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THE IMPORTANCE OF THE CELL AND GENE THERAPY MODEL FOR ACHIEVING EXPANDED ACCESS FOR SICKLE CELL DISEASE

Authors: Temi Afolabi, Maia Z. Laing, Ashley Valentine, MRes, Mariah J. Scott, MS, MPH

SUMMARY

The Cell and Gene Therapy Access Model (henceforth referred to as Access Model) is a voluntary model that tests whether a CMS-led approach to developing and administering outcomes-based agreements (OBAs) for cell and gene therapies (CGTs) improves Medicaid beneficiary access to innovative treatment, improves health outcomes for Medicaid beneficiaries, and reduces health care expenditures. In this issue brief, we outline how states should prioritize enrolling in the Model to improve the lives of this population.

Overview of the CGT Model

CGTs are a growing class of transformative, one-time medicines designed to treat previously intractable diseases. The Access Model will focus initially on CGTs for Sickle Cell Disease (SCD), a genetic blood disorder that affects 100,000+ people in the U.S. People with SCD have:

- An average lifespan more than 20 years shorter than average life expectancy in the U.S.
- Excruciating pain episodes, which can cause multiple hospitalizations.

The CGT Access Model is open to all U.S. states, D.C., and territories that participate in the Medicaid Drug Rebate Program (MDRP). Each state is eligible to fund up to $9.55 Million under Cooperative Agreements. Under the Notice of Funding Opportunity, there are two types of funding available under Cooperative Agreements:

Implementation funding to support required and optional Model implementation activities that involve staff/contractor time and infrastructure costs.

Optional Milestone Funding contingent on completed research projects related to expanded access to SCD CGT and promoting comprehensive care for SCD beneficiaries considering or receiving SCD gene therapy.

States should apply in response to the CMS Request for Applications (RFA) by **February 28, 2025.**

The importance of Applying the model to your state

- Individuals living with SCD and their families will have expanded access to cell and gene therapies to improve their quality of life
- Treatment centers in Maryland will have the resources and capacity to support SCD patients
- Providers and community-based organizations (CBO) will have access to the necessary resources required to support beneficiaries.

It is critical for states to participate in the Access Model to ensure that individuals living with SCD can receive life-changing CGT. Without participation, Medicaid beneficiaries with SCD and their families will continue to face barriers to access, limiting their ability to receive these innovative treatments that will improve their quality of life. Beneficiaries will also be forced to seek care out of state or forgo treatment altogether if your state forgoes participation in the model. Participation in this model also ensures that providers and CBOs have access to essential financial, educational, and logistical resources to support beneficiaries throughout their treatment journey. This will help enhance patient outcomes for one of the most underserved populations in the country.

Your State's Current Participation Status

To ensure Medicaid beneficiaries with SCD in Maryland have access to life-changing CGT therapies, it is critical to assess the state's engagement with the CMS Access Model. The following outlines Maryland's current participation status, including past engagement efforts, Medicaid leadership involvement, treatment centers and CBOs in your state or surrounding.

- As of 2024, Sick Cells implemented a State-Level Engagement plan with Maryland's Medicaid Director on the importance of applying the Model.
- As of February 18, 2025 Maryland has yet to formally announce participation in the CGT Access Model.

SCD Treatment Centers in Surrounding States

**No treatment centers in MD*

State	Treatment Center	Therapy
Virginia	Children's Hospital of Richmond at VCU	Lyfgenia
Pennsylvania	Children's Hospital of Philadelphia	Lyfgenia, Casgevy
Washington, DC	Children's National Hospital	Lyfgenia, Casgevy

SCD CBOs in Maryland

State	CBO
Maryland	Maryland Sickle Cell Association
	Sickle Cell Association of Harford and Cecil Counties

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