Will the CMS CGT Model Support Patient Access to Sickle Cell Disease CGTs?

July 25, 2024
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Cell and gene therapies can transform the paradigm of care for patients with chronic, complex conditions, but these therapies come at an up-front cost of several million dollars per treatment, complicating the pipeline of access to them.

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With the potential to improve the disease course and outcomes for patients battling certain chronic conditions, cell and gene therapies (CGTs) have become a rapidly growing mode of treatment. In 2023 alone, the FDA approved 5 gene therapies for rare genetic diseases, with even more approvals expected in 2024.[1] CGTs could transform the paradigm of care for patients with chronic, complex conditions, but these therapies come at an up-front cost of several million dollars per treatment. Commercial and government payers have generally been able to absorb the costs of CGTs used in small,

ultrarare patient populations, but the increasing number of CGTs entering the market, including many for use in larger patient populations, may compel payers to implement innovative management and financing strategies.

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With that said, CGTs create other challenges for payers, including budgeting short-term costs relative to long-term clinical benefit, uncertainty around real-world clinical benefit and durability, and ensuring equitable access for their populations.[2]

On December 8, 2023, the FDA approved 2 transformative therapies for the treatment of sickle cell disease (SCD): exagamglogene autotemcel (Casgevy) and lovotibeglogene autotemcel (Lyfgenia). SCD is a group of inherited blood disorders that affects approximately 100,000 individuals in the United States and is most common in African American individuals and, to a lesser extent, those who are Hispanic American. SCD is caused by a mutation in hemoglobin, which results in blood cells taking on a crescent (or "sickle") shape. The misshapen red blood cells can restrict blood flow through vessels and oxygen delivery to tissues, resulting in symptoms such as severe pain and vaso-occlusive events or vaso-occlusive crises. Estimated life expectancy for patients with SCD is reduced by 20 years on average. Both therapies are approved for the treatment of SCD in patients 12 years and older. Both products are made from the patient's own blood stem cells, which are modified and then administered as a 1-time, single-dose infusion as part of a hematopoietic stem cell transplant. This procedure requires the patient to receive high-dose chemotherapy as preparation for the administration.[3-5]

Medicaid has been a key payer for CGTs, as many of these therapies treat rare genetic conditions that impact pediatric populations. CGT cost and logistical considerations have presented coverage challenges for the Medicaid program, with many states not being equipped with sufficient resources or policies to support patient and provider access to treatment. Value-based agreements (VBAs) are one tool that could be used by states to support patient access to CGTs and to address payer uncertainty regarding the efficacy and value of CGTs; however, VBAs require an up-front investment of time

and resources to implement, which has reduced their uptake at a state level.[6]

The availability of exagamglogene autotemcel and lovotibeglogene autotemcel for the treatment of SCD will put additional pressure on Medicaid budgets, as approximately half of individuals with SCD are enrolled in Medicaid.[4] Furthermore, the distribution of patients with SCD across the United States varies by region, with 85% of patients with SCD being concentrated in just 17 states, which may result in some states having a disproportionate share of patients with SCD being treated with a CGT.[7]

Is There a Way to Reduce the Pressure on Medicaid and Manufacturers?

The US federal government believes there might be a way. On January 30, 2024, the CMS announced the creation of the CGT Access Model. The model will be the first CMS-led approach to negotiate and administer outcomes-based agreements (OBAs). The hope for CGTs is that the model will improve access and health outcomes while reducing health care costs. Although this model will initially focus on CGTs for SCD, it may expand to CGTs for other disease states in the future. Participation in the CGT Access Model will be voluntary for both manufacturers and states, with participation being open to all states. The **Figure** details the general process.[8]

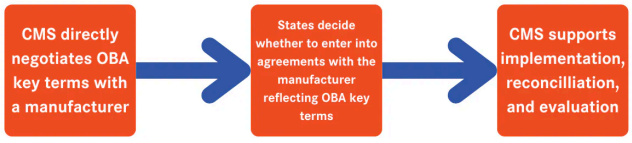

Figure. *CMS CGT Access Model General Process*[8]

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CGT, cell and gene therapy; OBA, outcomes-based agreement.

Beyond just focusing on the payment component of the gene therapy itself, the model also provides payment for wraparound services that can be crucial for positive outcomes with CGTs. For example, the model includes coverage for fertility preservation services and ancillary services to help connect patients to care, including travel expenses, case management, and behavioral health.8 This model has the potential to address many existing barriers to gene therapy, but as the old adage goes: "The devil is in the details." As those details are being worked out, we want to provide considerations to evaluate that could help deliver the program's promise.

The Details

The initial details provided by CMS in June 2024 spell out some specifics for how the program will operate. CMS will negotiate with CGT drug manufacturers to establish key terms for OBAs. Once finalized, these agreements will be available to all participating states. Throughout the model, manufacturers will be required to submit patient-level sales data to CMS. These data will be cross-referenced with patient claims data to ensure accuracy and compliance.

States will provide CMS with the necessary data for model operations and analysis through the Transformed Medicaid Statistical Information System (T-MSIS). CMS will also offer optional funding to states to support initiatives that promote equitable access to CGTs. States will be responsible for paying for the therapies but at a discounted rate, contingent on specific health outcomes negotiated by CMS.

The agreements between states and manufacturers, facilitated by CMS, will take the form of supplemental rebate agreements. The supplemental rebates will be in addition to the statutory rebates the states already receive. These agreements will allow states to include a separate Children's Health Insurance Program (CHIP), subject to different considerations. Manufacturers will be obligated to provide supplemental rebates to states, reflecting the terms negotiated by the model (eg, pricing, access standards, and outcomes). In turn, states must implement agreed-upon access policies.

CMS has provided the following time line for implementation of the OBA program:

- All states and territories that participate in the Medicaid Drug Rebate Program (MDRP) can participate in the model if they meet requirements.
- States will be able to express their intent to participate by submitting a letter of intent by April 2024. States may then apply to the model by responding to a request for applications (RFA) by February 2025. After states sign an agreement with CMS, states may begin participation in the model between January 2025 and January 2026.
- Manufacturers will be able to apply to the model by responding to an RFA by May 2024. Manufacturers who participate in the MDRP and market FDA-approved or -licensed gene therapies for the treatment of severe SCD are also eligible to participate in the model. Negotiations between CMS and manufacturers are scheduled to take place between May and November 2024.

Given that we are already halfway through 2024, the negotiations between CMS and manufacturers in the next few months will be a critical time, during which many details on how the OBA structures may emerge. With the deadline for states to apply for this model coming in February 2025, there is more work that needs to be done.

Considerations for Model Success

Although the CGT Access Model offers exciting potential to expand access to care for patients, significant questions must still be answered about how the program will be operationalized. In an evolving health care landscape, the implementation of state-level funding for patient education and provider awareness raises important questions about equity and efficiency. According to recently published guidance,[8] participating states can apply for additional, noncompetitive funding that will be available for implementation and at determined milestones. Additional funding will be available to states for optional activities related to increasing equitable access to care for eligible individuals. CMS plans to provide further information on this through a separate Notice of Funding Opportunity late this year.

An important area that will need to be ironed out is how stakeholders participating in the CMS OBA program collect, synthesize, and analyze data in situations where patients move or travel between states to receive a therapy—particularly for those patients for whom the nearest qualified treatment center is in a neighboring state. This concern might diminish as qualified treatment centers expand, allowing for more localized care delivery. CMS requires that applying states attest that patients will have access to a SCD gene therapy, including pre- and post care, through a qualified treatment center either in-state or with an out-of-state provider.

Interoperability issues due to varying electronic health record systems, state-specific data privacy regulations, and administrative hurdles may complicate seamless data transfer and continuity of care. Medicaid beneficiaries who relocate may face delays in coverage reactivation and difficulties accessing previous medical records, exacerbating care disparities.[9] With CGTs poised to provide clinical benefit for years after administration, CMS encourages states to consider entering into agreements with other states to aid Medicaid provider enrollment and payment terms in advance.

As noted above, the administration of outcomes will be managed through T-MSIS, which reviews and qualifies claims. This process involves multiple stakeholders, including medical

adjudicators, CMS, and state agencies—all of whom play essential roles in the triangulation of data. The integrity of this system is crucial, as claims management for multiyear OBAs not only requires frequent measures and audits by manufacturers and CMS but also a streamlined exchange of data to ensure effectiveness. Capturing accurate and comprehensive patient data is essential for determining a therapy's effectiveness and long-term value.

Although the implementation of T-MSIS in 2011 has significantly improved the reporting and management of CHIP and Medicaid programs ever since, issues remain around data quality, consistency, and the complexity of the reporting process. For instance, in a 2021 report, the Government Accountability Office found that 30 states did not submit acceptable data for inpatient managed care encounters, which are critical for ensuring proper beneficiary services and payments to managed care organizations.[10] Other challenges include consistency of eligibility data elements and the reporting of capitation payments. Some states have reported discrepancies in the plan types and provider identifiers, which complicate the linking of data across different files and undermine the reliability of the data.

States that apply must verify that they can meet the T-MSIS Outcomes-Based Assessment data quality targets by January 1, 2026, or provide an action plan to meet the targets. CMS will be responsible for gathering, aggregating, and analyzing data, as well as assessing whether the outcome measure benchmarks are met.

Furthermore, the access policy at the state level must be carefully considered. It is anticipated that states will align their policies with the FDA-approved indication and clinical trial population, who are unlikely to participate in an OBA that imposes more restrictive conditions than their approved labeling. Under the program, states can create additional criteria and access policies, but these cannot be more restrictive than the standardized policies that CMS negotiates with manufacturers.

The successful rollout of these programs has the potential to transcend government payers, influencing a significant portion of the payer mix in the private sector. If effectively executed, this model could dramatically enhance patient access to innovative therapies, reduce health care costs, and address disparities.

Each of these elements underscores the complexity of integrating innovative funding and treatment models within the

existing health care framework. The answers to these questions will shape the future of health care delivery and the extent to which innovative treatments can reach those in need.

Conclusion

The CMS CGT Access Model has the potential to make a significant step forward in the equitable and sustainable delivery of CGTs. By fostering collaboration among federal and state agencies, CGT manufacturers, and health care providers, this model aims to ensure that patients across the United States can access these innovative treatments. As this model evolves, it has the potential to set a precedent for other advanced therapies, ultimately leading to improved health outcomes and reduced health care disparities. Manufacturers, states, and other stakeholders are eagerly looking forward to learning additional details about how the program will work over the coming months.

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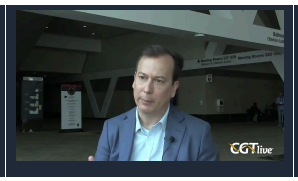

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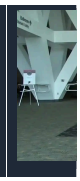
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The company also discontinued its other clinical programs as well, which included small molecule and monoclonal antibody modalities.

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