

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

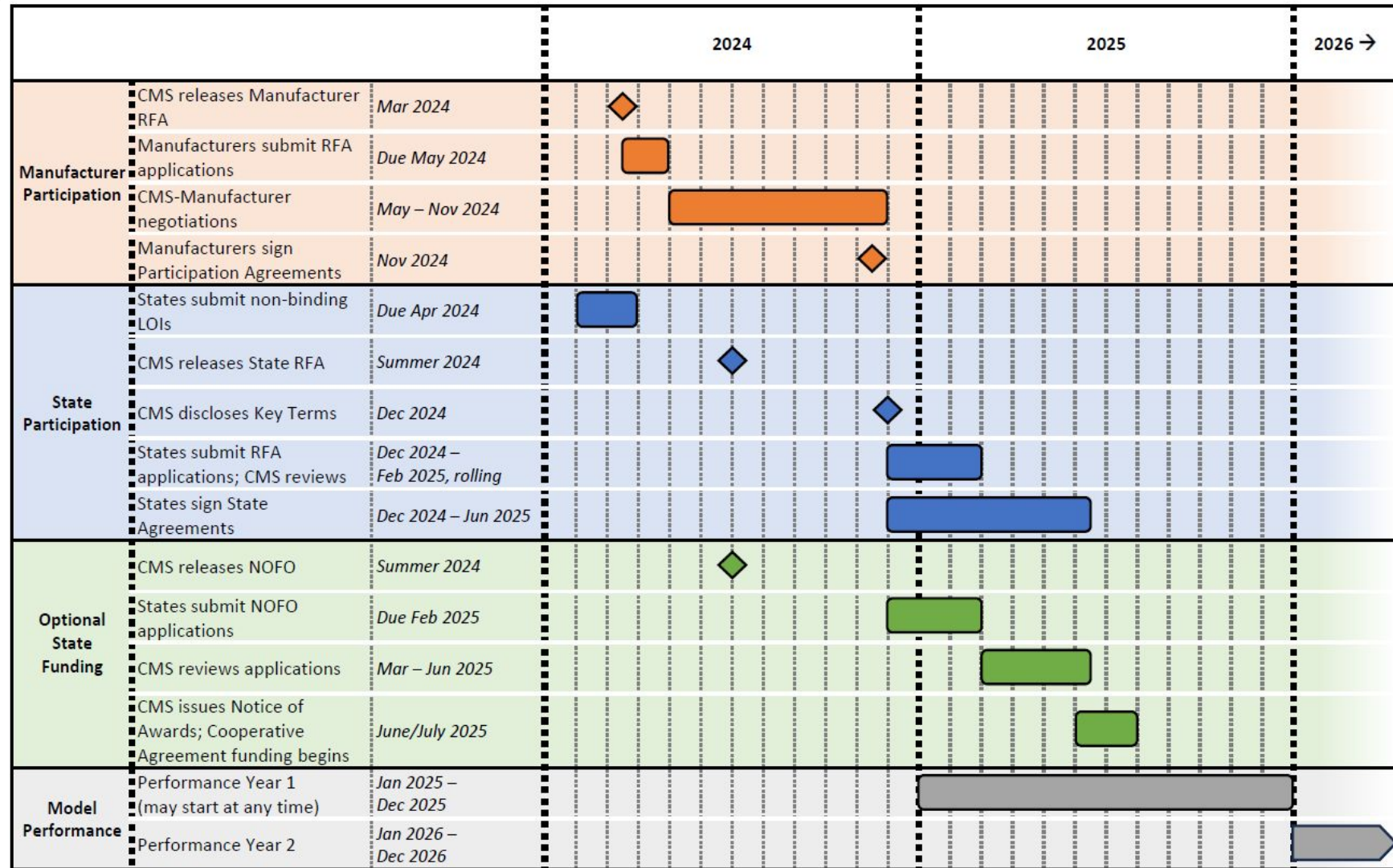
FDA-Approved Gene Therapies for SCD

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 **February 28, 2025**.
<https://www.cms.gov/priorities/innovation/innovation-models/cgt>

Electronic Application Due Date	February 28, 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>

New York 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	Not Required	No PA Required	Not Reviewed
Oxbryta	Not Required	No PA Required	Not Reviewed
Adakveo	Not Required	PA Required	Medical Benefit

New York CBO Contact Information

Sickle Cell Thalassemia Patient Network

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Thank you!

For further questions, please feel free to contact:

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