Medicaid Tries New Approach With Sickle Cell: Companies Get Paid Only if Costly Gene Therapies Work

Two new gene therapies offer a potential cure for many of the 100,000 primarily Black Americans with sickle cell disease.

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Serenity Cole enjoyed Christmas last month relaxing with her family near her St. Louis home, making crafts and visiting friends.

It was a contrast to how Cole, 18, spent part of the 2024 holiday season. She was in the hospital — a frequent occurrence with sickle cell disease, a genetic condition that damages oxygen-carrying red blood cells and for years caused debilitating pain in her arms and legs. Flare-ups often would force her to cancel plans or miss school.

“With sickle cell it hurts every day,” she said. “It might be more tolerable some days, but it’s a constant thing.”

In May, Cole completed a several-months-long gene therapy treatment that helps reprogram the body’s stem cells to produce healthy red blood cells.

She was one of the first Medicaid enrollees nationally to benefit from a new payment model in which the federal government negotiates the cost of a cell or gene therapy with pharmaceutical companies on behalf of state Medicaid programs — and then holds them accountable for the treatment’s success.

Under the agreement, participating states will receive “discounts and rebates” from the drugmakers if the treatments don’t work as promised, according to the Centers for Medicare & Medicaid Services.

That’s a stark difference from how Medicaid and other health plans typically pay for drugs and therapies — the bill usually gets paid regardless of the treatments’ benefits for patients. But CMS has not disclosed the full terms of the contract, including how much the drug companies will repay if the therapy doesn’t work.

The treatment Cole received offers a potential cure for many of the 100,000 primarily Black Americans with sickle cell disease, which is estimated to shorten lifespans by more than two decades. But the treatment's cost presents a steep financial challenge for Medicaid, the joint state-federal government insurer for people with low incomes or disabilities. Medicaid covers roughly half of Americans with the condition.

There are two gene therapies approved by the Food and Drug Administration on the market, one costing $2.2 million per patient and the other $3.1 million, with neither cost including the expense of the long hospital stay.

The CMS program is one of the rare health initiatives started under President Joe Biden and continued during the Trump administration. The Biden administration signed the deal with the two manufacturers, Vertex Pharmaceuticals [make of Casgevy] and Bluebird Bio [make of Lyfgenia], in December 2024, opening the door for states to join voluntarily.

"This model is a game changer," Mehmet Oz, the CMS administrator, said in a July statement announcing that 33 states, Washington, D.C., and Puerto Rico had signed onto the initiative.

Asked for further details on the contracts, Catherine Howden, a CMS spokesperson, said in a statement that the terms of the agreements are "confidential and have only been disclosed to state Medicaid agencies."

"Tackling the high cost of drugs in the United States is a priority of the current administration," the statement said.

Citing confidentiality, two state Medicaid directors and the two manufacturers declined to reveal the financial terms of agreements.

New Therapies

The gene therapies, approved in December 2023 for people 12 or older with sickle cell disease, offer a chance to live without pain and complications, which can include strokes and organ damage, and avoid hospitalizations, emergency room visits, and other costly care. The Biden administration estimated that sickle cell care already costs the health system almost $3 billion a year.

With many more expensive gene therapies on the horizon, the cost of the sickle cell therapies presages financial challenges for Medicaid. Hundreds of cell and gene therapies are in clinical trials, and dozens could get federal approval in the next few years.

If the sickle cell payment model works, it will probably lead to similar arrangements for other pricey therapies, particularly for those that treat rare diseases, said Sarah Emond, president and CEO of the Institute for Clinical and Economic Review, an independent research institute that evaluates new medical treatments. "This is a worthy experiment," she said.

Setting up payment for drugs based on outcomes makes sense when dealing with high treatment costs and uncertainty about their long-term benefits, Emond said.

“The juice has to be worth the squeeze,” she said.

Clinical trials for the gene therapies included fewer than 100 patients and followed them for only two years, leaving some state Medicaid officials eager for reassurance they were getting a good deal.

“What we care about is whether services actually improve health,” said Djinge Lindsay, chief medical officer for the Maryland Department of Health, which runs the state’s Medicaid program. Maryland is expected to begin accepting patients for the new sickle cell program this month.

Medicaid is already required to cover almost all FDA-approved drugs and therapies, but states have leeway to limit access by restricting which patients are eligible, setting up a lengthy prior authorization process, or requiring enrollees to first undergo other treatments.

While the gene therapy treatments are limited to certain hospitals around the country, state Medicaid officials say the federal model means more enrollees will have access to the therapies without other restrictions.

The manufacturers also pay for fertility preservation such as freezing reproductive cells, which could be damaged by chemotherapy during the treatment. Typically, Medicaid doesn’t cover that cost, said Margaret Scott, a principal with the consulting firm Avalere Health.

Emond said pharmaceutical companies were interested in the federal deal because it could lead to quicker acceptance of the therapy by Medicaid, compared with signing individual contracts with each state.

States are attracted to the federal program because it offers help monitoring patients in addition to negotiating the cost, she said. Despite some secrecy around the new model, Emond said she expects a federally funded evaluation will track the number of patients in the program and their results, allowing states to seek rebates if the treatment is not working.

The program could run for as long as 11 years, according to CMS.

“This therapy can benefit many sickle cell patients,” said Edward Donnell Ivy, chief medical officer for the Sickle Cell Disease Association of America.

He said the federal model will help more patients access the treatment, though he noted utilization will depend in part on the limited number of hospitals that offer the multimonth therapy.

Hope for Sickle Cell Patients

Before gene therapy, the only potential cure for sickle cell patients was a bone marrow transplant

— an option available only to those who could find a suitable donor, about 25% of patients, Ivy said. For others, lifelong management includes medications to reduce the disease's effects and manage pain, as well as blood transfusions.

About 30 of Missouri's 1,000 Medicaid enrollees with sickle cell disease will get the therapy in the first three years, said Josh Moore, director of the state's Medicaid program. So far, fewer than 10 enrollees have received it since the state began offering it in 2025, he said.

Less than a year into the federal program, Moore said it's too early to tell its rate of success — defined as an absence of painful episodes that lead to a hospital visit. But he hopes it will be close to the 90% rate seen over the course of a couple of years in clinical trials.

Moore said the federal program based on how well the treatment works was preferred over cutting fees for a new and promising therapy, which would put the manufacturers' ability to develop new drugs at risk. "We want to be good stewards of taxpayer dollars," he said.

He declined to comment on how much the state may save from the arrangement or disclose other details, such as how much the drug companies might have to pay back, citing confidentiality of the contracts.

Lately Cole, who underwent gene therapy at St. Louis Children's Hospital, has been able to focus on her hobbies — playing video games, drawing, and painting – and earning her high school diploma.

She said she was glad to get the treatment. The worst part was the chemotherapy, she said, which left her unable to talk or eat — and entailed getting stuck with needles.

She said that her condition is "way better" and that she has had no pain episodes leading to a hospital stay since completing the therapy last spring. "I'm just grateful I was able to get it."

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