

Biden-Harris Administration Takes Next Steps to Increase Access to Sickle Cell Disease Treatments

The Centers for Medicare and Medicaid Services have entered into agreements with the manufacturers of Lyfgenia and Casgevy.

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Today [December 4], the Biden-Harris Administration announced that two drug manufacturers with Food and Drug Administration-approved gene therapies for sickle cell disease have entered into agreements with the Centers for Medicare & Medicaid Services (CMS) to participate in the Cell and Gene Therapy Access Model.

The [voluntary model](#), led by the Center for Medicare and Medicaid Innovation, will test outcomes-based agreements for cell and gene therapies, with the aim to improve health outcomes, increase access to cell and gene therapies, and lower health care costs. These outcomes-based agreements will tie payments to whether the therapy improves health outcomes for people with Medicaid who receive these drugs.

Having secured agreements with the manufacturers of [Lyfgenia and Casgevy](#), CMS will now move forward with engaging states and U.S. territories that participate in the Medicaid Drug Rebate Program to help them decide whether to participate in the model.

“The Cell and Gene Therapy Access Model will increase access to promising therapies that improve the chances of people living longer, healthier lives,” said CMS Administrator Chiquita Brooks-LaSure. “This is a new frontier in providing access for people with sickle cell disease to potentially transformative treatments. CMS is pleased that these two drug manufacturers have agreed to participate in the model and work with the agency and states in providing access to potentially curative treatments that might otherwise be out of reach.”

Sickle cell disease can be an extremely painful condition that significantly impacts overall quality of life for those affected. This disease disproportionately impacts Black and Hispanic Americans and has historically had limited treatment options. In the United States, more than 100,000 people live with sickle cell disease, with approximately 50% to 60% enrolled in Medicaid. Individuals with the disease have a shorter life expectancy, by more than 20 years on average. Hospitalizations and other adverse health episodes related to sickle cell disease cost the health system almost \$3

billion annually.

“Cell and gene therapies hold significant potential to improve patient outcomes and transform lives, ultimately reducing long-term health care spending,” said Deputy Administrator and Director of the CMS Innovation Center Liz Fowler. “However, due to the high costs, these therapies can pose challenges to state budgets. This model will afford state Medicaid agencies more budget predictability while helping improve access to these innovative therapies for people with Medicaid with sickle cell disease.”

[The FDA approved](#) Lyfgenia (lovotibeglogene autotemcel), from bluebird bio, Inc., and Casgevy (exagamglogene autotemcel), from Vertex Pharmaceuticals, Inc., in December 2023. CMS negotiated outcomes-based agreements with both manufacturers on behalf of states and will provide states that choose to participate in the model with the technical assistance and data infrastructure to implement these agreements.

The Cell and Gene Therapy Access Model launches in January 2025, and states may choose to begin participation anytime between January 2025 and January 2026. The [state application portal](#) goes live this month — December 2024 — and will remain open through February 28, 2025. In addition, states may apply for optional model funding by responding to the [notice of funding opportunity](#), but they are not required to respond to the notice of funding opportunity to participate in the model. The deadline for responding to the notice of funding opportunity is also February 28, 2025.

The model is part of the Administration’s broader effort to further increase access to novel therapies and drive down prescription drug costs. The Innovation Center developed the model in response to an Executive Order that President Biden issued in October 2022, directing the U.S. Department of Health and Human Services to consider developing models that address access to novel therapies and lower drug costs. After launching in 2025, this model may be expanded to other types of cell and gene therapies in the future.

For additional information, see the [Cell and Gene Therapy Access Model Overview Factsheet](#) and the [Cell and Gene Therapy Access Model page](#).

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