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

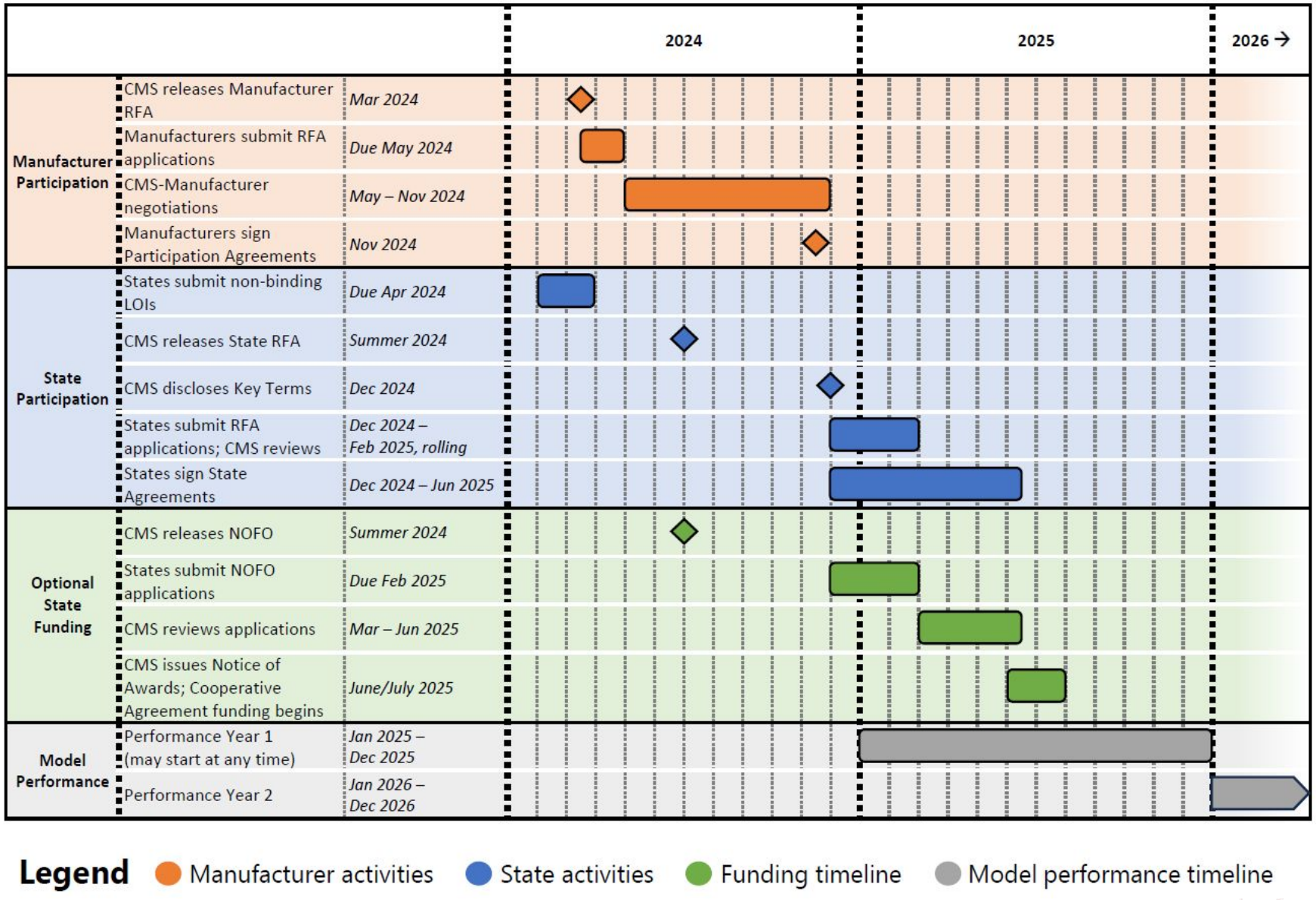
FDA-Approved Gene Therapies for SCD

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


2024
2025
2026 →
Manufacturer Participation
CMS releases Manufacturer RFA
Mar 2024
Manufacturers submit RFA applications
Due May 2024
CMS-Manufacturer negotiations
May – Nov 2024
Manufacturers sign Participation Agreements
Nov 2024
State Participation
States submit non-binding LOIs
Due Apr 2024
CMS releases State RFA
Summer 2024
CMS discloses Key Terms
Dec 2024
States submit RFA applications; CMS reviews
Dec 2024 – Feb 2025, rolling
States sign State Agreements
Dec 2024 – Jun 2025
Optional State Funding
CMS releases NOFO
Summer 2024
States submit NOFO applications
Due Feb 2025
CMS reviews applications
Mar – Jun 2025
CMS issues Notice of Awards; Cooperative Agreement funding begins
June/July 2025
Model Performance
Performance Year 1 (may start at any time)
Jan 2025 – Dec 2025
Performance Year 2
Jan 2026 – Dec 2026
Legend
Manufacturer activities
State activities
Funding timeline
Model performance timeline

State Participation Timeline

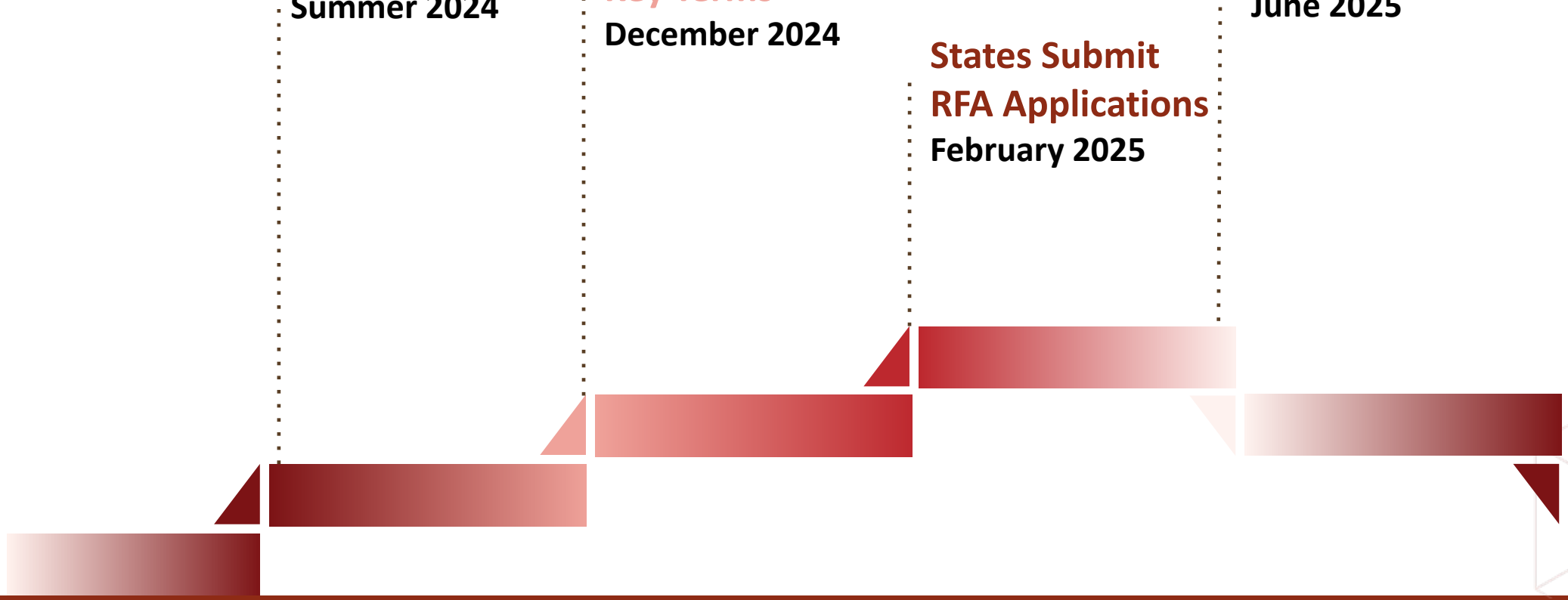
States Submit Non-binding LOI
April 1, 2024

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: *There are two periods of performance to be aware of in this Model*	
Model Performance Period (anticipated)	*January 1, 2025 – December 31, 2035*
Cooperative Agreement Period of Performance (anticipated)	*July 1, 2025 – December 31, 2035*

Rhode Island SCD Therapies Data

Therapy	Step Therapy or "fail first"	Prior Authorization	Additional Information/PDL Status
Hydroxyurea	N/A	N/A	Not Reviewed
Droxia	N/A	N/A	Not Reviewed
Siklos	N/A	N/A	Not Reviewed
Endari	N/A	N/A	Not Reviewed
Oxbryta	N/A	N/A	Not Reviewed
Adakveo	N/A	N/A	Not Reviewed

Thank you!

For further questions, please feel free to contact:

Timour Razek

trazek@sickcells.org

Sr. Program Manager, Sick Cells