CAPITOL STREET

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Cell & Gene Therapy CMS Model Prioritizes Sickle Cell

Agency Marches Forward; Dollars Provided for Health Equity Focus

Relevant Companies

Our Take & Next Up

We had said in February that sickle cell was the disease state that prompted the cell & gene outcomes-based arrangement creation (due to 50-60% SCD having Medicaid coverage). CMS announced today it is prioritizing SCD as the first therapeutic area of focus for the Cell and Gene Therapy (CGT) model (start January 2025). Link here. Participation is voluntary and it remains to be seen how many states will join the model in 2024 and 2025. At the same time, BLUE remains in discussion with Medicaid agencies on reimbursement for their recently approved sickle cell disease gene therapy, Lyfgenia. CMS anticipates releasing a Request for Application (RFA) to manufacturers (VRTX, CRSP, BLUE) in early spring 2024 and an RFA and Notice of Funding Opportunity to states in summer 2024.

Key Points

As a reminder, the Cell and Gene Therapy (CGT) Access Model was initially announced in February 2023 as directed by the Biden administration's executive order on prescription drug costs. Under the CGT Access Model, CMS will negotiate outcomes-based agreements (OBAs) with manufacturers on behalf of states and also support financial and clinical outcome measures development, reconciliation of data, and evaluation of results. Our CMMI analysis from October 2023 is here.

The 2025 cell & gene model will operate essentially as a supplemental rebate agreement. The model's goal is to increase state access through a central negotiation process for OBAs. The contract between states and manufacturers, with key terms as negotiated by CMS on behalf of states, will be structured as a supplemental rebate agreement. Negotiations will include additional pricing rebates and a standardized access policy. Manufacturers and states will have the option to include separate CHIP beneficiaries.

The agency highlights $3B costs per year in hospitalizations & health episodes related to sickle cell. CMMI notes that approximately 50% to 60% of people living with SCD are enrolled in Medicaid with a cost of $3B a year in hospitalization and other health episodes related to SCD.

SCD has a disproportionate implication on Black Americans, with 50-60% of approx 100,000 patients being on Medicaid. To make health equity a priority, CMS will also offer optional funding to states for additional activities that increase access to cell and gene therapies, including expanding or increasing reimbursement rates for behavioral health or care management.

The 11-year model is set to start in January 2025. States can express their intent to participate by submitting a Letter of Intent (LOI) by April 2024. Manufacturers will be able to apply by responding to a RFA by May 2024. Negotiations between CMS and manufacturers are scheduled to take place between May - November 2024. After states sign an agreement with CMS, states may begin participation in the model between January 2025 and January 2026. The model is expected to operate for up to approximately 11 years, depending on the OBA terms.

This is potentially good news for manufacturers (CRSP/VRTX and BLUE), but the unknowns around rebating levels make it tough to predict until we have more details. As a reminder, FDA approved SCD gene therapies cost $2.2 M for Casgevy (CRSP & VRTX) and $3.1 M for Lyfgenia (BLUE). Despite the potential to reduce future costs for sickle cell disease patients, the therapies present a huge cost challenge for Medicaid due to its list price. Additionally, it remains unknown how many people will access the therapies each year, complicating Medicaid agencies' ability to predict their costs. BLUE remains in discussions with more than 15 Medicaid agencies on reimbursement and the company is offering an outcomes-based agreement that is solely available to state Medicaid programs.

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