

Cell and Gene Therapy (CGT) Model State Medicaid Director Briefing

Sick Cells

Cell And Gene Therapy (CGT) Model: Background

- **Goal:** Increase beneficiary access to cell and gene therapies while reducing health care expenditures
 - Sickle cell disease—first disease area of focus
- **How:** Create a standard framework for outcomes based agreements (OBAs) between States and pharmaceutical manufacturers
- **Who:**
 - CMS: Negotiate terms of OBAs with manufacturer, including pricing (reflects rebates), access standards and outcome measures to be met
 - CMS will reconcile data, monitor results, and evaluate outcomes with respect to the negotiated OBAs
 - Manufacturers: Must have an FDA approved therapy launched by May 2024, must participate in the MDRP
 - States: Any state or territory that participates in the MDRP
 - Can apply for option additional funding via NOFO issued by CMS
 - Beneficiary: Medicaid or Medicaid expansion CHIP
 - States may separately negotiate with manufacturers for coverage of beneficiaries covered by state's separate CHIP program

CGT Model Focus

- The Cell and Gene Therapy (CGT) Access Model seeks to **test a CMS-led approach** to negotiating and administering OBAs for cell and gene therapies.
- The Model will focus initially on CGTs for sickle cell disease (SCD), a genetic blood disorder that affects 100,000+ people in the U.S.
- SCD disproportionately impacts individuals from the Black and Brown communities. The CGT model aims to **improve access and health outcomes** for people with Medicaid; **address existing barriers to equitable access** to cell and gene therapies; and **reduce health care costs**.
- **Existing health disparities** have led people with SCD to experience:
 - An average lifespan more than 20 years shorter than average life expectancy in the U.S.
 - Excruciating pain episodes, which can cause multiple hospitalizations

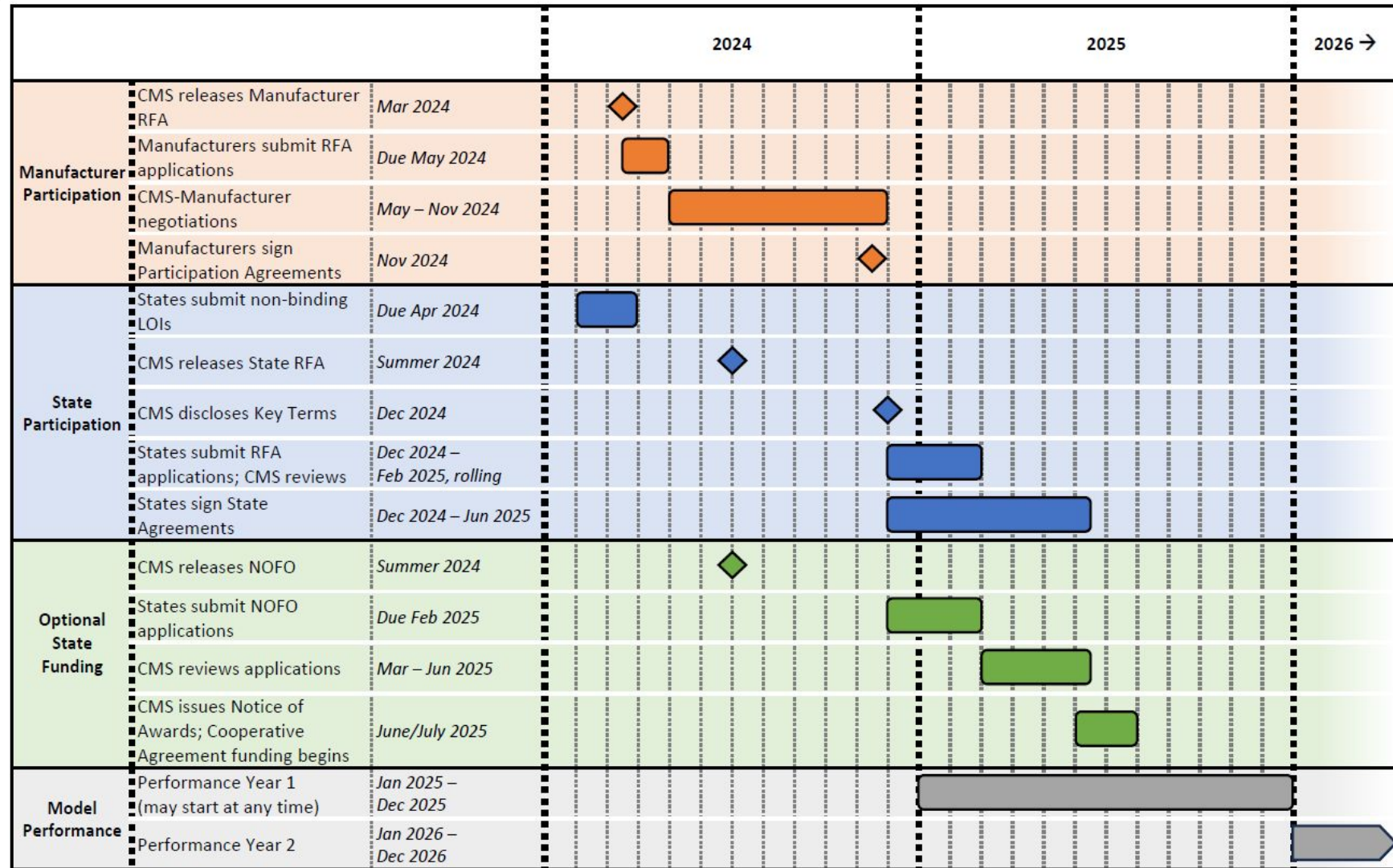
FDA-Approved Gene Therapies for SCD

What new gene therapies are FDA-approved for SCD, and how do they work?

- On December 8th, 2023, the Food and Drug Administration (FDA) approved **CASGEVY** and **Lyfgenia** for patients with sickle cell disease patients 12 years of age or older and a history of vaso-occlusive events (VOEs).
- Both therapies are **one-time gene therapies** that use the body's own stem cells to decrease or stop VOEs.
- CASGEVY uses your **own stem cells** that are collected from the patient. Therefore, no donor is needed. These cells are sent for gene editing done by CRISPR/CAS 9. This gene-editing tool will edit a specific gene called BCL11A., As a result, there is an increase in the production of **fetal hemoglobin**, which binds to oxygen very well however, produces less after birth. Once more **fetal hemoglobin** is made, this can stop the production of sickled cells and later prevent VOEs.
- Lyfgenia uses a similar method of using your **own stem cells**. This gene therapy uses a vector to deliver a functional gene called HbAT87Q, which mimics regular hemoglobin. It binds to oxygen like normal hemoglobin, limiting sickled cell production and reducing VOEs.

Source: [Sick Cells Gene Therapy FAQ](#)

Key Events



Legend Manufacturer activities State activities Funding timeline Model performance timeline

State Participation Timeline

**States Submit
Non-binding LOI**
April 1, 2024

**CMS Releases
State RFA**
Summer 2024

**CMS Discloses
Key Terms**
December 2024

**States Submit
RFA Applications**
February 2025

**States Sign
Agreements**
December 2024-
June 2025

Notice of Funding Opportunity (NOFO)

- A **notice of Funding Opportunity (NOFO)** is a formal announcement inviting award applications to states who would like to participate in the CGT Model.
- **States are not required to respond to the NOFO in order to participate in the CGT Model.**
- The **CGT Model NOFO** was released in **Summer 2024**
<https://grants.gov/search-results-detail/354875>
- A notice of awards and initial funding will be released in **Summer 2025**
- Information on the type of funding, available award amounts, the number of awardees to be selected, eligibility requirements, and the process for submission are forthcoming.

Application Timeframe

- The CGT Model is now accepting state applications through **March 14, 2025**.
<https://www.cms.gov/priorities/innovation/innovation-models/cgt>

Electronic Application Due Date	March 14, 2025, 11:59 pm EST
Anticipated Issuance Notice(s) of Award	July 1, 2025
Periods of Performance: <i>There are two periods of performance to be aware of in this Model</i>	
Model Performance Period (anticipated)	<i>January 1, 2025 – December 31, 2035</i>
Cooperative Agreement Period of Performance (anticipated)	<i>July 1, 2025 – December 31, 2035</i>

North Carolina SCD Therapies Data

Therapy	Step Therapy or “fail first”	Prior Authorization	Additional Information/PDL Status
Hydroxyurea	N/A	No PA Required	Not reviewed
Droxia	N/A	No PA Required	Not reviewed
Siklos	N/A	No PA Required	Not reviewed
Endari	N/A	No PA Required	Not Reviewed
Oxbryta	N/A	No PA Required	Not Reviewed
Adakveo	N/A	No PA Required	Not Reviewed

North Carolina SCD Agency Contact Information

Piedmont Health Services and Sickle Cell Agency

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Thank you!

For further questions, please feel free to contact:

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