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CMS Speeds Cell & Gene Therapy Model Start Date

FDA Accelerated Approval Model May Take Different Shape As Data Come In

Relevant Companies

Yesterday, CMS released a one-year update to the Biden administration's October 2022 executive order on prescription drug costs. The update provides new info related to the three CMMI selected models on drug affordability and access. The three models are (1) Medicare $2 drug list model, (2) cell and gene therapy access model, and (3) accelerating clinical evidence model. Link to blog here.

Our Take & Next Up

Cell & gene therapy model start will now be accelerated by a year to 2025 as IRA implementation begins, with litigation in the wings (and likely a successful NVO lawsuit). CMMI is aiming for rolling launch dates with states joining the model throughout 2025 (originally meant to launch in 2026). The update also notes that the models complement ongoing IRA drug provisions (including Part D redesign and drug negotiations). Drug negotiation implementation marches forward with all manufacturers agreeing to the negotiation process for 2026. CMS will now begin collecting data and information from companies, clinicians, researchers, and the public to inform their initial offer prices to manufacturers (expected by February 2024). Concurrently there are nine lawsuits proceeding. The latest lawsuit by NVO argues that CMS violated the IRA by "grouping together products with the same active ingredient or moiety" (in addition to the common 1st and 5th amendment violations). The novel argument likely puts NVO in a favorable position to win. Next up, the amended complaint in the Chamber's case (as requested by the Judge) is due October 13. On the government's side, the DOJ is due to respond to Bristol Myers Squibb's and J&J's combined cases by Oct. 16, to AstraZeneca's case by Nov. 1 and to Boehringer Ingelheim's case by Dec. 6.

Key Points

Cell & gene therapies are gaining regulatory traction with the FDA looking for ways to improve review (START Program). In the update, CMS noted the expansion of the cell and gene therapy pipeline and recent approvals. In September, the FDA launched the Support for Clinical Trials Advancing Rare Disease Therapeutics (START) pilot program for rare disease therapies including cell & gene therapies. The pilot allows six sponsors to request ad hoc communications with FDA on product-specific development issues, including clinical study design, and selecting patients for clinical trials. Earlier this year, the agency also restructured their cell & gene division by establishing the Office of Therapeutic Products (OTP) to improve review.

The CMMI cell and gene model will start earlier than expected – sickle cell is the potential budget-buster (BLUE, VRTX & CRSP). CMS is eager to get a handle on cell & gene therapy costs with recent approvals of Roctavian (BMRN) for Hemophilia A and accelerated approval of Elevidys (SRPT) for Duchenne muscular dystrophy (DMD). The agency is also mindful of the expectation of an increasing number of approvals in the coming years and

rising costs to states as a result. As a reminder, the model establishes a multi-state approach for pursuing and administering outcomes-based agreements (OBAs) in Medicaid. The agency is currently collecting stakeholder feedback on outcomes measurement, barriers to care for Medicaid beneficiaries, the need for complementary services, and cross-state access. It aims to have states join the model on a rolling basis throughout 2025.

For FDA accelerated approval reforms, CMS is finding that post market studies are indeed being completed for most new drugs in Medicare. CMMI found through its review of Part B accelerated approval drug trends:

- More than 90% of accelerated approvals for Part B drugs over the past five years were for oncology indications.
- The time to complete confirmatory trials for Part B drugs for oncology indications has remained below five years (60 months) since 2014.
- Outside of oncology, long delays in confirmatory trial completion occur for a small number of drugs that are often approved for orphan indications.

We believe that CMS may slow the accelerating clinical evidence model – likely to start 2025/26 -- to review both the FDA's new authorities and the shifting mix of products that are granted accelerated approval. The agency notes that there is still an opportunity for a model that would test ways to encourage completion of confirmatory trials. But CMS will have to factor in the FDA's new accelerated approval council and the agency's new authorities to require confirmatory trials before approval and ability to withdraw drugs with failed confirmatory trials. As a reminder, the accelerating clinical evidence model would make Part B payment adjustments to providers for drugs with accelerated approval as a potential method to encourage the completion of confirmatory trials.

$2 generic model is a no brainer based on current benefit design (though no start date provided). As a reminder, CMMI's generic model would allow Part D plans to offer a fixed (up to $2 per month supply) co-payment across all cost-sharing phases (up to the out-of-pocket limit) for a standard Medicare-defined list of generics. The agency is currently conducting background research on generic costs and benefits in Part D through a review of the 2023 Part D plan offerings. The review found that only 20.5% of Part D beneficiaries (8 million beneficiaries) are enrolled in plans offering a benefit equivalent to what is proposed for the model. Almost all these enrollees were in Medicare Advantage Prescription Drug (MA-PD) Enhanced Alternative (EA) plans as EA plans have the flexibility to use supplemental dollars from Part C bids to subsidize Part D beneficiary cost sharing. The model is expected to start soon (but no start date yet). The agency will release additional details about this model as soon as is feasible.

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