

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

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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

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- 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

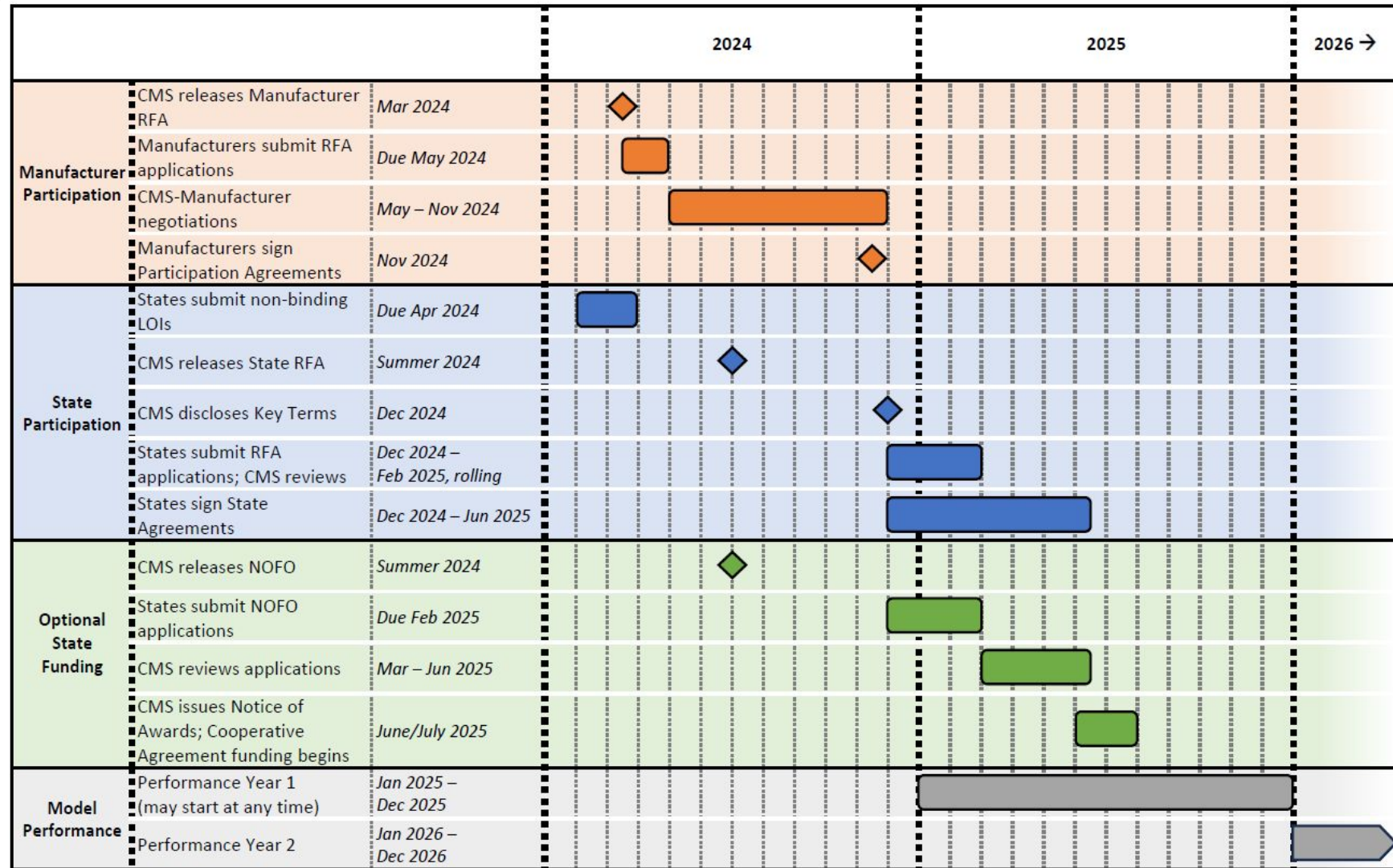
# FDA-Approved Gene Therapies for SCD

**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)

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- 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>

|                                                                                                         |                                            |
|---------------------------------------------------------------------------------------------------------|--------------------------------------------|
| <b>Electronic Application Due Date</b>                                                                  | February 28, 2025, 11:59 pm EST            |
| <b>Anticipated Issuance Notice(s) of Award</b>                                                          | July 1, 2025                               |
| <b>Periods of Performance:</b> <i>There are two periods of performance to be aware of in this Model</i> |                                            |
| <b>Model Performance Period</b><br>(anticipated)                                                        | <i>January 1, 2025 – December 31, 2035</i> |
| <b>Cooperative Agreement Period of Performance</b> (anticipated)                                        | <i>July 1, 2025 – December 31, 2035</i>    |

# Oregon SCD Therapies Data

| Therapy     | Step Therapy or “fail first” | Prior Authorization | Additional Information/PDL Status |
|-------------|------------------------------|---------------------|-----------------------------------|
| Hydroxyurea | N/A                          | No PA Required      | Preferred                         |
| Droxia      | N/A                          | PA Required         | Not Preferred                     |
| Siklos      | N/A                          | PA Required         | Not Preferred                     |
| Endari      | 3-month Hydroxyurea          | PA Required         | Not Preferred                     |
| Oxbryta     | 3-month Hydroxyurea          | PA Required         | Not Preferred                     |
| Adakveo     | 3-month Hydroxyurea          | PA Required         | Not Preferred                     |

# Thank you!

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For further questions, please feel free to contact:

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