A Report in Response to the Executive Order on

Lowering Prescription Drug Costs for Americans

Frequently Asked Questions

The Medicare High-Value Drug List Model

1. What is the problem the model is trying to solve?

Beneficiaries often do not know the cost of a drug until they are filling their prescription at the pharmacy. Providers find it challenging to write prescriptions with patient out-of-pocket costs in mind because of variations in formularies and plan designs. This lack of price transparency may cause Medicare beneficiaries to forego taking prescribed medications or not be aware of lower-cost alternatives, leading to reduced adherence, lower quality of care, and increased overall health care costs.

2. How is the model trying to solve this problem?

The *Medicare High-Value Drug List Model* would define a standard set of high-value generic drugs that treat many of the most common conditions among Medicare beneficiaries, for example, hyperlipidemia and hypertension. Plans would offer the Medicare $2 Drug List, a list of drugs at a co-payment of $2 (or less) for a month's supply. The model would give beneficiaries and health care providers a standard list of effective, affordable medications with low out-of-pocket costs to consider when treating common conditions.

3. What are the proposed goals of the model test?

This model would test whether a standard list of high-value drugs would improve adherence to medications and improve access to high-value prescription drugs. Ultimately, this could lead to better health outcomes and lower spending for Medicare beneficiaries and, lower Medicare program costs.

4. How will the model affect Medicare beneficiaries?

The model would give beneficiaries an additional tool that may reduce their out-of-pocket drug costs. The *Medicare High-Value Drug List Model* would offer a standard list of drugs at a low monthly cost.

The Cell and Gene Therapy Access Model

1. What is the problem the model is trying to solve?

Cell and gene therapies (CGTs) are a rapidly-growing class of treatments that have the potential to treat or even cure previously intractable diseases, such as sickle cell disease, beta thalassemia, or cancer. Though treatment with CGTs can have potential for serious side effects and long hospital stays, there is growing interest in the beneficiary advocacy community, as well as

among patients and providers, in this new class of specialty drugs. The treatments are usually given in a single course that can cost as much as $1,000,000. The high, typically one-time expense puts financial pressure on state Medicaid agencies, making it difficult for them to finance CGTs, and causing them to increase cost-sharing (within limits) and/or impose restrictions on how patients can qualify for these potentially life-changing treatments, thus diminishing access.

2. How is the model trying to solve this problem?

The *Cell and Gene Therapy Access Model (CGT Access Model)* would create a new financing approach for patients enrolled in Medicaid to receive these potentially life-changing therapies to which they may not have had access. The model would allow state Medicaid agencies to delegate authority to CMS to coordinate and facilitate outcomes-based payment arrangements (OBAs) with cell and gene therapy manufacturers. The OBAs would align incentives, paying manufacturers based on the effectiveness of their treatments for patients.

3. What are the proposed goals of the model test?

The *CGT Access Model* would allow for collaboration among CMS, manufacturers, and state Medicaid agencies, and would test a new approach for administering OBAs that may help Medicaid beneficiaries gain access to potential life-changing, high-cost specialty drugs. The model would seek to expand access and reduce the cost of cell and gene therapies, while also reducing the administrative and financial burden on states to administer the OBAs on their own.

4. How will the model affect patients?

Medicaid beneficiaries, including those in underserved communities, could benefit from potential cures early in life or early in the course of the disease. This Model could expand access to cell and gene treatments for difficult-to-treat diseases and potentially reduce the amount patients would pay out-of-pocket for the treatments.

Paying for Drugs that Work: The Accelerating Clinical Evidence Model

1. What is the problem the model is trying to solve?

Under the Accelerated Approval Program (APP), the Food and Drug Administration (FDA) can approve a new product or a new indication for an approved product that addresses patients' unmet medical needs on an expedited basis based on surrogate endpoints that are reasonably likely to predict clinical benefit. APP also allows this for a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, and require a confirmatory trial. However, incomplete and delayed data from confirmatory trials may result in ongoing utilization of drugs that subsequently fail to confirm clinical benefit, which is concerning for patients and payers. The Consolidated Appropriations Act, 2023, among other things, provided FDA new authorities to require that confirmatory trials be ongoing at the time of accelerated approval or within a specified time period after the date of approval, and details expedited

procedures prior to withdrawal. Given these changes and the topic for this model, CMS plans to consult with FDA on model development.

2. How is the model trying to solve this problem?

The *Accelerating Clinical Evidence Model* would adjust Medicare Part B payment amounts for Accelerated Approval Program (AAP) drugs to give manufacturers an incentive to expedite and complete confirmatory clinical trials.

3. What are the proposed goals of the model test?

Giving manufacturers an incentive to expedite and complete confirmatory clinical trials may reduce the time and delays in completing confirmatory clinical trials. Faster trial completion may allow beneficiaries and prescribers to have more accurate and complete clinical information and, therefore may enable better treatment decisions.

4. How will the model affect patients?

Because of the high cost and lack of confirmed effectiveness of drugs receiving accelerated approval, private insurers, and government payers have begun closely evaluating their coverage of accelerated approval drugs, potentially limiting patient access to the latest treatments. With greater confidence that drugs on the market are effective, this model would seek to improve quality of care and reduce cost, and it could help ensure patients continue to have access to the drugs they need.

Additional Areas of Research

1. What is being proposed in the *Additional Areas of Research* section?

In addition to the three selected models, the Secretary has identified three areas for additional research. The ideas presented in this section require additional development and research and do not have a defined implementation timeline at this time. However, these areas represent additional areas of interest to the Secretary with the potential to lower prescription drug costs as prescribed in the executive order. Input from stakeholders on these ideas is welcome.