CMS 2024 Quality Conference
Resilient and Ready Together

Creating an Optimal Environment for Quality Healthcare for Individuals, Families, and Communities

# Improving Care for People with Sickle Cell Disease

## Centers for Medicare & Medicaid Services (CMS) and the National Heart, Lung, and Blood Institute (NHLBI) at the National Institutes of Health (NIH) Updates

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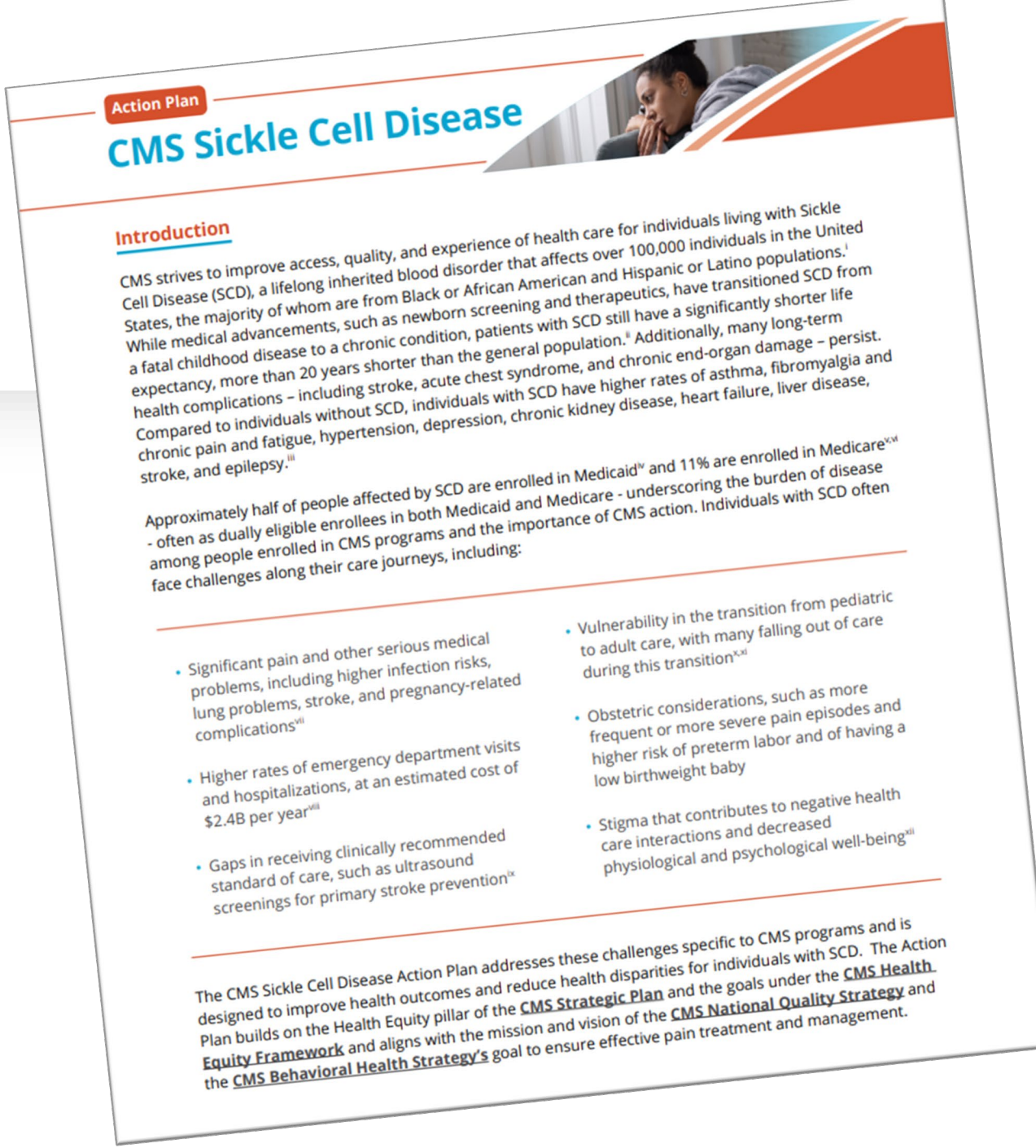
# CMS Sickle Cell Disease Action Plan

The Action Plan focuses on four key areas:

1. Expanding coverage and access
2. Improving quality and the continuum of care
3. Advancing equity and engagement
4. Examining data and analytics



Action Plan

CMS Sickle Cell Disease

## Introduction

CMS strives to improve access, quality, and experience of health care for individuals living with Sickle Cell Disease (SCD), a lifelong inherited blood disorder that affects over 100,000 individuals in the United States, the majority of whom are from Black or African American and Hispanic or Latino populations.[i] While medical advancements, such as newborn screening and therapeutics, have transitioned SCD from a fatal childhood disease to a chronic condition, patients with SCD still have a significantly shorter life expectancy, more than 20 years shorter than the general population.[ii] Additionally, many long-term health complications – including stroke, acute chest syndrome, and chronic end-organ damage – persist. Compared to individuals without SCD, individuals with SCD have higher rates of asthma, fibromyalgia and chronic pain and fatigue, hypertension, depression, chronic kidney disease, heart failure, liver disease, stroke, and epilepsy.[iii]

Approximately half of people affected by SCD are enrolled in Medicaid[iv] and 11% are enrolled in Medicare[v,vi] - often as dually eligible enrollees in both Medicaid and Medicare - underscoring the burden of disease among people enrolled in CMS programs and the importance of CMS action. Individuals with SCD often face challenges along their care journeys, including:

- Significant pain and other serious medical problems, including higher infection risks, lung problems, stroke, and pregnancy-related complications[vii]
- Higher rates of emergency department visits and hospitalizations, at an estimated cost of $2.4B per year[viii]
- Gaps in receiving clinically recommended standard of care, such as ultrasound screenings for primary stroke prevention[ix]
- Vulnerability in the transition from pediatric to adult care, with many falling out of care during this transition[x,xi]
- Obstetric considerations, such as more frequent or more severe pain episodes and higher risk of preterm labor and of having a low birthweight baby
- Stigma that contributes to negative health care interactions and decreased physiological and psychological well-being[xii]

The CMS Sickle Cell Disease Action Plan addresses these challenges specific to CMS programs and is designed to improve health outcomes and reduce health disparities for individuals with SCD. The Action Plan builds on the Health Equity pillar of the **CMS Strategic Plan** and the goals under the **CMS Health Equity Framework** and aligns with the mission and vision of the **CMS National Quality Strategy** and the **CMS Behavioral Health Strategy's** goal to ensure effective pain treatment and management.

# 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

**100k+**

**People affected in the U.S.**

**~60%**

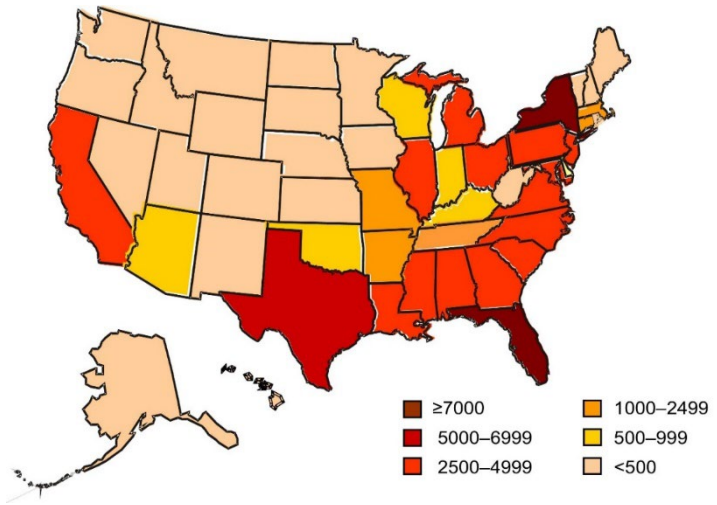
**Of people with SCD are enrolled in Medicaid**

**$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

- The lifelong effects of SCD result in individuals' 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.

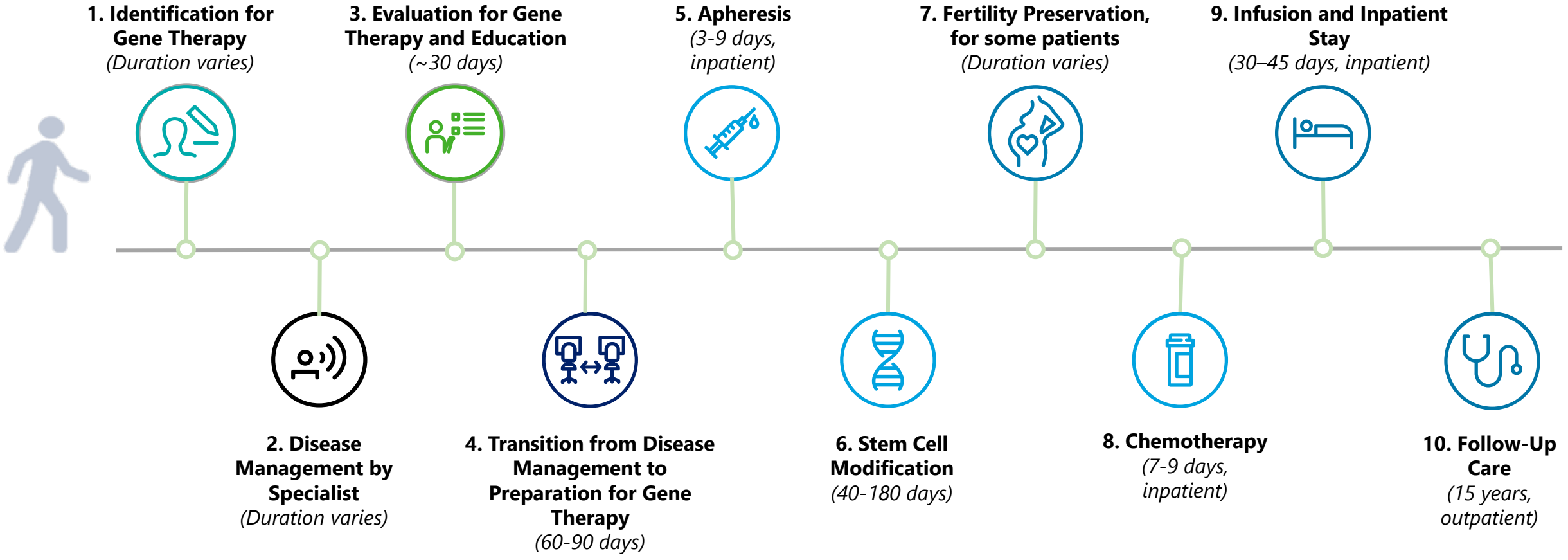
## Potential of CGTs

**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.**

# Care Journey

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



# Medicaid Coverage of Gene Therapy Drug

- Outpatient prescription drug coverage is an optional benefit that all state Medicaid agencies currently provide under the Medicaid statute (section 1905(a)(12) of the Social Security Act).
- State Medicaid agencies that provide outpatient prescription drug coverage are required to cover all covered outpatient drugs offered by any manufacturer that agrees to provide rebates.
- Drugs that are administered in an inpatient hospital setting are considered covered outpatient drugs if they are directly reimbursed.
- As both Casgevy and Lyfgenia are expected to be administered in inpatient settings, they may therefore be covered outpatient drugs and subject to Medicaid rebates, if they are directly reimbursed.
- States have discretion to establish certain utilization controls such as prior authorization.

# Separate CHIP Coverage of Gene Therapy Drug

- Prescription drugs are an optional benefit states may cover in a separate CHIP.

- Unlike Medicaid, separate CHIPs that cover prescription drugs are not required to cover all covered outpatient drugs offered by manufacturers that agree to provide rebates.
  - Separate CHIPs are not included in the Medicaid Drug Rebate Program, so this requirement does not apply to separate CHIPs.

- States have the option to seek rebates from manufacturers for prescription drugs covered in separate CHIPs, but they are **not** best price exempt.

- Therefore, states have the option to cover both Casgevy and Lyfgenia and states may establish utilization controls.

# State Opportunities in Value-Based Purchasing

- We encourage states to explore innovative contracting arrangements with willing manufacturers. [4]
  - For example, several states have received CMS approval to enter into value-based purchasing (VBP) supplemental rebate agreements with manufacturers. Such arrangements are intended to allow states to collect supplemental rebates for certain drugs when linked to an observed or expected therapeutic or clinical value in a select population.
  - States that have not yet done so may obtain CMS approval through a State Plan Amendment (SPA) to enable states and willing manufacturers to enter into such agreements.
  - Alternatively, manufacturers may offer a VBP arrangement to all states, even those states without an approved SPA, if they want to report varying best price amounts.

[4] https://www.medicaid.gov/sites/default/files/2022-03/state-rel-189-vbp.pdf

# Services Related to Gene Therapy

- The process for gene therapy includes:
  - Evaluation for gene therapy
  - Preparation during which patients may have changes in medications and transfusion therapy
  - Aphresis for cell harvesting
  - Chemotherapy and then infusion, both in an inpatient setting
  - Follow-up care and monitoring

- Federal law outlines mandatory Medicaid and CHIP benefits, which states are required to provide, and optional benefits that states may cover if they choose.
  - Examples of mandatory Medicaid benefits include inpatient hospital services, laboratory and X-ray services, physician services, and family planning services.
  - Examples of mandatory CHIP benefits include well-baby and well-child visits, mental health and substance use disorder prevention, and age-appropriate vaccines.

# Access to Out-of-State Providers in Medicaid

- Some beneficiaries will require access to out-of-state providers.
  - In accordance with 42 CFR 431.52(b)(3), if a state determines, on the basis of medical advice, that needed medical services are more readily available in another state (for example, if there are no in-state SCD gene therapy providers), then the state must pay for services from the out-of-state provider. Note: All applicable provider enrollment requirements must be followed.
  - While there are no similar requirements for separate CHIPs, states may provide access to out-of-state providers through the access to care assurances in the CHIP state plan as described at 42 CFR 457.495(c).

- The Medicaid transportation assurance is a requirement to make certain that every Medicaid beneficiary who has no other means of transportation has access to transportation needed to receive covered care.*
  - This also includes related travel expenses such as the cost of travel, lodging, and meals for beneficiaries and their caregivers as necessary for the beneficiary to receive the covered service.
  - This requirement generally does not apply to separate CHIPs, except for those that provide the Early and Periodic Screening, Diagnostic, and Treatment (EPSDT) benefit consistent with Medicaid requirements.

*For information, please see the Medicaid Transportation Coverage Guide.

# Medicaid Optional Benefit for Sickle Cell Disease (1/2)

- The American Jobs Creation Act of 2004 created an optional Medicaid SCD benefit under which States can cover additional services that might not otherwise be covered in the state plan.*
  - Under the optional SCD benefit, states may add new optional benefits (such as genetic counseling) for individuals with SCD or increase the rates at which they pay for mandatory or already covered optional benefits. As determined by the state, services may be provided via telehealth.
- While the American Jobs Creation Act of 2004 did not extend these options to separate CHIPs, states may elect to add SCD services as a covered benefit in separate CHIP.

[6]*See SMDL 05-003 for additional information.

# Medicaid Optional Benefit for Sickle Cell Disease (2/2)

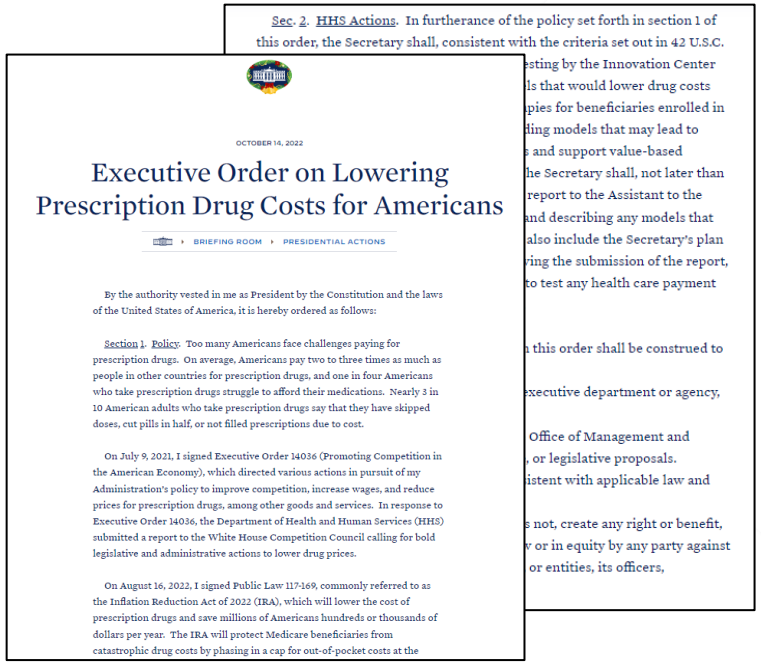
- Using this optional benefit, states may pay for SCD services at a different rate than they pay for similar services provided to individuals with other diseases.*
  - For example, under this benefit, if a state wanted to increase payment rates for SCD blood transfusions, it could do so through rate setting for the SCD benefit without having to increase payment for all Medicaid blood transfusions. Payment levels, however, must still be set within Federal requirements, including under section 1902(a)(30)(A) of the Act.
- States may also use this benefit to establish different coverage limits for SCD services under Federal amount, duration, and scope provisions at 42 CFR section 440.230 from those that apply to services in other benefit categories in section 1905(a) of the Act.
- The optional benefit also allows for payment for administrative expenditures for activities related to conducting public education campaigns if they are performed specifically with respect to SCD.

* See SMDL 05-003 for additional information.

# Model Background

The CGT Access Model aims to reduce healthcare costs by creating outcomes-based agreements between manufacturers and states.

The Cell and Gene Therapy (CGT) Access Model was developed in response to **President Biden's Executive Order 14087, Lowering Prescription Drug Costs for Americans** and intends to drive down prescription drug costs, building on the Inflation Reduction Act.



OCTOBER 14, 2022

Executive Order on Lowering Prescription Drug Costs for Americans

BRIEFING ROOM › PRESIDENTIAL ACTIONS

By the authority vested in me as President by the Constitution and the laws of the United States of America, it is hereby ordered as follows:

Section 1. Policy. Too many Americans face challenges paying for prescription drugs. On average, Americans pay two to three times as much as people in other countries for prescription drugs, and one in four Americans who take prescription drugs struggle to afford their medications. Nearly 3 in 10 American adults who take prescription drugs say that they have skipped doses, cut pills in half, or not filled prescriptions due to cost.

On July 9, 2021, I signed Executive Order 14036 (Promoting Competition in the American Economy), which directed various actions in pursuit of my Administration's policy to improve competition, increase wages, and reduce prices for prescription drugs, among other goods and services. In response to Executive Order 14036, the Department of Health and Human Services (HHS) submitted a report to the White House Competition Council calling for bold legislative and administrative actions to lower drug prices.

On August 16, 2022, I signed Public Law 117-169, commonly referred to as the Inflation Reduction Act of 2022 (IRA), which will lower the cost of prescription drugs and save millions of Americans hundreds or thousands of dollars per year. The IRA will protect Medicare beneficiaries from catastrophic drug costs by phasing in a cap for out-of-pocket costs at the

Sec. 2. HHS Actions. In furtherance of the policy set forth in section 1 of this order, the Secretary shall, consistent with the criteria set out in 42 U.S.C. esting by the Innovation Center ls that would lower drug costs pies for beneficiaries enrolled in ding models that may lead to s and support value-based he Secretary shall, not later than report to the Assistant to the nd describing any models that also include the Secretary's plan ring the submission of the report, to test any health care payment

this order shall be construed to

executive department or agency,

Office of Management and , or legislative proposals. istent with applicable law and

s not, create any right or benefit, or in equity by any party against or entities, its officers,

The model is a framework wherein **CMS negotiates with manufacturers on behalf of states** for outcomes-based agreements, or OBAs, for CGTs that cover beneficiaries for whom Medicaid is the primary payer.

## MODEL GOALS

- Improve Beneficiary Access to Transformative CGT Therapies
- Reduce Health Care Utilization and Expenditures
- Improve Health Outcomes

# Model Structure

The CGT Access Model seeks to test whether a CMS-led approach to negotiating and administering OBAs for CGTs, in the context of a comprehensive strategy for addressing a range of barriers to equitable access to cell and gene therapies, will improve access and health outcomes for people with Medicaid, and reduce health care costs.

## Role of CMS

CMMI will negotiate key terms and agreements between states and manufacturers, including CGT market access and rebate payments.

## Participant Eligibility

**All States and Territories** that participate in the Medicaid Drug Rebate Program (MDRP) can participate in the model if they meet requirements.

**Manufacturers** must participate in the MDRP and market FDA-approved or -licensed gene therapies for the treatment of severe SCD.

**CMS will negotiate discounted pricing**

with manufacturers to relieve the burden on states and increase access for beneficiaries.

**CMS will tie manufacturer payment to specific outcomes,**

such as reduction in pain-crises and patient-reported outcomes.

**CMS will offer optional funding**

to states to support activities that promote equitable access to care.

**CMS will support states to operationalize the model,**

such as providing technical assistance, specifying requirements on data collection, and negotiating the OBAs as well as collecting clinical and claims outcomes.

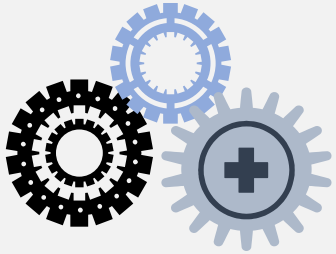
# Addressing Health Equity

The CGT Access Model aims to enable states to improve equitable access to included CGTs for all eligible Medicaid beneficiaries, in three key areas.

## COST

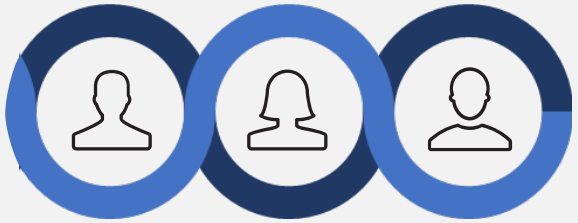
- Contracts between states and manufacturers reflecting CMS-negotiated key terms will potentially lower the cost of CGTs and enable more Medicaid beneficiaries to access potentially transformative treatment.

## ACCESS BARRIERS



- States will be offered optional funding for activities that reduce access barriers for people with Medicaid.
- Manufacturers will be required to **cover a defined scope of fertility preservation services,** as the care journey for SCD CGT typically results in infertility, which presents a significant access barrier.

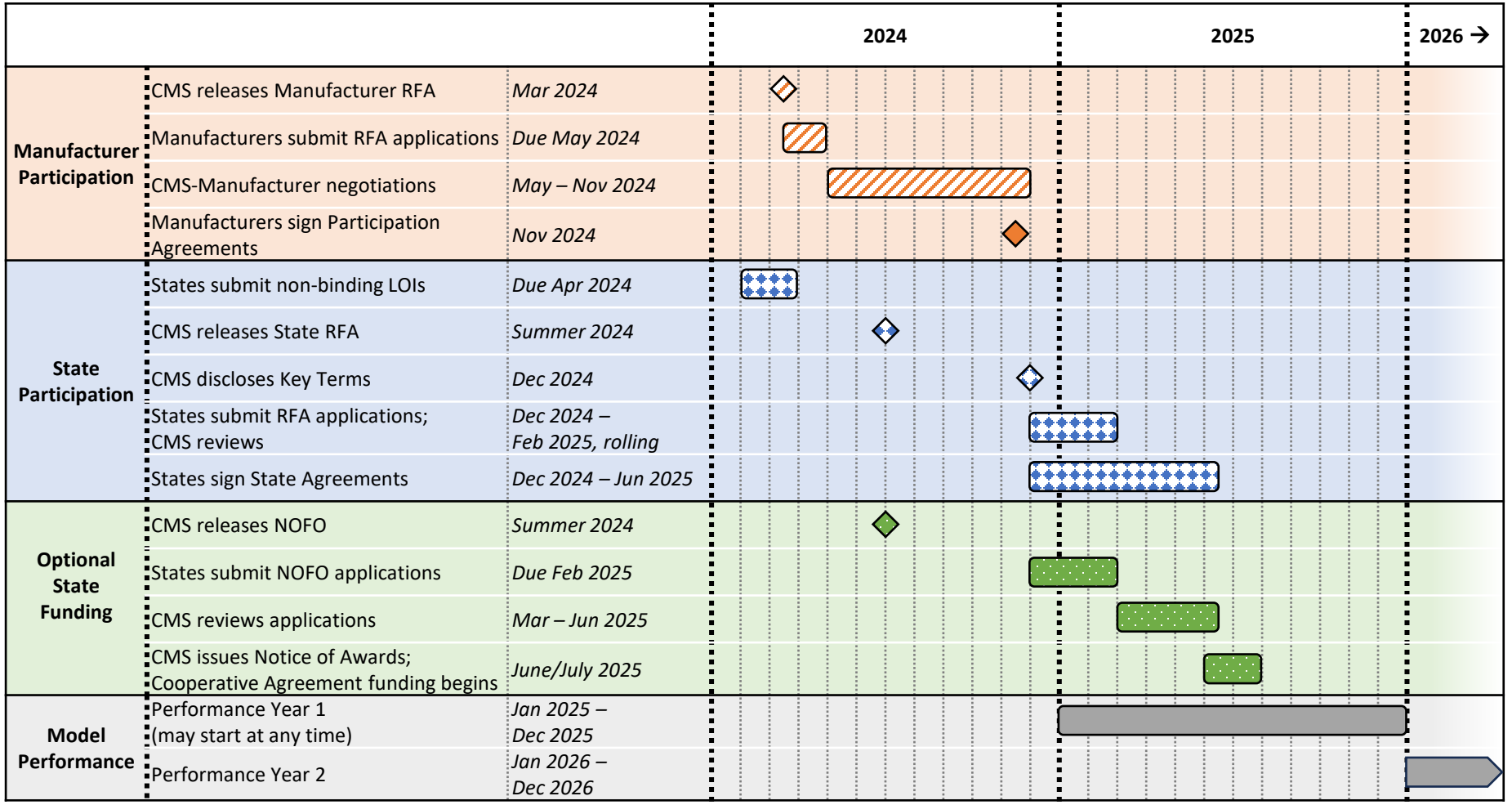
## HEALTH DISPARITIES



- Racial bias and treatment disparities are present among individuals with SCD, that have limited access to specialized care and treatments.
- By **increasing access to transformative therapies** for SCD, the Model could help address these historic disparities, poor health outcomes, and low life expectancy.

# Model and Application Timeline

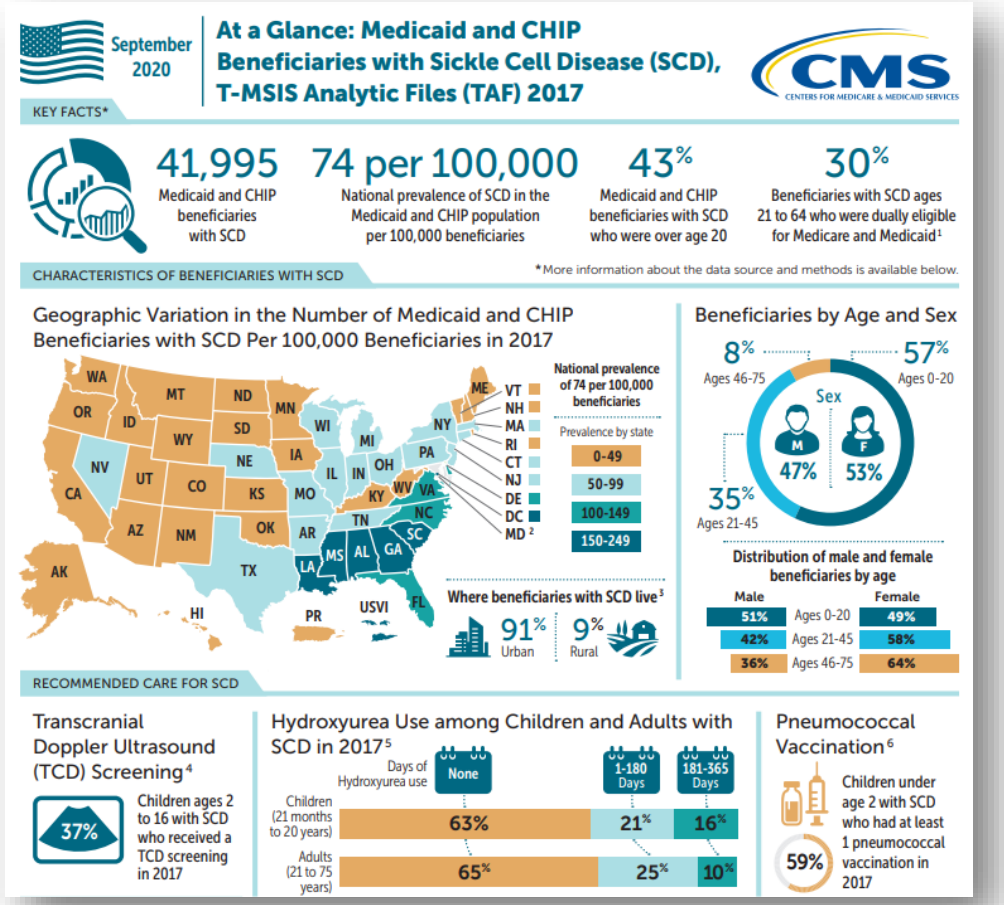
The CGT Access Model will operate for up to 11 years, depending on the OBA term for each state.




## LEGEND

- Manufacturer activities
- State activities
- Funding timeline
- Model performance timeline

# Opportunities to Improve Care in SCD




Infographic available at https://www.medicaid.gov/medicaid/quality-of-care/downloads/sickle-cell-disease-infographic.pdf

- States have opportunities to improve the care of children and adults with SCD.
  - Data show that gaps in recommended care for Medicaid and CHIP beneficiaries with SCD, specifically in rates of transcranial doppler ultrasound screening and pneumococcal vaccination for children, and in rates of hydroxyurea use among children and adults in 2017.
- The CMCS Quality Improvement (QI) Program provides state Medicaid and CHIP QI partners with information, tools, and expert support.
  - Technical assistance is available to help states build QI knowledge and skills, develop QI projects, and implement, spread, and scale-up QI initiatives.
  - Please contact MedicaidCHIPQI@cms.hhs.gov

For additional information on the CMCS QI program, see https://www.medicaid.gov/medicaid/quality-of-care/quality-improvement-initiatives/index.html

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# Resources

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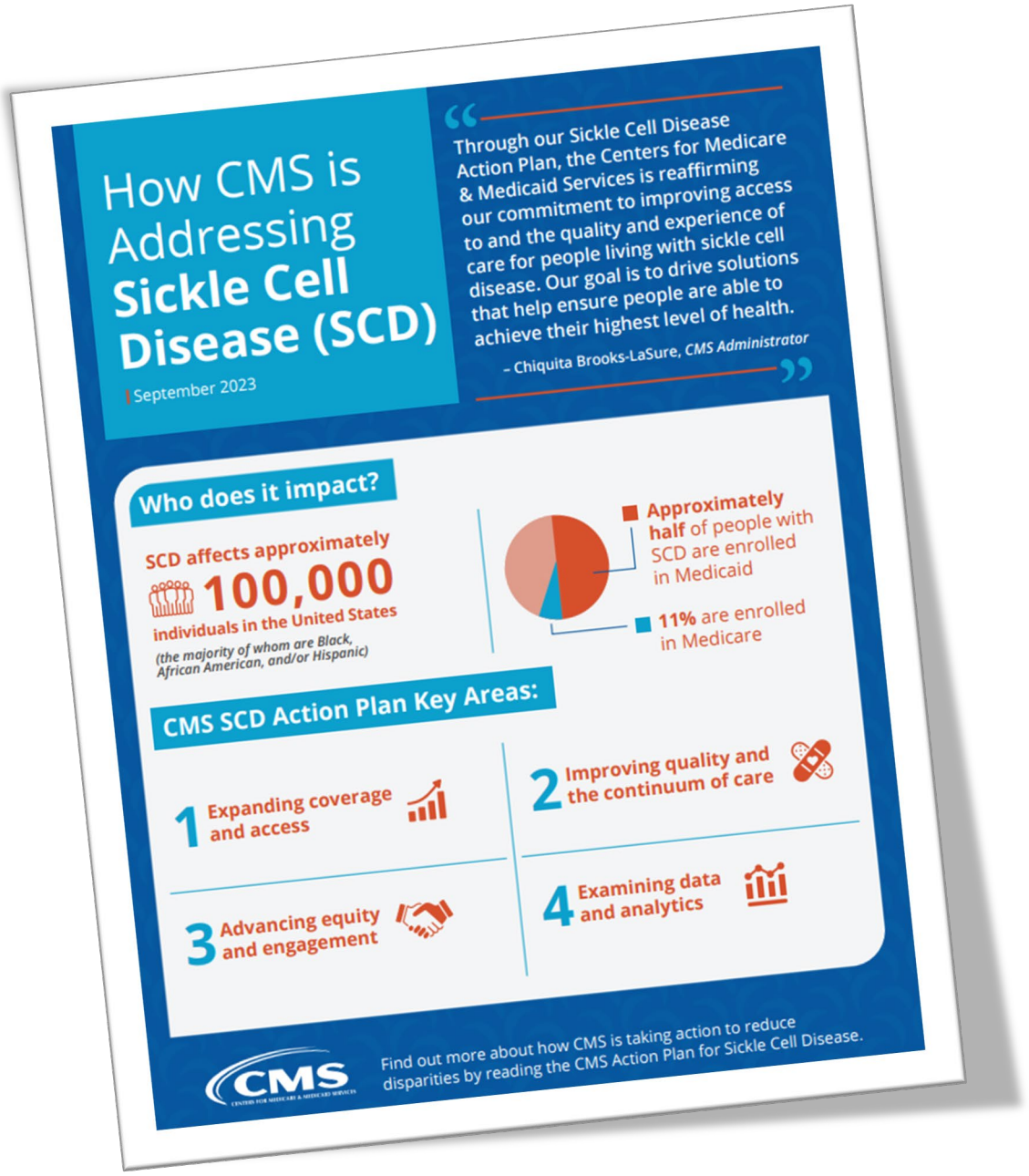



# CMS OMH Recognizes Sickle Cell Disease

Every year, CMS OMH recognizes National Sickle Cell Disease Awareness Month during the month of September.

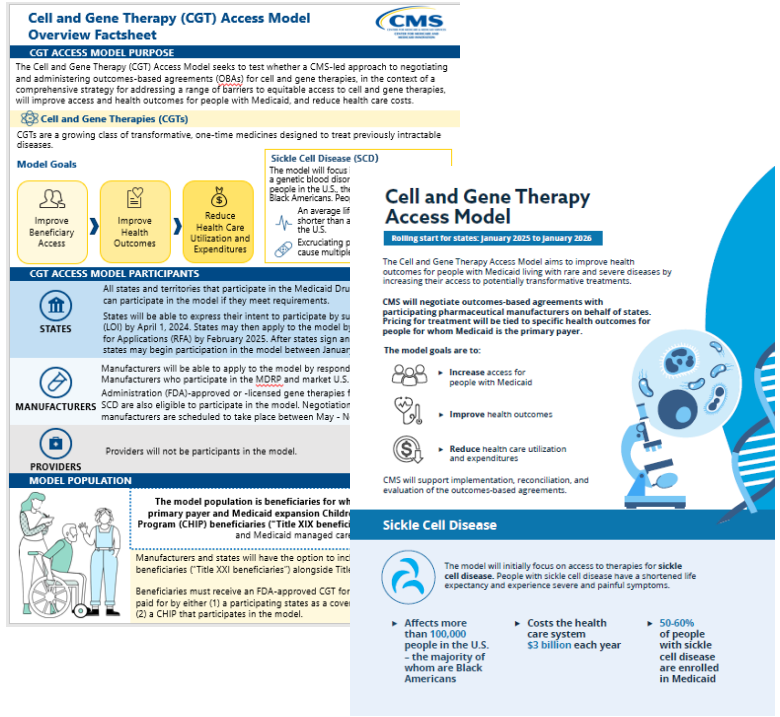
- CMS OMH has curated the following resources related to Sickle Cell Disease:
  - Sickle Cell Disease Video
  - Addressing Sickle Cell Disease Infographic
  - The Invisible Crisis: Understanding Pain Management in Medicare Beneficiaries with Sickle Cell Disease
  - Prevalence of Sickle Cell Disease among Medicare Fee-for-Service Beneficiaries Age 18-75 Years, in 2016

# HHS Recognizes Sickle Cell Disease

- National Institutes of Health's National Heart Lung Blood Institute (NIH/NHLBI) - "Today's Faces of SCD", where NHLBI highlights people living with SCD, their loved ones, researchers, and others, every Friday during the month of September.
- *Blood Diseases & Disorders Education Program (BDDEP)* webpage
  - Sickle Cell Awareness Month
  - SCD Fact Sheets
  - SCD: Milestones in Research and Clinical Progress Booklet
  - *Blood Health Network*
  - SCD Social Media Resources (graphics, GIFs, & post copy)
  - Follow us! *BDDEP* X (Twitter)
- The Cure Sickle Cell Initiative's webpage – highlights their patient-focused research effort designed to accelerate promising genetic therapies to cure sickle cell disease.
- Review the Centers for Disease Control & Prevention's Sickle Cell Disease Fact Sheet for a collection of resources to increase understanding of the disease.

# Model Resources

The CGT Access Model team has a host of resources to support interested organizations. To see the latest resources, visit the model's website at https://www.cms.gov/priorities/innovation/innovation-models/cgt.




## Model Factsheet and Infographic

Read through the CGT Model Overview Factsheet and the CGT Model Infographic on the model website to learn more.

## Helpdesk

If you have questions for the model team, please reach out to us via email at to CGTModel@cms.hhs.gov.

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# Questions

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