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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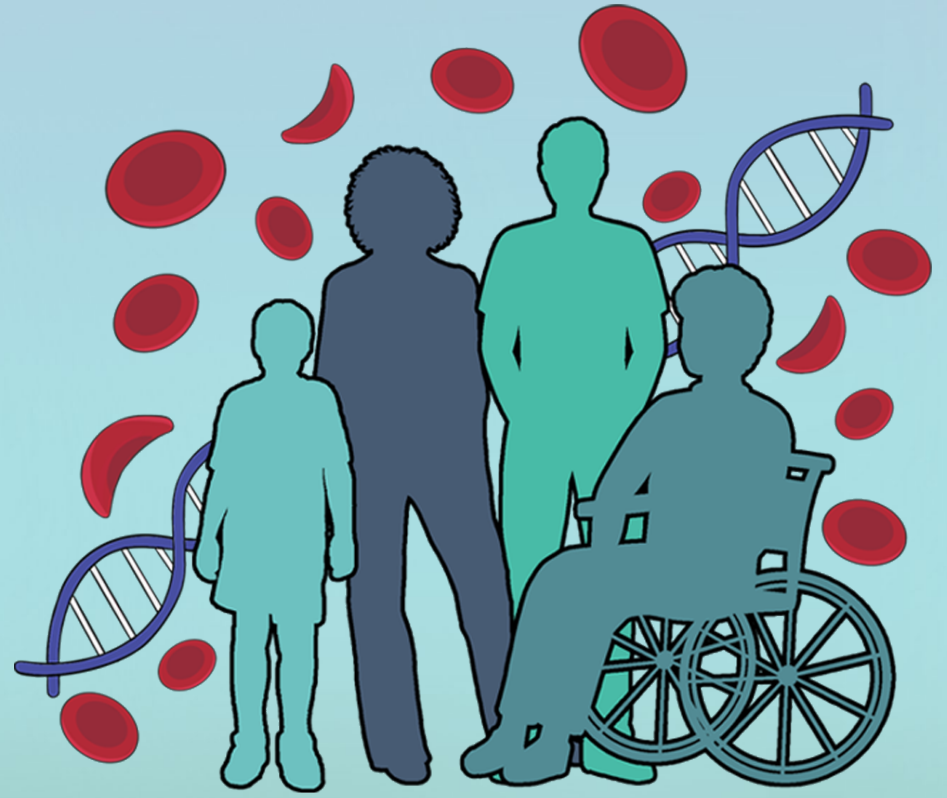

This convening is supported by the U.S. Department of Health and Human Services (HHS) Office of Minority Health (OMH).

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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

U.S. Department of Health and Human Services
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

CMS
CENTERS FOR MEDICARE & MEDICAID SERVICES
CENTER FOR MEDICARE AND MEDICAID INNOVATION

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.

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

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**

Model Design

CMS incorporated insights and expertise through conversations with interested parties (states, manufacturers, beneficiary groups, clinicians).

Nov 2022-Dec 2023

Model Announcement

Information about the CGT Access Model is available via the model website, the Model Overview Webinar, and the State Webinar.

Jan 2024

Engagement with Interested Parties

States had the opportunity to meet with CMS and submit non-binding letters of intent.

CMS sought additional input from states, clinicians, and patient groups on the model.

Jan 2024- May 2024

Manufacturer Negotiation

CMS negotiated Key Terms of an outcomes-based agreement (OBA) on behalf of states.

May – Nov 2024

State Applications Open

Key Terms of the OBA were disclosed to states through the CMS MDP System.

States had the opportunity to decide whether to participate.

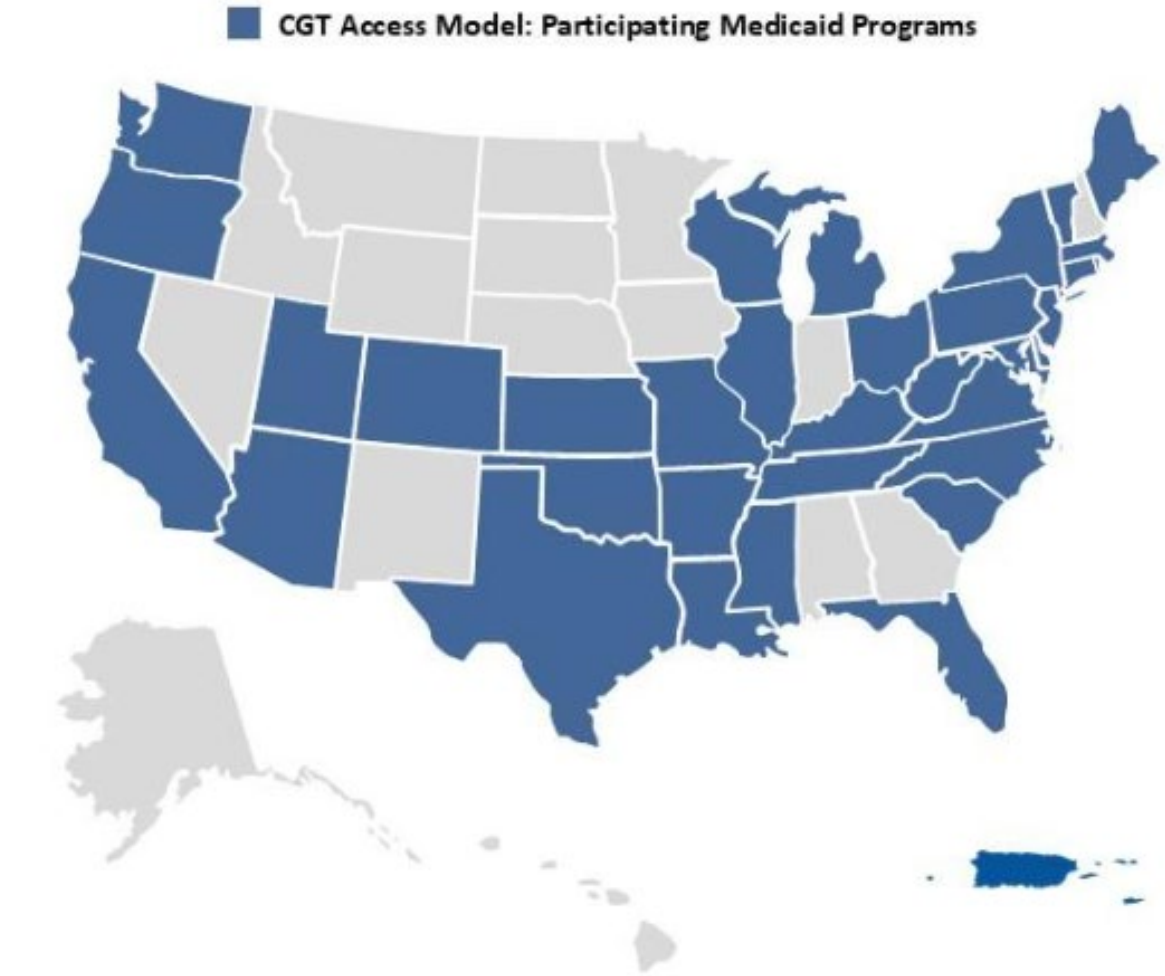
Dec 2024 – March 2025

States Begin Model Performance

CMS is supporting states in administering OBAs through collection of clinical and claims data, payment reconciliation, and evaluation of data, as well as technical assistance.

Jan 2025-Jan 2026

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



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* Participating States also receiving CGT Access Model Cooperative Agreement funding

Source: Centers for Medicare & Medicaid Services

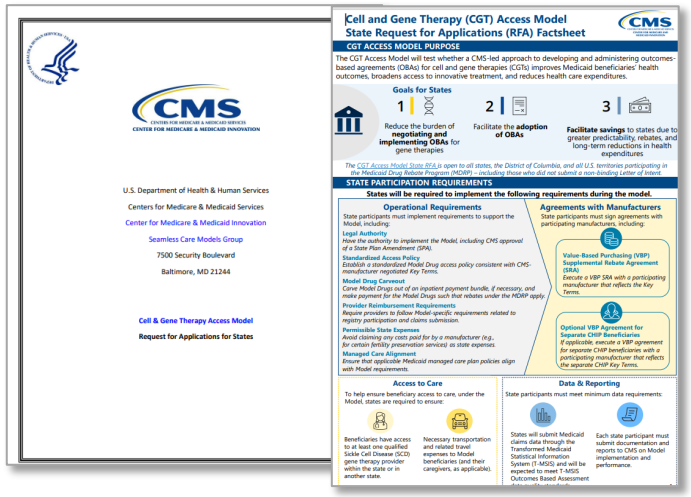
CGT Model | Recent and upcoming milestones

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."

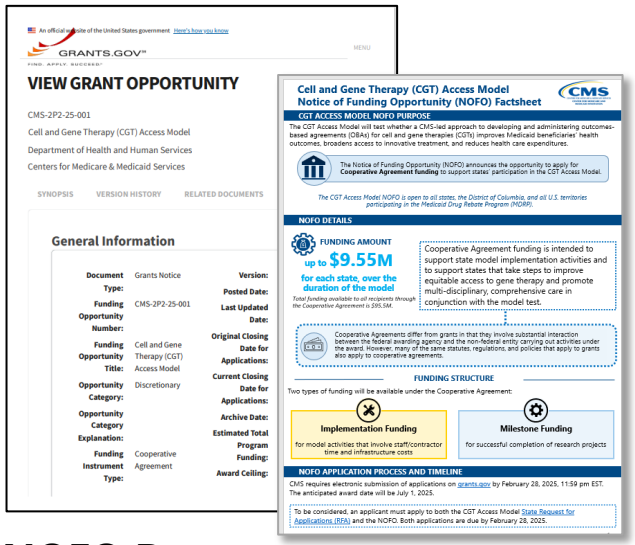
Model Resources

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.



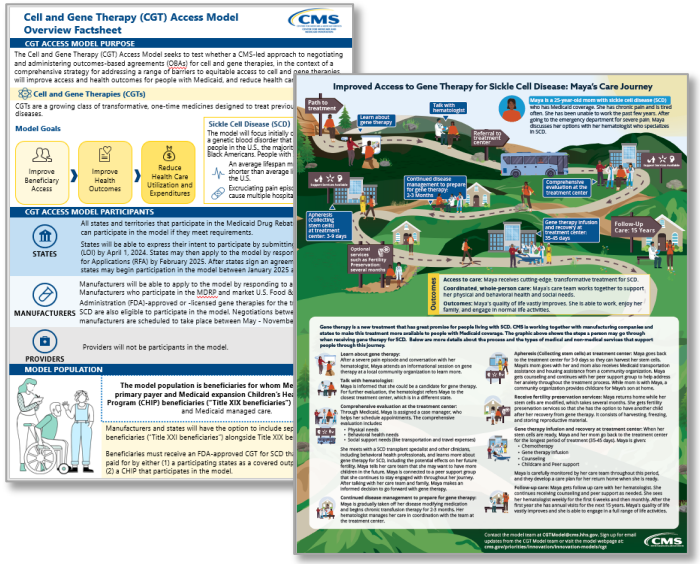
State RFA Resources

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!

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Director Eunice Medina
Dr. Kevin Wessinger

South Carolina Department of Health and Human Services

SOUTH CAROLINA DEPARTMENT OF HEALTH AND HUMAN SERVICES
Healthy Connections MEDICAID

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.)

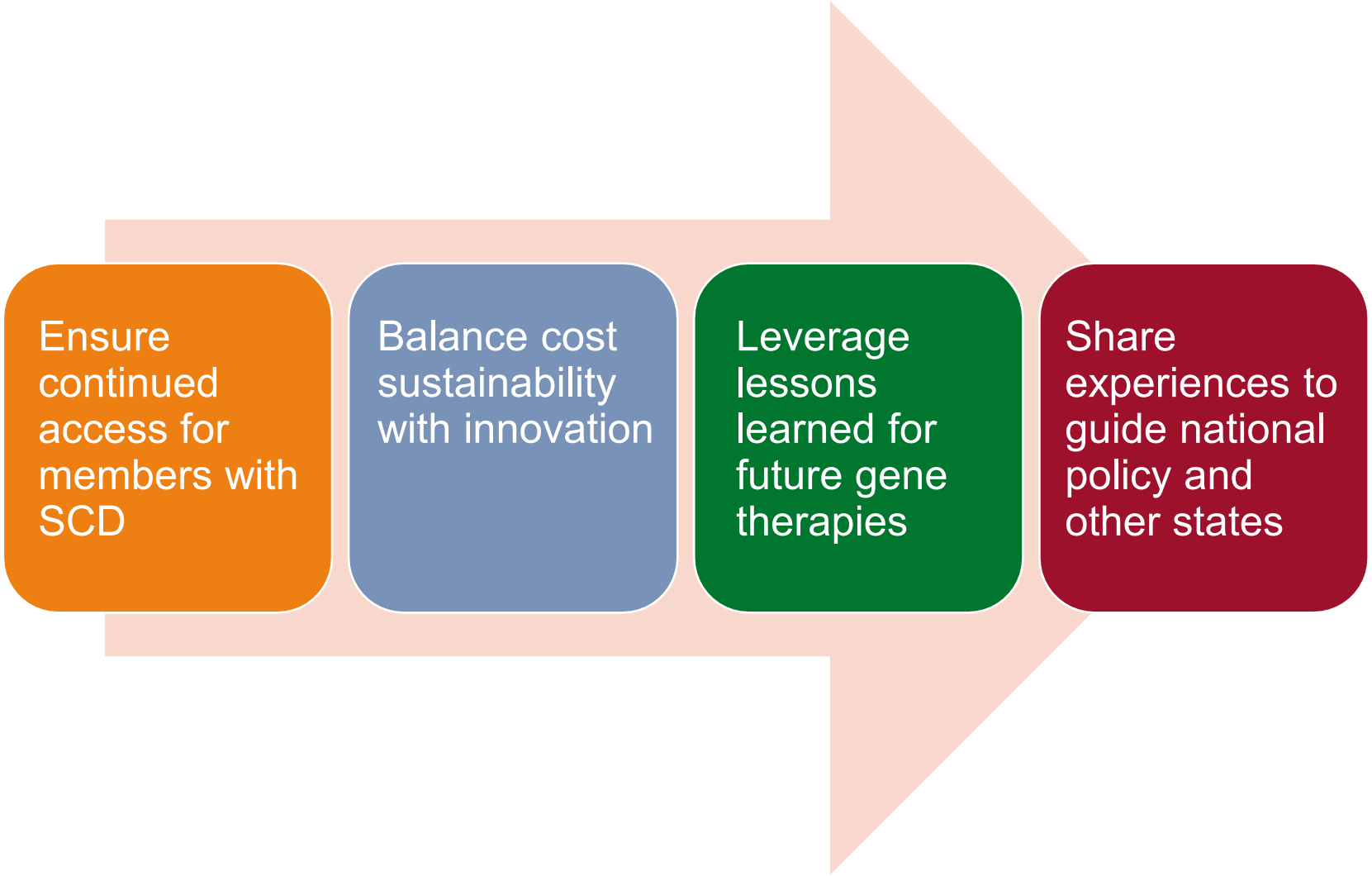
State Perspective: CMMI Access Model

- 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

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Mr. Jimi Olaghere

Sickle Cell Disease Patient Advocate

Q&A

CAPT Mahyar Mofidi

Director, HHS Office of Minority Health

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

THANK YOU!

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