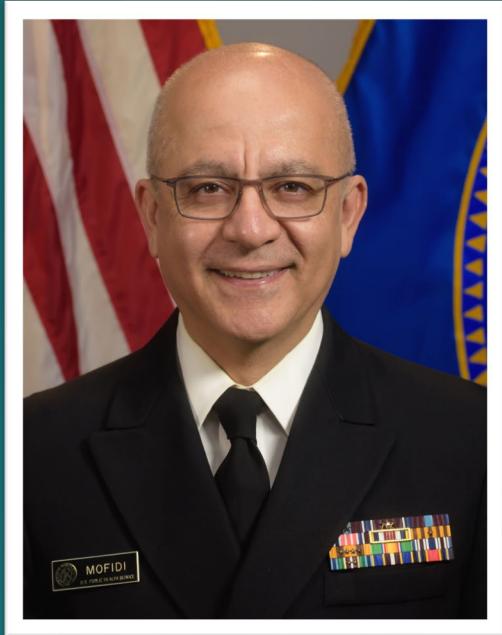
- Welcome (*Moderator, CDR Matthew Johns*)
- Opening Remarks (*CAPT Mahyar Mofidi-OMH Director*)
- Cell and Gene Therapy (CGT) Access Model (*CMS/CMMI*)
- State Perspective (*South Carolina*)
- Impact of Gene Therapy on Quality of Life (*SCD Patient Advocate*)
- Q&A
- Closing Remarks (*CAPT Mahyar Mofidi-OMH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



We want to hear from you!



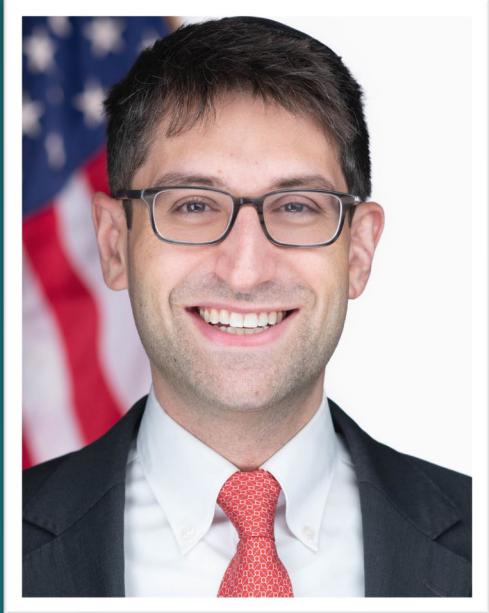
Email us at
MinorityHealthInfo@hhs.gov or
scan our QR code and let us know
what part of today's session you
found most useful and if you have
ideas for how we can improve
future events.



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.

Role of CMS

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



CMS will negotiate discounted pricing

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

Participant Eligibility



All States and Territories

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



CMS will tie manufacturer payment to specific outcomes,

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



Manufacturers

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



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

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



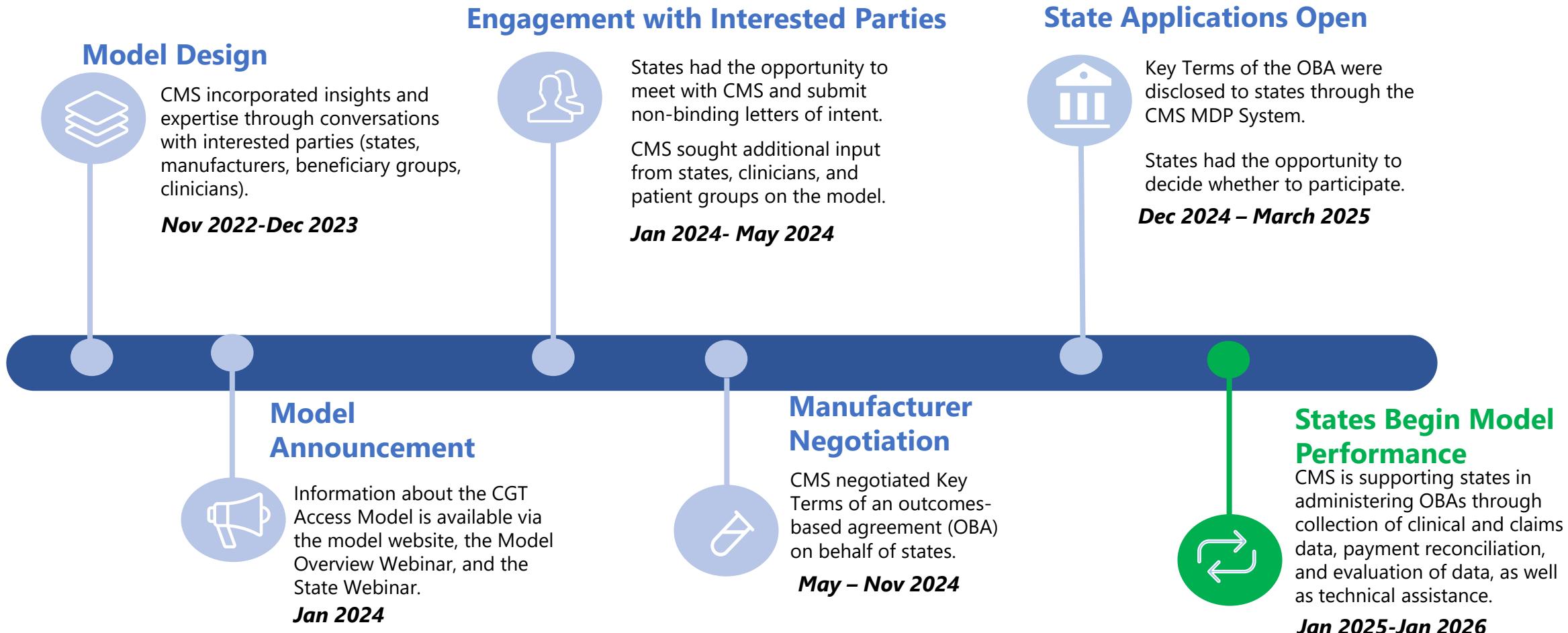
- Harvesting, freezing, and storage of oocytes
- Collecting, freezing, and storing spermatozoa
- Extracting, freezing, and storing testicular tissue
- Associated consultations, counseling, testing, imaging, bloodwork, medications, procedures, and practitioner services

Other manufacturer requirements

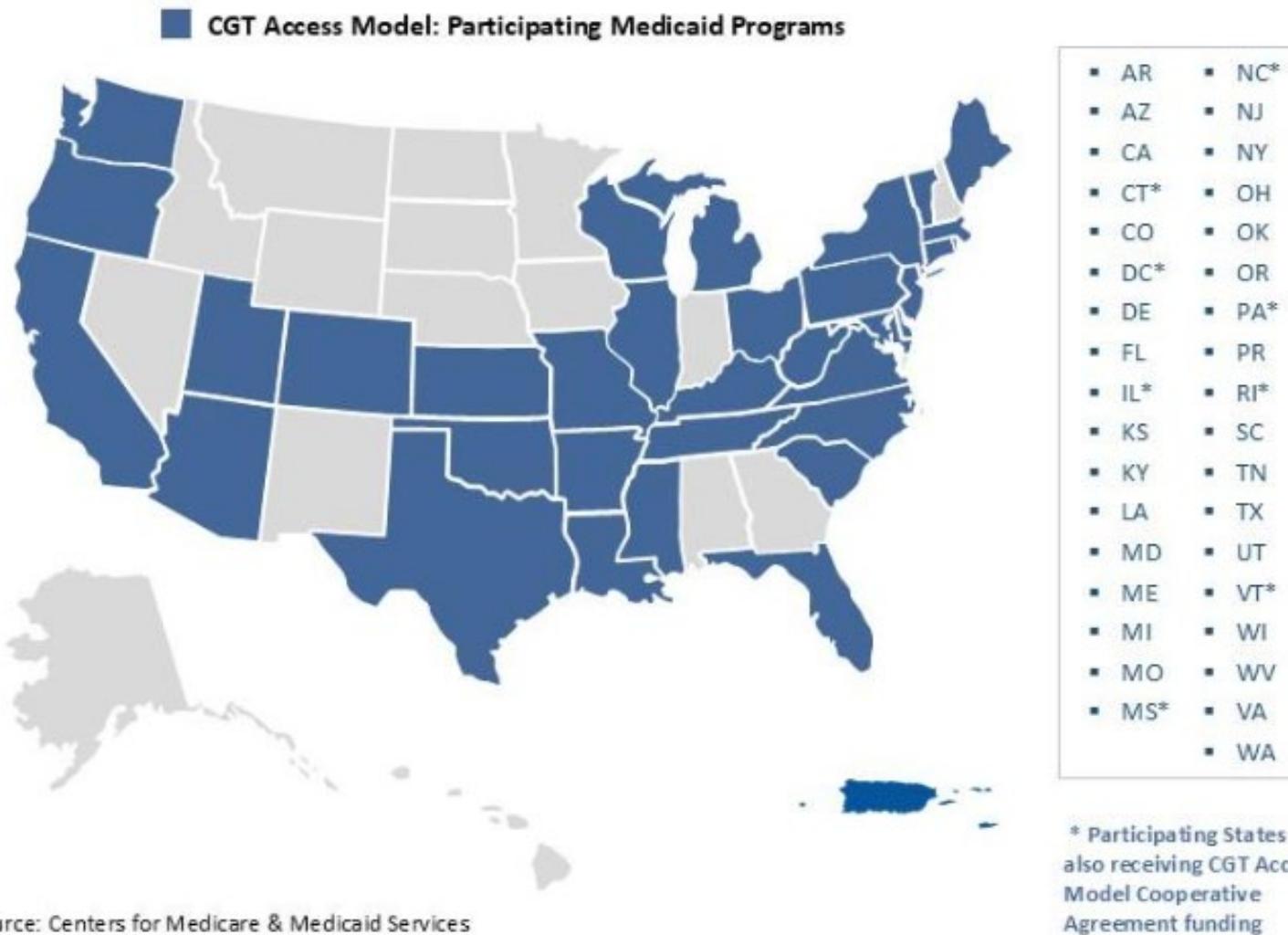


- Both manufacturers will pay for storage of reproductive material for fifteen years
- Qualifying lodging, meals, and travel associated expenses will be covered by the manufacturers for beneficiaries who are traveling long distances to receive this care.

CGT Model | Model Timeline



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



CGT Model | Recent and upcoming milestones

March-Jun 2025	<ul style="list-style-type: none">• 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
<hr/>	
August 2025	<ul style="list-style-type: none">• All Cooperative Agreements awarded
<hr/>	
January 2026	<ul style="list-style-type: none">• All participating states will have gone live; TA and cooperative agreement support to states continue
<hr/>	
Ongoing	<ul style="list-style-type: none">• 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>.

 <p>MASSACHUSETTS THE BAY STATE</p>  <p>CENTER FOR MEDICARE & MEDICAID SERVICES CENTER FOR MEDICARE & MEDICAID INNOVATION</p>	<p>Cell and Gene Therapy (CGT) Access Model State Request for Applications (RFAs) Fact Sheet</p> <p>CGT Access Model</p> <p>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.</p>	<p>Goals for States</p> <p>1  Reduce the burden of negotiating and implementing agreements for gene therapies</p> <p>2  Facilitate the adoption of OBAs</p> <p>3  Facilitate savings to states due to greater predictability, rebates, and long-term reductions in health care expenditures</p>
<p>U.S. Department of Health & Human Services</p> <p>Centers for Medicare & Medicaid Services</p> <p>Center for Medicare & Medicaid Innovation</p> <p>Seamless Care Models Group</p> <p>7500 Security Boulevard</p> <p>Baltimore, MD 21244</p>	<p>The CGT Access Model State RFAs will open to all states, the District of Columbia, and all 50 territories participating in Medicaid. CMS will accept applications from states that did not submit a letter of intent to request a waiver.</p>	<p>STATE PARTICIPATION REQUIREMENTS</p> <p>States will be required to implement the following requirements during the model.</p> <p>Operational Requirements</p> <p>State participants must implement requirements to support the model, including:</p> <ul style="list-style-type: none"> Legal Authority: Establish authority to implement the Model, including CMS approval of a State Plan Amendment (SPA). Establish CGT Access Model: Establish a CGT Access Model drug access policy consistent with CMS-identified model key terms. Model Drug Coverage: Model Drug Drugs of an opioid payment bundle, if necessary, and establish a payment bundle for other drugs that reduce under the Medicaid applicable payment rate. Provider Reimbursement Requirements: Establish requirements for providers related to registry participation and claims submission. Permissible State Expenses: Permissible expenses for a manufacturer (e.g., for certain entity presentation services), as state expenses. Ensure the applicable Medicaid managed care plan policies align with Model requirements
<p>Cell & Gene Therapy Access Model</p> <p>Request for Applications for States</p>	<p>To help ensure beneficiary access to care, under the Model, states are required to:</p> <ul style="list-style-type: none"> Beneficiaries have access to at least one qualified gene therapy provider: A gene therapy provider (e.g., hospital or in another state) or its designated caregivers, as applicable. Necessary transportation and related travel expenses: To and from the gene therapy provider (e.g., hospital or in another state) or its designated caregivers, as applicable. 	<p>Access to Care</p> <p>To help ensure beneficiary access to care, under the Model, states are required to:</p> <ul style="list-style-type: none"> Beneficiaries have access to at least one qualified gene therapy provider: A gene therapy provider (e.g., hospital or in another state) or its designated caregivers, as applicable. Necessary transportation and related travel expenses: To and from the gene therapy provider (e.g., hospital or in another state) or its designated caregivers, as applicable.
<p>Cell & Gene Therapy Access Model</p> <p>Request for Applications for States</p>	<p>Agreements with Manufacturers</p> <p>State participants sign agreements with manufacturers to:</p> <ul style="list-style-type: none"> Value-Based Purchasing (VBP) Supplemental Rebate Agreement: Establish a VBP with a participating manufacturer that reflects the key terms. Optional VBP Agreements for Separate CHIP Beneficiaries: Establish a VBP with a participating manufacturer that reflects the key terms. Optional VBP Agreements for Separate CHIP Beneficiaries with a Participating Manufacturer: Establish a VBP with a participating manufacturer that reflects the key terms. 	<p>Data & Reporting</p> <p>State participants must meet minimum data requirements:</p> <ul style="list-style-type: none"> State: Will submit Medicaid claims data through the CMS Data Extraction System (DES) and Statistical Information System (SIS) to CMS. Each state participant: Must submit data on Medicaid implementation and performance.

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.

An official website of the United States government [View our繁體中文](#)

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MENU

VIEW GRANT OPPORTUNITY

CMS-2P2-25-001

Cell and Gene Therapy (CGT) Access Model

Department of Health and Human Services

Centers for Medicare & Medicaid Services

SYNOPSIS

VERSION HISTORY

RELATED DOCUMENTS

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

CGT ACCESS MODEL: NOFO PURPOSE

The CGT Access Model uses a new, more efficient approach to developing and administering outcomes-based agreements (OBAs) for cell and gene therapies (CGTs) to improve Medicaid beneficiaries' health outcomes, broadens access to innovative treatments, and helps health care expenditures.

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

The CGT Access Model NOFO is open to all states, the District of Columbia, and all U.S. territories participating in the Medical Drug Rebate Program (MDRP).

NOFO DETAILS

FUNDING AMOUNT up to \$9.5MM

for each state, over the duration of the model

Total funding available to all states through grants and cooperative agreements.

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

FUNDING STRUCTURE

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

Implementation Funding

for model activities that involve staff/contractor implementation and infrastructure costs.

Milestone Funding

for successful completion of research projects

NOFO APPLICATION PROCESS AND TIMELINE

CMS requires electronic submission of applications on [grants.gov](#) by February 28, 2025, 11:59 pm EST. 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 Participation \(SFRP\)](#) and the NOFO. Both applications are due by February 28, 2025.

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!

INNOVATIONS AND ADVANCES IN SICKLE CELL DISEASE GENE THERAPIES | SEPTEMBER 25, 2025



**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



4,500+ estimated to be living with SCD in SC



- 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

Ensure continued access for members with SCD

Balance cost sustainability with innovation

Leverage lessons learned for future gene therapies

Share experiences to guide national policy and other states





Mr. Jimi Olaghere

Sickle Cell Disease Patient Advocate



Q&A



CAPT Mahyar Mofidi

Director, HHS Office of Minority Health



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