Cell and Gene Therapy (CGT) Access Model for Sickle Cell Disease (SCD) Treatment

Overview for MAPOC

January 10, 2025

Key Background Points

- The **FDA has approved, and Medicaid is required to cover**, two new gene therapies for the treatment of sickle cell disease (brand names are Casgevy and Lyfgenia)

- The treatments are **potentially transformative** for individuals living with severe sickle cell disease; however treatments are highly involved, entail risk, and are costly: (Casgevy list price = $2.2M, Lyfgenia list price = $3.1M)

- CMS, on behalf of state Medicaid programs, has negotiated terms with both the manufacturers that include a "money back guarantee" known as an **outcomes-based agreements (OBAs)**

- Essentially, if the treatments do not work according to pre-established clinical criteria, the state is eligible to receive a partial refund. **OBA's are a type of value-based purchasing strategy** that could be particularly important as more high-cost treatments come to market.

- States **may choose to participate** in the CMS-negotiated terms*. Doing so entails meeting requirements related to tracking data, providing access, and supporting members receiving treatments

- CMS has made a funding opportunity available for states that opt to participate in the CGT Access Model to support these required activities in addition to some optional ones. (Up to $9.55M over a 10.5 year period per state)

- **Today's presentation focuses on the funding opportunity that DSS is pursuing.** (*Attribution note: we will be using selected CMS-created slides for our presentation. For source and detailed information see Cell and Gene Therapy (CGT) Access Model | CMS*)

* CT's participation will be subject to ongoing legal review of the terms and requirements

Overview of Sickle Cell Disease

CMS is initially focusing the CGT Access Model on gene therapies for sickle cell disease (SCD) to increase access to potentially curative therapies for all individuals with SCD for whom gene therapy may be an appropriate option.

Fast Stats

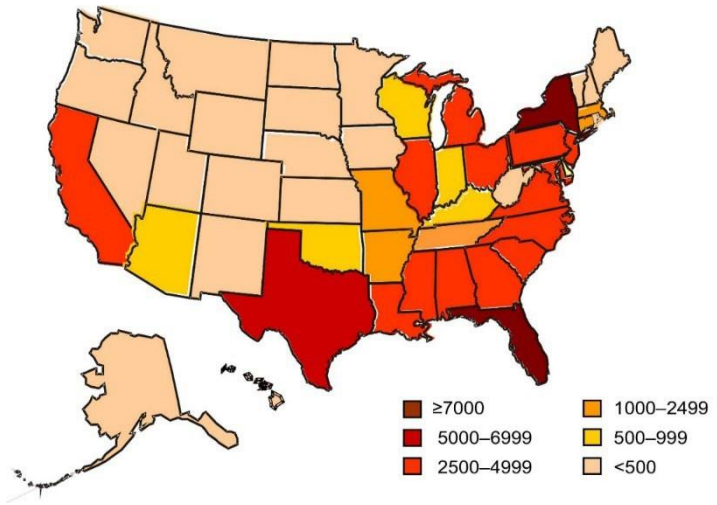
100,000+
People affected in the U.S.

~60%
Of people with SCD are enrolled in Medicaid

$2.98B
In costs per year to the U.S. health system *(mostly accrued to Medicaid)*

Description

SCD is a genetic blood disorder that affects 100,000+ people in the U.S., the majority of whom are Black Americans. This disease is unevenly spread across the U.S., as shown in the state-by-state patient counts to the right.



Biopsychosocial Challenges of Individuals with Severe SCD

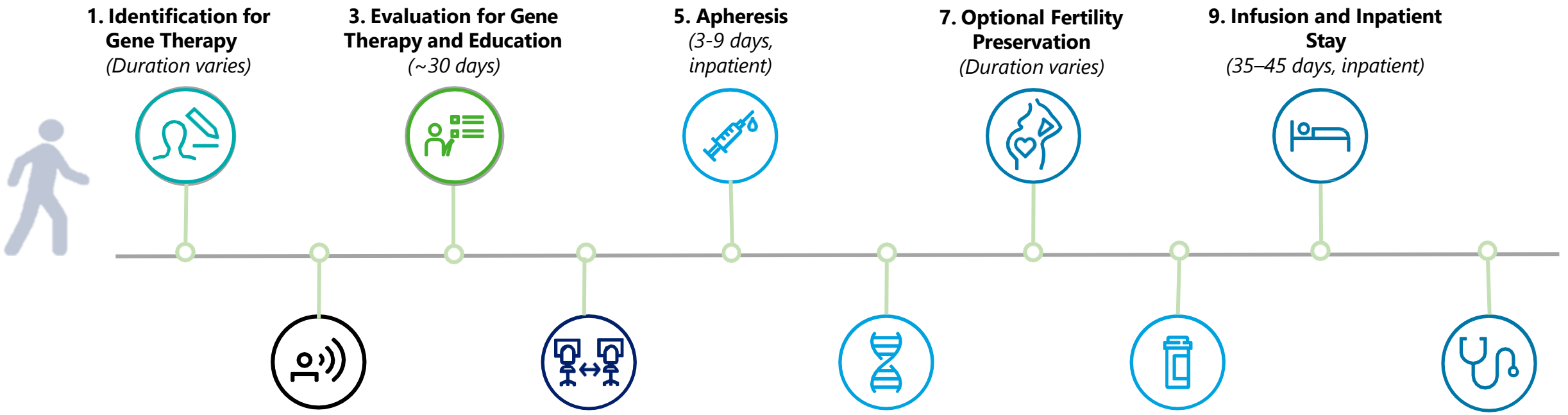
- The lifelong effects of SCD result in individuals' average lifespans being reduced by 20+ years compared to average life expectancy in the U.S.
- Individuals have excruciating pain episodes leading to multiple hospitalizations per year and the need for prescription pain medication.
- Frequent pain has broad effects on a patient's life, impacting educational attainment and employment.
- SCD gives rise to other conditions, such as mental health challenges.

Potential of CGTs

On December 8, 2023, the FDA approved two gene therapies for SCD, Casgevy and Lyfgenia.
Both products hold the promise of dramatically improving the lives of people with SCD by potentially **reducing or fully eliminating the occurrence of severe pain crises.**

Illustrative Patient Care Journey

The care journey for SCD gene therapy is long, rigorous, and complex.



Potential Care Delivery Gaps

Cooperative Agreement funding is designed to help states address some of the potential care delivery gaps individuals with Medicaid coverage may experience in the SCD gene therapy care journey.

Topic	Potential Care Delivery Gaps
Patient Knowledge	• Patient awareness of gene therapy • Patient knowledge of & access to non-emergency medical transportation (NEMT)
SCD Care	• Access to SCD specialist • Access to out-of-state providers
Other Specialty Care	• Access to behavioral health providers • Access to other specialty care services and providers
Social Needs	• Health-related social needs (HRSNs), including childcare
Care Coordination	• Care coordination / patient navigation • Navigating changes in insurance coverage

Eligibility for Cooperative Agreement Funding

Eligible applicants can apply to the NOFO to seek Cooperative Agreement funding.

WHO CAN APPLY?

Eligible applicants are states, the District of Columbia, and any U.S. territory participating in the Medicaid Drug Rebate Program (MDRP).

Eligible applicants must:

- ✓ Apply to the State RFA by no later than February 28, 2025
- ✓ Apply to the NOFO by no later than February 28, 2025
- ✓ Sign a State Agreement with CMS by no later than June 1, 2025

PARTNER ORGANIZATIONS

States can use Cooperative Agreement funding to partner with community-based organizations (CBOs), treatment centers qualified to administer gene therapy for SCD, and/or academic institutions. These organizations may be sub-recipients or contractors.

Implementation Funding

Implementation Funding will support required and optional model activities that involve staff/contractor time and infrastructure costs.

Applicants may request Implementation Funding for:

Required model activities

Activities that are necessary to implement requirements of the model.

Optional model activities

Activities that would increase access to SCD gene therapy and promote multi-disciplinary, comprehensive care for beneficiaries with SCD who are considering, or undergoing, gene therapy.

Cooperative Agreement funding cannot be used to pay for state share of any expanded Medicaid benefits or increased reimbursement rates. Funding may be used to pay for staff/contractor time and infrastructure costs related to implementing these benefits and services.

Excerpt of Required Model Activities

2. CMS and State: State Participation in Model (State Agreement)

Anticipated Effective Dates: January 1, 2025*** – December 31, 2035

Description:

1) Formalizes the terms of State participation in the Model.
2) Requires States to include Medicaid beneficiaries in the Model when Medicaid is the primary payer for a Model Drug. Beneficiaries enrolled in fee-for-service Medicaid must be included by the beginning of the Performance Period. Beneficiaries enrolled in Medicaid managed care plans must be included by no later than January 1, 2026.
3) Allows States to include separate CHIP beneficiaries in the Model by no later than January 1, 2026, subject to separate, optional agreement with Manufacturers.
4) Establishes State requirements for Model participation. For instance, States must:
    a. Have or obtain the necessary authority to implement the Model, including CMS approval of a SPA to enter into a VBP SRA.
    b. Establish a standardized Model Drug access policy consistent with the CMS-Manufacturer negotiated Key Terms.
    c. Carve Model Drugs out of an inpatient payment bundle, if necessary, and pay for the Model Drugs in a manner such that rebates under the MDRP apply.
    d. Require providers to follow Model-specific requirements related to registry participation and claims submission.
    e. Ensure that applicable Medicaid managed care plan policies align with Model requirements.
    f. Execute a VBP SRA with a participating Manufacturer that incorporates the CMS-Manufacturer negotiated Key Terms.
    g. If applicable, execute a VBP agreement for separate CHIP beneficiaries with a participating Manufacturer that incorporates the CMS-Manufacturer negotiated separate CHIP Key Terms.
    h. Attest that included beneficiaries will have access to gene therapy care with at least one qualified SCD gene therapy provider within the state or in another state.
    i. Attest that the State will ensure necessary transportation and related travel expenses to Model beneficiaries (and their caregivers, as applicable).
    j. Meet minimum data requirements and conduct data quality activities.
    k. Submit reports to CMS on Model implementation.

*** States will sign SAs on a rolling basis following CMS acceptance of their applications (which may be submitted between December 2024 and February 2025).

Source: Cell & Gene Therapy Access Model, page 17

Partnerships with Community-Based Organizations (CBOs)

States can use Implementation Funding to partner with CBOs with a focus on providing services to individuals with sickle cell disease (SCD).

Implementation Funding can be used to pay CBOs for the following purposes:

Increasing awareness and education of gene therapy among patients and health care providers

Increasing awareness and access to supportive ancillary services necessary for beneficiaries to receive gene therapy (e.g., transportation, nutritional, lodging, and childcare supports)*

Providing services to address health-related social needs (HRSNs)*

Providing community health worker/patient navigator and peer supports

*See the NOFO Section A6.8.1 for additional requirements applicable to the use of Implementation Funding to pay CBOs to provide housing and nutrition services and supports or childcare.

Milestone Funding

Milestone Funding will support successful completion of research projects related to increasing equitable access to SCD gene therapy and promoting multi-disciplinary, comprehensive care for beneficiaries with SCD who are considering or receiving SCD gene therapy.

Projects must answer a research question about how and whether the state's Medicaid (and CHIP, if applicable) beneficiaries with SCD were able to equitably access SCD gene therapy and/or receive multi-disciplinary, comprehensive care related to SCD gene therapy.

Projects must be related to the patient care journey for SCD gene therapy.

Projects may be designed to study:

The current state of patient care, barriers, and potential opportunities for improvement.

Example: A project designed to study challenges model beneficiaries experienced in accessing mental health services before and after gene therapy.

The effect of a policy change or intervention.

Example: a project designed to examine the effect of a state policy change on model beneficiaries' access to family planning services.

Application Timeline

CMS strongly recommends that states do not wait until the application due date to begin the application submission process.

Applications for both the State RFA and NOFO must be submitted by **February 28, 2025, at 11:59 pm EST**.

- NOFO applications must be submitted to Grants.gov.
- State RFA applications must be submitted through a separate application portal (which will open in December 2024).

June 2024
State RFA released

Aug 2024
NOFO released

Dec 2024
Key Terms disclosed to states; states may begin applying

Jan 1, 2025
Model PY1 begins

Feb 28, 2025
Applications due for both State RFA and NOFO

Jun 1, 2025
State Agreements must be signed

Jul 1, 2025
Anticipated issuance of Notice of Award, BP1 begins

Jan 1, 2026
Model PY2 begins

Please visit Grants.gov to view the NOFO application materials and begin the registration process.