DEPARTMENT OF HEALTH AND HUMAN SERVICES
200 Independence Avenue, SW
Washington, DC 20201

CENTERS FOR MEDICARE & MEDICAID SERVICES
CENTER FOR MEDICARE AND MEDICAID INNOVATION

DATE: December 4, 2024[1]

TO: Treatment Centers that Administer Gene Therapies for Sickle Cell Disease (SCD)

FROM: Laura T. McWright, Deputy Director
Seamless Care Models Group
Center for Medicare and Medicaid Innovation

SUBJECT: Centers for Medicare & Medicaid Services, Cell and Gene Therapy Access Model

Dear Provider,

The Centers for Medicare & Medicaid Services (CMS) is reaching out to treatment centers that administer gene therapies for sickle cell disease (SCD) to provide information about the Center for Medicare and Medicaid Innovation's new Cell and Gene Therapy (CGT) Access Model. Your treatment center may have patients who qualify for the model; therefore, this letter summarizes:

- A clinical study involving treatment centers that CMS is supporting in conjunction with the model;
- Requirements of States that elect to participate in this initiative, including certain access criteria and direct reimbursement to treatment centers for gene therapies for SCD when Medicaid fee-for-service or Medicaid managed care is the primary payer for the individual's gene therapy;
- Supports available to qualifying patients in participating States;
- Optional research funding available to States; and
- A CMS-sponsored provider-focused learning community.

The CGT Access Model aims to maximize access for individuals with Medicaid coverage to novel and transformative therapies and is initially focused on gene therapy for SCD. Under the model, on behalf of States, CMS has negotiated the key terms of an outcomes-based agreement (OBA) with the two manufacturers of gene therapies for SCD, Vertex Pharmaceuticals Incorporated for CASGEVY™ and Genetix Biotherapeutics Inc. for LYFGENIA™. The OBAs tie payment for these gene therapies to outcomes for patients with SCD. The manufacturers will offer their OBA to State Medicaid agencies, who will implement this initiative for people with Medicaid with severe SCD who may benefit from gene therapy.

[1] This document was revised on February 18, 2025 and again on January 15, 2026. Revisions were non-substantive and do not impact any model requirements, policies, or processes.

States may choose to participate in the model with respect to one or both manufacturers. State Medicaid agencies may join the model as early as January 1, 2025, and no later than January 1, 2026.[2] CMS has worked closely with States and the manufacturers of gene therapies for SCD to develop the CGT Access Model coverage and payment terms. These terms aim to reduce administrative burden for providers and patients, support access to these therapies, and enhance our understanding of how these therapies impact patients who receive them through studying clinical and patient-reported outcomes.

We invite you to read the material provided within this document and visit the CGT Access Model website, where we will post the list of the States that have decided to participate in the model and how you can contribute to the learning generated through this initiative.

Center for International Blood and Marrow Transplant Research (CIBMTR) Study

As part of the model, CIBMTR is receiving federal funding under a cooperative agreement to implement an Investigational Review Board-approved study protocol which includes collection of clinical data and patient-reported outcomes data for people with Medicaid who receive gene therapies for SCD in all States.[3] Participating Medicaid programs will require that patients receiving gene therapies for SCD do so at treatment centers that are active members of CIBMTR and participate in CIBMTR's Research Database study protocol. Treatment centers will be responsible for approaching patients to request their consent to participate in the CIBMTR Research Database Protocol under which this study will be governed and submitting data to CIBMTR, per the CGT Access Model study requirements. CIBMTR will obtain patient consent to be contacted for the patient-reported outcomes (PRO) data collection and contact consenting patients to gather survey responses from patients and caregivers under its Protocol for Collection of PRO Data. CIBMTR member centers will receive information about the CGT Access Model study directly from CIBMTR. For more information about the CIBMTR study, please contact CGTModel@mcw.edu or visit the CIBMTR webpage.

Participating State Obligations Related to Providers

States participating in the model will adopt a range of policies to support access to gene therapies for SCD, including:

- Aligning coverage criteria and utilization management with the key terms negotiated by CMS and publishing the State's coverage criteria or utilization management policy;
- Making payment for gene therapy in the form of direct reimbursement and not as part of a bundled inpatient reimbursement in accordance with the State Plan; in general, this amount is not less than the provider's actual acquisition cost of the gene therapy;
- Issuing billing guidance to providers on how to properly submit claims for gene therapy, its administration, and related services;
- Maintaining a contract with at least one provider qualified to administer a gene therapy for SCD, whether in-state or out-of-state, and ensuring that such provider is enrolled in the State Medicaid program;

[2] During 2025, States can choose to participate with fee-for-service, managed care, or both. By 2026, they will have to participate with both fee-for-service and managed care.

[3] For more information on the supplement, see: https://reporter.nih.gov/search/7_c4fgQWqU6JeXF99k_1SQ/project-details/11160283

- Identifying a primary and secondary point of contact for providers regarding single-case agreements, prior authorizations, and provider enrollment; and
- Ensuring that Medicaid-managed care plans align with the model access, reimbursement, claims submission, and contracting requirements.

Additional Supports for Patients Available Through the CGT Access Model

Because chemotherapy administered prior to gene therapy infusion typically results in infertility, manufacturers participating in the model are required to financially support a defined scope of fertility preservation services at no cost to states participating in the model or people with Medicaid receiving the manufacturer's gene therapy for SCD who are living in a state that is participating in the model. The participating manufacturer will communicate with providers that administer its gene therapy regarding the scope of fertility preservation services that will be covered and which states are participating in the model so that they may inform potentially eligible people with Medicaid about the availability of these services.

In addition, participating States may partner with community-based organizations to address access barriers experienced by individuals for whom gene therapy for SCD is clinically appropriate.

Research Project Funding Opportunity

Participating States may apply for optional funding to conduct research projects related to increasing access to SCD gene therapy and promoting multi-disciplinary, comprehensive care for individuals with SCD who are considering or receiving SCD gene therapy. States may partner with treatment centers to conduct these projects; and if so, centers could receive funding as a subrecipient or contractor. For more information, please contact the relevant State Medicaid Agency to inquire about engagement with the CMS Notice of Funding Opportunity.

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CMS appreciates the opportunity to work with and learn from SCD treatment centers. For more information, please visit the Innovation Center's CGT Access Model website. If you have questions, you may contact the CGT Access Model team at CGTModel@cms.hhs.gov.

Sincerely,

Laura McWright
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CMS Innovation Center