February 2024

CMS announces first focus of Gene Therapy Access Model

Overview

On January 30, 2024, Centers for Medicare & Medicaid Services (CMS) announced the first focus of the Cell and Gene Therapy (CGT) Access Model. The model will prioritize gene therapies for the treatment of sickle cell disease (SCD). The model is intended to increase access to CGTs while tying the costs of the therapies to clinical outcomes for Medicaid patients. The initial phase of the CGT Access Model will involve outcomes-based agreements (OBAs) for the treatment of SCD and will begin in 2025; CMS could expand the model to other types of CGTs in the future.

In addition to a CMS-negotiated OBA with participating manufacturers, CMS has indicated negotiations will also include additional rebates and a standardized access policy. States will have the opportunity to enter into an agreement with manufacturers based on the terms negotiated by CMS. Participating states would then be required to offer the agreed-upon standard access policy in exchange for rebates as negotiated by CMS. The states will still be responsible for their share of the cost of the CGT, but at a pricing rebate tied to specific outcomes, as negotiated by CMS. CMS will play a role in reconciling data, monitoring results, and evaluating outcomes to ensure the effectiveness of the CGT Access Model. The CGT Access Model is scheduled to begin in January 2025. States may begin participation at a time of their choosing between January 2025 and January 2026. This allows states to align their implementation plans with their specific needs and circumstances.

CMS aims to relieve the administrative burden on state Medicaid programs by negotiating with manufacturers on their behalf. This approach allows states to concentrate on enhancing access to CGTs and improving health outcomes for their Medicaid beneficiaries. By taking on the negotiation process, CMS aims to streamline the procurement and reimbursement processes, making it easier for states to provide these therapies to eligible patients. Additionally, CMS hopes to address other challenges for patients receiving CGT during the OBA negotiation process, including requiring manufacturers to include fertility preservation services when individuals receive gene therapy for treatment of SCD. CMS will also offer optional funding to states that engage in activities that increase equitable access to CGTs.

CMS plans to review the model in upcoming webinars with the public on February 6, 2024 and the states on February 8, 2024. Interested states are encouraged to submit a Letter of Intent (LOI) describing potential interest in participating in the model by April 1, 2024. The LOI is non-binding and not required to participate however CMS would like to gauge interest to better support the design of the model and the manufacturer negotiation process.

Two Gene Therapies Approved for Sickle Cell Disease

On December 8, 2023, the FDA simultaneously approved two gene therapies — Casgevy™ and Lyfgenia™ — both indicated for the treatment of SCD, a genetic blood disorder. Casgevy, priced at $2.2M, and Lyfgenia priced at $3.1M. SCD affects approximately 100,000 patients in the US and is more prevalent than the conditions targeted by other gene therapies on the market today; however, given the complex administration, chemotherapy-associated infertility, cost, and availability of other treatment options, market uptake of these therapies may be initially slow.

Health Equity Consideration

African Americans are disproportionately affected by SCD, with an incidence rate in the US of about 1 in 365 births. SCD patients have been a historically marginalized population, with experts indicating these patients often face systemic inequity in medical care. About 45% of SCD patients are Medicaid beneficiaries. State Medicaid programs should consider health equity when developing coverage criteria and considering the CMS-negotiated agreement for the new gene therapies, and how health disparities may impact access and pursuit of treatment in the patient population.

Strategies for State Medicaid Programs to Consider

State Medicaid programs should evaluate how to best manage these therapies in the fee-for-service and managed care environments. In particular, states should consider the following:

- Submitting an LOI, if interested.
- Attending the upcoming state/CMS webinar.
- CMS-negotiated OBA agreement and alignment with individual state goals and priorities related to OBAs.
- CMS has stated that “all states and U.S. territories that participate in the Medicaid Drug Rebate Program are eligible to apply to participate in the model.” States should ensure that current state plan and waiver documents align with CMS requirements for participation.
- Gene therapies are administered by healthcare professionals in specially designated treatment facilities; they require high-touch clinical support for optimal health outcomes and are typically billed through the medical benefit. In addition to the high cost of the drug itself, substantial administration and procedural fees may also come into play. States should evaluate site of service and claims payment and establish policy to ensure consistency and efficiency in payment and administration.

- Prior authorization and medical necessity criteria development, as appropriate. Health equity should be considered when developing coverage criteria. Additionally, states should also consider how health disparities may impact access and pursuit of treatment in the SCD patient population.
- Managed care capitation rate impact potential, in select populations.
- Risk mitigation arrangements such as risk pools, risk corridors, and/or individually carved out drugs, as appropriate.

For More Information

Please contact Dr. Bethany Holderread, Dr. Mark England, or your Mercer pharmacy consultant to talk through the potential impact of these new therapies and updates to your specific state program. You may also email us at: mercer.government@mercer.com.

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