CAPITOL STREET

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Cell & Gene Model Update

BLUE & VRTX to Participate in CMMI Model, State Deadline February 2025

Relevant Companies

Our Take & Next Up

The Cell & Gene model at CMMI is moving forward, starting Jan 1, with CMS finishing out outcomes-based agreement (OBA) negotiations with sickle cell disease gene therapy manufacturers, BLUE & VRTX. Next, CMS will target states and U.S. territories to encourage participation (2025 model start). Key terms of the agreement will be disclosed to states this month and states may begin applying (application here). CMS is encouraging model participation, particularly for states (e.g., NY, southeast US, TX, FL) that have a high sickle cell disease burden. We will hear state participation announcements throughout 1H2025, and CMS will know all the state participants by June 2025. We anticipate that lower state participation may be a factor for the first few years of the model, as states may be wary of outcome-based agreements. Under a GOP trifecta (President Trump + GOP House & Senate), CMS could see (1) a more restricted budget and/or (2) less flexibility in funding for states as the federal government aims to reduce/reorganize on agency spending.

Key Points

Both Bluebird bio and Vertex have agreed to participate in the CMMI cell & gene model. Link here. Bluebird bio's LYFGENIA and Vertex/CRISPR's CASGEVY are the first gene therapies to treat sickle cell disease ($3.1 M and $2.2 M respectively). CMS negotiated outcomes-based agreements (OBAs) with both manufacturers and states are expected to provide the terms of the agreement this month. Throughout the pilot, manufacturers will submit patient-level sales data to CMS to cross-check against claims data of patients who receive the gene therapy.

Fertility will be covered & the model is scheduled to last 11 years. Manufacturers are required to cover certain fertility preservation services, due to the infertility risks of gene therapy (link here). The model will operate for up to approximately 11 years, depending on the OBA term for each state.

CMS pivots to encouraging state participation, with funding of up to $10 M per state, but may still prove to be challenging. As a reminder, the model establishes a multi-state approach for pursuing and administering outcomes-based agreements (OBAs) in Medicaid. States may receive optional additional funding under the model to ease implementation challenges. CMS will provide additional funding for implementation and milestone

funding for completion of research projects. CMS anticipates awards of up to $9.55 M for each state over the 10.5 years of the award, pending federal availability of funds

Approximately 50% - 60% of sickle cell disease patients are enrolled in Medicaid (NY, TX, PA, VA, LA, GA, MI, OH i.e., a mixture of Red & Blue states). Clearly, this is a policy priority due to CMS and state budget concerns. For SCD, states with high disease burden include New York, Florida, Texas, Pennsylvania, New Jersey, Virginia, Louisiana, Mississippi, Georgia, and North and South Carolina, Maryland, California, Michigan, Illinois, and Ohio.

The Cell and Gene Therapy Access Model launches in January 2025, and states may choose to start participation any time before January 2026. The state application portal goes live later this month and will remain open through February 28, 2025. Important dates to consider include:

- December 2024: Disclosure of key terms to states; states may begin applying
- January 1, 2025: Model Program Year 1 begins
- February 28, 2025: Applications due for Notice of Funding Opportunity (NOFO) and State Request for Applications (RFA)
- June 1, 2025: Deadline for states to sign agreement with CMS
- July 1, 2025: Anticipated issuance of Notice of Award, BP1 begins
- January 1, 2026: Model PY2 begins

BACKGROUND

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.

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 disease. CMMI notes the cost of $3B a year in hospitalization and other health episodes related to SCD. To increase access, 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.

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