



Report to the Assistant to the President for Domestic Policy

A Report in Response to the Executive Order on Lowering Prescription Drug Costs for Americans

Secretary Xavier Becerra | U.S. Department of Health and Human Services

Table of Contents

Executive Summary	3
Introduction	5
Causes of High Drug Prices & Affordability Challenges	5
Prescription Drug Provisions in the Inflation Reduction Act of 2022	5
Executive Order 14087: “Lowering Prescription Drug Costs for Americans”	6
Role of the CMS Innovation Center	6
Approach for Developing Models	7
Stakeholder Input and Ideation	7
Criteria for Model Development and Prioritization	7
The Secretary’s Selected Models	7
1) Affordable Drugs Made Simple – The Medicare High-Value Drug List Model	8
2) The Cell & Gene Therapy Access Model	10
3) Paying for Drugs that Work: The Accelerating Clinical Evidence Model	14
Additional Areas of Research.....	17
1) Accelerating Biosimilar Adoption	17
2) Data Access Changes to Support Price Transparency	17
3) Cell and Gene Therapy Access in Medicare Fee-for-Service	18
Conclusion & Next Steps	18
Acknowledgements.....	18
Endnotes.....	19

Executive Summary

Prescription drug prices in the United States are higher than in other comparable countries.¹ The elevated prices result in affordability and access challenges for millions of Americans. In the past year, three in ten American adults reported not taking their medications as prescribed due to cost.² The underlying causes of high prescription drug prices are well documented and include market failures, such as lack of price and data transparency, and misaligned financial incentives for providers and others in the drug supply chain, which allow the growth of prescription drug prices to outpace inflation.³ These high prices can force Americans to make difficult choices between paying their household expenses and rationing their medication doses or worse yet, not filling their prescriptions at all. Medication non-adherence is strongly correlated with poor health outcomes and preventable medical costs.⁴

Enactment of the Inflation Reduction Act of 2022 (IRA) (P.L. 117-169) brought sweeping changes to help address rising prescription drug prices in Medicare Parts B and D. These changes include, but are not limited to, caps on beneficiary out-of-pocket spending, access to certain vaccines at no cost to the beneficiary, drug inflation rebates, and a provision that requires the Secretary of Health and Human Services (HHS) to negotiate the prices of certain drugs.

To build on the IRA, on October 14, 2022, President Biden issued Executive Order (EO) 14087, “Lowering Prescription Drug Costs for Americans,” to further address prescription drug affordability through the work of the Centers for Medicare & Medicaid Services’ (CMS) Center for Medicare and Medicaid Innovation (the Innovation Center).⁵ The Innovation Center tests innovative payment and service delivery models designed to reduce program expenditures while preserving or enhancing the quality of care furnished to its beneficiaries.⁶

The EO directs the Secretary of HHS to “consider whether to select for testing by the Innovation Center new health care payment and delivery models that would lower drug costs and promote access to innovative drug therapies for beneficiaries enrolled in the Medicare and Medicaid programs, including models that may lead to lower cost-sharing for commonly used drugs and support value-based payment that promotes high-quality care.”⁷ The EO further directs the Secretary to submit a report to the White House’s Assistant to the President for Domestic Policy no later than 90 days from the date of the EO “enumerating and describing any models that the Secretary has selected,” including “the Secretary’s plan and timeline to test any such models,” and to “take appropriate actions to test any health care payment and delivery models discussed in the report.”

To develop and prioritize model options for consideration by the Secretary, the Innovation Center used specific criteria consistent with the Center’s strategic priorities, notably, affordability, accessibility, and feasibility of implementation.⁸ To help identify model options, the Innovation Center solicited input from experts within CMS, other federal agencies, and over 40 external stakeholders, including but not limited to beneficiary and caregiver advocacy groups; trade associations representing manufacturers; payers and pharmacy benefit managers (PBMs); independent consultants; academic research institutions; hospital systems; provider groups; and data vendors.

This report in response to the EO (the Report) describes three models the Secretary has selected for testing by the Innovation Center that will complement the prescription drug provisions contained in the IRA. The Secretary believes the selected models will help lower the high cost of drugs and promote accessibility to life-changing drug therapies while maintaining and/or improving quality of care and beneficiary experience. In addition to the selected models, the Secretary identifies three areas for additional research.

Table 1. The Secretary’s Selected Drug Affordability & Accessibility Models

Area	Model	Test Question	Design
Medicare Part D	<p data-bbox="342 254 610 317">Medicare High-Value Drug List</p> 	<p data-bbox="662 260 1057 495">What is the impact of standardizing the Part D benefit for high-value generic drugs on beneficiary affordability, access, health outcomes, and Medicare spending?</p>	<p data-bbox="1101 260 1516 495">Part D plans would be encouraged to offer a low, fixed co-payment across all cost-sharing phases of the Part D drug benefit for a standardized Medicare list of generic drugs.</p>
Medicaid	<p data-bbox="342 758 610 821">Cell & Gene Therapy Access</p> 	<p data-bbox="662 764 1045 999">Does a CMS-led approach to administering outcomes-based agreements for certain cell and gene therapies improve beneficiary access and equity and reduce health care costs?</p>	<p data-bbox="1101 764 1516 999">State Medicaid agencies would assign CMS to coordinate and administer multi-state outcomes-based agreements with manufacturers for certain cell and gene therapies.</p>
Medicare Part B	<p data-bbox="342 1262 610 1325">Accelerating Clinical Evidence</p> 	<p data-bbox="662 1268 1078 1629">Do targeted adjustments in Part B fee-for-service payments for drugs approved by the Food and Drug Administration (FDA) under the accelerated approval pathway improve timely confirmatory trial completion and reduce Medicare spending, while maintaining or improving quality of care?</p>	<p data-bbox="1101 1268 1500 1587">CMS would develop payment methods for drugs approved under accelerated approval, in consultation with FDA, to encourage timely confirmatory trial completion and improve access to post-market safety and efficacy data.</p>

Introduction

Causes of High Drug Prices & Affordability Challenges

Americans pay more for their prescription drugs than people in other nations. The HHS Assistant Secretary for Planning and Evaluation (ASPE) examined prices for prescription drugs in the United States relative to comparison countries and found that U.S. prices for drugs in 2018 averaged about 250% of those in the 32 Organization for Economic Co-operation and Development (OECD) comparison countries.⁹ Prices for brand name drugs have also consistently risen faster than the rate of inflation.¹⁰ While private insurers and government programs finance the largest share of payment for drugs, high drug costs are ultimately passed on to patients through higher premiums and cost sharing.¹¹

America's high drug prices result in access and health equity challenges.¹² In the past year, three in ten American adults reported not taking their medications as prescribed due to cost.¹³ Individuals who have low incomes, experience serious health conditions, or take four or more medications are more likely to have difficulty affording their prescription drugs, as are Black and Hispanic adults.¹⁴ The underlying causes of high prescription drug costs are well documented and include market failures, such as lack of price and data transparency and misaligned financial incentives for providers and others in the supply chain.¹⁵ These causes are interrelated, often reinforcing one another.

In the past year, three in ten American adults reported not taking their medications as prescribed due to cost.

Prescription Drug Provisions in the Inflation Reduction Act of 2022

The Inflation Reduction Act of 2022 (IRA) (P.L. 117-169) introduced sweeping changes to help address rising drug prices and out-of-pocket costs in Medicare. Under the law, beginning in October 2022, drug manufacturers are required to pay rebates when they raise their prices for many Part D drugs faster than the rate of inflation. In addition, the law temporarily increases the payment for Medicare Part B qualifying biosimilars—a policy aimed at increasing access to biosimilars and lowering drug costs through increased competition. Beginning in 2023, inflation rebates will apply to certain Medicare Part B drugs, and monthly beneficiary cost-sharing for insulin will be limited to \$35 per month's supply per prescription. Also, beginning in 2023, adult vaccines that are covered under Medicare Part D and recommended by the Advisory Committee on Immunization Practices (ACIP) will be covered at no cost for Medicare beneficiaries. Additionally, under the IRA, most beneficiaries enrolled in Medicaid and the Children's Health Insurance Program (CHIP) will have coverage of approved, ACIP-recommended adult vaccinations, without cost-sharing, starting in October 2023.

The IRA also expands eligibility for full benefits under the Medicare Part D Low-Income Subsidy (LIS) Program and restructures the Medicare Part D benefit with \$0 beneficiary cost-sharing during the catastrophic phase of the benefit, beginning in 2024. The law places an annual out-of-pocket cap of \$2,000 (indexed annually for inflation) on Medicare Part D and allows for beneficiaries' Part D cost-sharing expenses to be "smoothed" over the course of the year, beginning in 2025. Finally, the law requires the Secretary of Health and Human Services (HHS) to negotiate the prices of certain high-spend Medicare Part D and Part B drugs with manufacturers. The IRA's implementation timeline ensures that the impact on Medicare beneficiaries will be swift, particularly for those who rely on insulin or need vaccinations.¹⁶ The Congressional Budget Office (CBO) forecasts that the drug pricing provisions in the IRA will reduce the federal deficit by \$237 billion over the next ten years.¹⁷

Executive Order 14087: “Lowering Prescription Drug Costs for Americans”

While the scope of the IRA in lowering prescription drug costs is expansive, opportunities remain. On October 14, 2022, President Biden issued Executive Order (EO) 14087, “Lowering Prescription Drug Costs for Americans,” to further address prescription drug affordability and access through the administrative authorities of CMS’s Innovation Center.¹⁸ The EO directs the Secretary of HHS to “consider whether to select for testing by the Innovation Center new health care payment and delivery models that would lower drug costs and promote access to innovative drug therapies for beneficiaries enrolled in the Medicare and Medicaid programs, including models that may lead to lower cost-sharing for commonly used drugs and support value-based payment that promotes high quality care.”

Role of the CMS Innovation Center

The CMS Innovation Center was established by Section 3021 of the Patient Protection and Affordable Care Act (P.L. 111-148) to test innovative payment and service delivery models to preserve or enhance the quality of care for beneficiaries in Medicare, Medicaid, and CHIP, while reducing program expenditures. The Secretary of HHS is authorized to expand the scope and duration of successful models, through rulemaking, that meet specific criteria.¹⁹

Since its inception in 2010, the Innovation Center has tested models impacting prescription drug utilization, costs, affordability, and quality. For example, the Part D Senior Savings (PDSS) Model, which runs from 2021 to 2023, caps the out-of-pocket cost of certain insulin drugs at \$35 per month, and has reached over 17 million Medicare beneficiaries, 800,000 of whom used insulin in 2022 (as mentioned above, the IRA provides a similar benefit to all Part D beneficiaries nationwide starting in 2023).²⁰ The Medicare Advantage Value-Based Insurance Design (VBID) Model allows participating Medicare Advantage organizations to reduce or eliminate cost-sharing for Part D benefits covered by participating Medicare Advantage Prescription Drug Plans (MAPDs) for targeted populations, such as those who qualify for the LIS, or are dually eligible beneficiaries and reside in certain areas. For 2023, over six million beneficiaries are projected to be offered \$0 cost-sharing for all Part D drugs through the VBID Model.²¹ In addition, the Oncology Care Model (OCM) motivated clinicians to focus on supportive care therapies and high-value prescribing, using a value-based approach, which led to a shift to lower-cost supportive care drugs.²² The independent evaluation of OCM also showed that as OCM practices prescribed more biosimilars, drug spending and beneficiary costs decreased.

In 2021, the Innovation Center launched a refresh of its strategy, laying out a vision for the next decade. The strategy refresh focuses the Center’s efforts on five strategic objectives: driving accountable care, advancing health equity, supporting innovation, addressing affordability, and partnering to achieve system transformation. In its one-year status report on implementation of the strategy refresh, the Innovation Center identified two affordability goals and calculated baselines as well as targets for the years 2025 and 2030. The goals are: reducing the percent of Innovation Center model beneficiaries who, in the last 12 months, indicate that they (1) delayed medical care due to cost and (2) “often” or “sometimes” delayed filling prescription drugs due to cost.²³

Approach for Developing Models

Stakeholder Input and Ideation

In developing model options that could meet the criteria for selection in response to EO 14087, the Innovation Center solicited input and ideas from a variety of sources. Activities included reviewing policy reports, prescription drug affordability studies, and previous model concepts from internal and external stakeholders.

The Innovation Center then solicited input from experts within CMS, other federal agencies, and from over 40 external stakeholders, including but not limited to beneficiary and caregiver advocacy groups; trade associations representing manufacturers, payers and PBMs; independent consultants; academic research institutions; hospital systems and provider groups; and data vendors. Many critical insights and valuable perspectives from these experts were helpful in further developing the concepts that have been selected by the Secretary and are presented in this report.

Criteria for Model Development and Prioritization

Consistent with the criteria for model selection enumerated in the EO and the Innovation Center's strategic priorities, three specific criteria were used to develop and prioritize model options:

- 1. Affordability:** Does the model have the potential to improve affordability of prescription drugs by either lowering overall drug prices or directly lowering out-of-pocket drug costs for Medicare and/or Medicaid beneficiaries?
- 2. Accessibility:** Does the model promote access to innovative drug therapies and high-value care, thereby enhancing the quality of care, beneficiary experience, and outcomes, while advancing health equity?²⁴
- 3. Feasibility of Implementation:** Does the model align with the goals of the IRA, and is it consistent with operational and regulatory limitations? For example, would the model be consistent with implementing the IRA without delay or interference and would the model have the potential to scale beyond the test's initial scope and duration?

Model development and priorities were established within the framework of the Innovation Center's goal of preserving or enhancing quality, while decreasing or maintaining costs, and requiring evidence that the model addresses a defined population for which there are deficits in care leading to poor clinical outcomes or potentially avoidable expenditures.²⁵

The Secretary's Selected Models

The Secretary has selected three models for testing by the Innovation Center. These models address themes outlined in the EO and meet the selection criteria of affordability, accessibility, and feasibility of implementation. These models will test important policy questions and may inform policymakers' decision-making when developing future legislation or regulatory guidance. The Innovation Center may also consider testing these concepts as demonstrations, if appropriate. The models are:

1. The Medicare High-Value Drug List Model
2. The Cell & Gene Therapy Access Model
3. The Accelerating Clinical Evidence Model

The design, background, rationale, evaluation, and timeline for each of these models are described in the sections to follow.

1) Affordable Drugs Made Simple – The Medicare High-Value Drug List Model

The Secretary has selected for testing by the Innovation Center a Part D model allowing Part D Sponsors to offer a Medicare-defined standard set of approximately 150 high-value generic drugs with a maximum co-payment of \$2 for a month's supply, applying across all phases of Part D coverage up to the out-of-pocket limit. The included drugs would target common chronic conditions among Medicare beneficiaries, such as hyperlipidemia and hypertension, and would not be subject to step therapy, prior authorization, quantity limits, or pharmacy network restrictions.²⁶ Part D Sponsors could offer this benefit in basic and enhanced drug benefit types.²⁷ CMS will explore flexibilities to encourage plans to participate in this model.



Population: Part D beneficiaries enrolled in Medicare Advantage Prescription Drug (MA-PD) plans or standalone prescription drug plans (PDPs)

Model Participants: Part D Sponsors.

Test Question: Does providing access to a standard list of high-value generic medications, at stable, predictable co-payments, increase beneficiary adherence to chronic care medications, improve clinical outcomes, and reduce health care costs?

Background

Affordability and limited price transparency are two of the primary reasons beneficiaries fail to take prescribed medications.^{28,29} While most Part D plans include a nominal co-payment generic formulary tier, the offering is not standard, the specific drugs vary by plan, and a deductible often applies.³⁰ The addition of preferred versus non-preferred tiering, coinsurance versus co-payments, step therapy requirements, and quantity limits layers on complexity. Ultimately, beneficiaries can rarely be certain of the cost of a drug until they are at the pharmacy counter.³¹

Providers face similar uncertainty in assessing beneficiaries' cost for the drugs they prescribe, finding brand drugs on generic tiers, similar strengths with different quantity limits, and large differences in price based on the type of packaging or dosage form.³² The traditional thinking that generics are more affordable and accessible does not consistently hold true, and for some beneficiaries costs can be higher with the generic.³³ While providers report considering costs when prescribing, they struggle to reliably identify the most cost-effective option for their patients.³⁴

In 2019, more than five million Medicare beneficiaries reported affordability problems with prescriptions, and 3.7 million beneficiaries reported not getting their needed medications due to cost.³⁵ Black and Hispanic beneficiaries reported similar difficulties at even greater rates, 1.5 to 2 times higher than White beneficiaries.³⁶

Designed to improve access, address affordability, and advance health equity and outcomes, the Secretary has selected this Model for the Innovation Center to test providing Medicare beneficiaries enrolled in Part D consistent access to a standardized list of high-value and low-cost generics.

Rationale

1) Offers a New Tool for Prescribers and Patients

The Medicare High-Value Drug List Model attempts to test a solution to two interrelated issues that may increase program expenditures and impede the quality of care for Medicare beneficiaries: 1) limited beneficiary cost transparency; and 2) difficulty identifying the optimal drug when multiple options may be available. A standardized list with consistent cost-sharing would allow providers to easily identify and prescribe appropriate medications without the worry of high prices for their patient or the stress of an unexpected prior authorization, step therapy, or quantity limit.

For beneficiaries, the list would offer consistent, predictable co-payments for high-value medications, and for providers, limit the stress of unexpected access hurdles. Helping beneficiaries access affordable, stable, predictably priced generic medications may improve their adherence and, in turn, their health outcomes, which could lead to reduced medical costs.^{37,38} The Medicare High-Value Drug List offered in the Model could become a tool that providers and beneficiaries jointly use for navigating formularies and enabling higher quality, lower cost care.

A standardized “drug list” is already well understood by consumers and providers.³⁹ Many large retail pharmacy chains offer a defined list of prescription medications at low, fixed prices.⁴⁰ Medicare does not have this same type of standardization, and while many Part D Sponsors offer lower cost-sharing for generics, there is variation in coverage among plans. This model would build on the concept used by large retail chains but be tailored for Part D beneficiaries and plans.

2) Builds on IRA Affordability Provisions

Starting in 2025, the IRA limits annual Part D out-of-pocket costs to \$2,000 (indexed to inflation in subsequent years) and requires Part D Sponsors to offer a maximum monthly cap option to their plan enrollees under which out-of-pocket costs are to be paid in monthly amounts spread over the year, often referred to as “smoothing”.⁴¹ These changes provide financial security to beneficiaries who develop conditions that require the use of high-cost brand and specialty drugs.

For these beneficiaries and their providers, the Medicare High-Value Drug List could be an impactful offering that provides easy to understand, predictable co-payments for high-value medications.

We expect the Medicare High-Value Drug List Model to complement these IRA provisions and be relevant to all Part D beneficiaries, especially those with chronic conditions (e.g., hypertension, hyperlipidemia). For these beneficiaries and their providers, the Medicare High-Value Drug List could be an impactful offering that provides easy to understand, predictable co-payments for high-value medications.

3) Builds off the Part D Senior Savings (PDSS) Model

CMS’s experience testing voluntary co-payment limits for insulins informed this Model option. The PDSS Model, which has a performance period of Contract Years 2021 through 2023, allows plans to set a \$35 (or less) co-payment for select insulins across all non-catastrophic phases of the benefit. Based on feedback from stakeholders and model operation considerations, the Secretary believes that the simplicity and consistency of the Model allowed Part D Sponsors to easily communicate the design to beneficiaries, thereby contributing to the Model’s significant uptake.⁴² A formal evaluation of the longer-term clinical impact is pending, but initial data suggests the Model may be leading to an improvement in adherence to insulins.⁴³

Evaluation

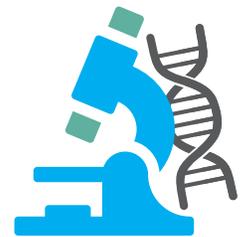
CMS's leading indicators for monitoring and evaluation of the Medicare High-Value Drug List Model could focus on impacts on program expenditures and quality of care. Regarding program expenditure metrics, these could include gross drug costs and certain medical costs (e.g., hospitalizations, emergency room visits). Regarding quality of care metrics, these could include beneficiary focused metrics, such as beneficiary satisfaction, generic drug utilization, out-of-pocket spending, adherence to chronic care medications, and other outcomes or clinical measures (to the extent they can be ascertained by CMS and plans).

Implementation & Timeline

CMS could explore leveraging existing systems, which would allow for a streamlined implementation. The Secretary directs CMS to request input from stakeholders (e.g., beneficiaries and their advocates, Part D Sponsors, manufacturers and providers) and announce the Model specifications in advance of a Model start date, as soon as operationally feasible.

2) The Cell & Gene Therapy Access Model

The Secretary has selected for testing by the Innovation Center a Medicaid-focused model, the Cell & Gene Therapy (CGT) Access Model. The CGT Access Model would establish a partnership among CMS, manufacturers and state Medicaid agencies, and would test a new approach for administering outcomes-based agreements (OBAs) to help Medicaid beneficiaries gain access to potentially life changing, high-cost specialty drugs. In lieu of state Medicaid agencies pursuing manufacturer agreements individually, state Medicaid agencies would have the option of assigning CMS to structure and coordinate multi-state OBAs with participating manufacturers. CMS would also take on the responsibility of implementing, monitoring, reconciling, and evaluating the financial and clinical outcomes outlined in the OBAs. This Model would target CGTs for illnesses like sickle cell disease and cancer.



Beneficiary Population: Medicaid beneficiaries.

Model Participants: Voluntary participation from state Medicaid agencies and selected manufacturers.

Test Question: Does a CMS-led approach for administering OBAs for CGTs improve beneficiary access and outcomes and reduce health care costs?

Background

CGTs are a subset of specialty drugs reflecting a rapidly expanding class of treatments with the potential to address certain severe diseases and disorders, such as cancer, as well as genetic and infectious diseases. As described by the Food and Drug Administration, “cellular therapy products include cellular immunotherapies, cancer vaccines, and other types of both autologous and allogeneic cells for certain therapeutic indications, including hematopoietic stem cells and adult and embryonic stem cells. Human gene therapy seeks to modify or manipulate the expression of a gene or to alter the biological properties of living cells for therapeutic use.”⁴⁴ These novel approaches have the

potential to treat, or even cure, previously intractable diseases such as sickle cell anemia, beta thalassemia, or even type 1 diabetes, which disproportionately impact underserved communities.^{45,46} There are currently 26 approved CGTs, but momentum and investment in this class is building.⁴⁷ Forecasts indicate up to 21 cell-based and 31 gene-based therapies could be launched in 2024.⁴⁸ Over the next 10 years, the National Bureau of Economic Research (NBER) predicts over one million Americans will have a condition that may be treated through the use of a CGT, with spending to reach \$25 billion annually.⁴⁹

Though treatment with CGTs can have the 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 the promise of this new class of specialty drugs.^{50,51} The high upfront cost for these therapies (often exceeding \$500,000 per course of treatment) poses a challenge to beneficiaries and payers, including state Medicaid agencies.⁵² The combination of rare indications and limited utilization and outcomes data, to date, makes underwriting and assessing value of the therapies difficult. In addition, beneficiary enrollment changes among payers make any single payer hesitant to pay the high, one-time cost for a CGT when another payer may reap the future benefit.⁵³ As a temporary solution, payers, including state Medicaid agencies, are increasing cost-sharing, instituting stringent utilization management programs, and/or purchasing reinsurance, as legally permissible and generally applicable.⁵⁴ These actions may impede access to CGTs for beneficiaries, particularly for those from underserved communities, who would benefit from potentially curative treatments early in life or in the course of a disease.

In response, a national approach for supporting OBAs related to CGTs could offer: better access to treatment for beneficiaries with rare and severe diseases, stability to payers, and payment certainty to providers who are hesitant to acquire and deliver these high-cost treatments.⁵⁵ The use of OBAs could enable manufacturers to be compensated based on the clinical impact of CGTs, therefore increasing accountability of the manufacturer to deliver treatments that improve quality at a more sustainable cost.

The Medicaid program could offer a compelling place to start, improving beneficiary access and outcomes, and reducing health care costs related to CGTs. State Medicaid agencies have voiced concerns about paying for high-cost CGTs in the absence of longer-term clinical effectiveness data and are, in some cases, establishing stringent utilization management requirements, which may limit access for beneficiaries.⁵⁶ State Medicaid agencies can partially address CGT spending by directly negotiating with manufacturers of CGTs to establish supplemental rebates that manufacturers pay to states (in addition to those rebates required under federal law), in some cases also incorporating a value-based component. However, as the number of approved CGTs increases, state Medicaid agencies may be limited in their capacity to execute these complex arrangements without federal support. Since Medicaid is a joint state and federal program, with generally at least 50% of funding coming from the federal budget through federal matching, financing CGTs is both a state and federal fiscal issue.⁵⁷

Rationale

1) Facilitates Negotiations between Medicaid and Manufacturers

Regulatory changes to the Medicaid Drug Rebate Program (MDRP) now allow manufacturers to report to CMS multiple best prices associated with a value-based purchasing (VBP) arrangement as long as such arrangements are offered to all states.^{58,59} While state Medicaid agencies may take advantage of these arrangements to reduce their spending on high-cost prescription drugs, as of November 2022 there are no options for state Medicaid agencies to take advantage of multiple best prices as manufacturers have not yet utilized the option to report a VBP.⁶⁰ Additionally, state Medicaid agencies can negotiate VBP arrangements through CMS-authorized Supplemental Rebate Agreements (SRAs) by seeking CMS approval to do so through State Plan Amendments (SPAs). As of November 2022, 15 states have state plans that reflect these CMS-authorized SRAs, but only two agreements are specific to CGTs.⁶¹ Multi-state rebate

pools, administered by private entities, have also not yet established OBAs for CGTs. The complexity of data collection and evaluation and a desire to negotiate for more meaningful outcomes are likely deterrents to a larger number of OBAs for CGTs. Additional federal support (e.g., administrative funding and monitoring/evaluation support) may be necessary to obtain better, timelier terms from manufacturers, as well as to efficiently administer and evaluate OBAs.

The CGT Access Model would attempt to reduce the burden and standardize the process for state Medicaid agencies to establish and maintain OBAs for CGTs. In a budget neutral manner, the CGT Access Model would allow CMS, on behalf of states, to: 1) pool bargaining power to obtain discounted pricing; 2) condition the ultimate cost of the CGT on outcomes; and 3) shift the burden of administering complex OBAs from state Medicaid agencies to CMS during the model test. For manufacturers, participation in the CGT Access Model would simplify market access. Since the design of each state Medicaid prescription drug benefit program is unique, negotiating and signing a single multi-state agreement facilitated by CMS could simplify measurement of OBAs and give better revenue predictability. The opportunity to execute a CMS-facilitated multi-state OBA could be a strong incentive for CGT manufacturers to offer larger discounts tied to meaningful clinical outcomes.

2) Builds on Precedent from State Options to Negotiate with Drug Manufacturers

Although the economics and clinical needs of OBAs for each CGT will be unique, helpful precedents for CMS and state Medicaid agencies navigating payment arrangements, rebate agreements, and structuring an OBA include: (1) state experience in pooled purchasing arrangements; (2) the variety of state-level approaches to date; and (3) state drug transparency processes.⁶²

Building off of state-level initiatives, this federal model would be a multi-state test that could inform a more permanent framework for evaluating, financing, and delivering CGTs on a broader scale.

- 1) **State Experience in Pooled Purchasing Arrangements:** As of 2019, 46 states had preferred drug lists in place, of which 31 participated in one of three interstate pooling purchasing arrangements: the Sovereign States Drug Consortium (SSDC), TOP\$, and the National Medicaid Pooling Initiative (NMPI).^{63,64}
- 2) **Variety of State-Level Approaches to Date:** Through CMS-authorized supplemental rebate agreements with manufacturers, states have taken other routes to negotiate prices with manufacturers for both drugs that have therapeutic competitors and those drugs that do not. For example, Louisiana⁶⁵ and Washington⁶⁶ state Medicaid agencies signed manufacturer agreements in 2019 to finance hepatitis C drug costs using value-based arrangements, through a CMS-authorized SRA. These states chose a single manufacturer to supply an unlimited quantity of treatments for a fixed aggregate cost. The ‘subscription’ model fits the treatment and population profile: oral medications, over 10 years of clinical data since FDA approval, and a solution for a public health problem.
- 3) **State Drug Transparency Example:** Massachusetts, through state legislative actions, has established drug price transparency processes that may require manufacturers to provide a rationale as to how they determined their prices, especially for high-cost drugs with no therapeutic competitors. One goal of this effort has been to maximize pressure on these manufacturers to offer better rebates such that more patients can access the treatments, including through value-based purchasing agreements. The process is estimated to have saved close to \$200 million for the state since its inception in 2019.⁶⁷

Legislators, policy experts, economists, and manufacturers have suggested the need for the federal government to take a leadership and coordination role in expanding access to CGTs.^{68,69,70} Building off of these state-level initiatives, this federal model would be a multi-state test that could inform a more permanent framework for evaluating, financing, and delivering CGTs on a broader scale.

Evaluation

To monitor and evaluate the success of this Model, CMS could consider tracking metrics including changes in access to therapies over time, clinical and patient experience outcomes associated with those therapies, drug spending and utilization, out-of-pocket spending, total program expenditures, and drug discounts.

Implementation & Timeline

There are different approaches to consider in implementing the CGT Access Model via federal support. This would likely require CMS consultation with public health experts, health economists, clinicians, and manufacturers. CMS could explore a variety of options including (but not limited to):

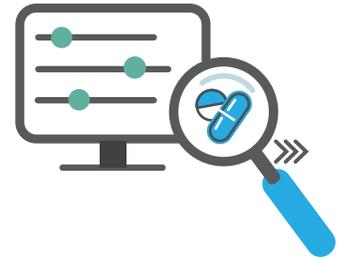
- Outcomes-Based Payments, with a portion of payment up front, and the remainder based on clinical milestones;
- Outcomes-Based Rebates, with payment up front and a rebate if a specific clinical outcome is not achieved; and
- Outcomes-Based Annuities, with fixed price payments spread over time if beneficiaries receiving treatment continue to achieve specific clinical outcomes.

Ultimately, CMS would tailor its approach based on the clinical evidence, pricing data, and utilization patterns observed for CGTs at the time of agreement.

The Secretary directs CMS to begin model development in 2023, consider announcing the model specifications in 2024-2025, and launch the model test as early as 2026. CMS should consider starting the CGT Access Model with a single therapeutic indication, such as sickle cell disease. If improvements in beneficiary access, clinical outcomes, and cost are documented, the Secretary would evaluate additional therapeutic indications.

3) Paying for Drugs that Work: The Accelerating Clinical Evidence Model

The Secretary has selected for testing by the Innovation Center the Accelerating Clinical Evidence Model. The 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. Working in consultation with FDA, CMS could consider various approaches to adjust payments to the provider for AAP drugs, seeking to balance incentives for developing novel treatments with potential harms of delayed confirmatory clinical trials. Any adjustments would be structured in a manner that attempts to avoid penalizing physicians or beneficiaries for choosing (or avoiding) an accelerated approval treatment. By incentivizing timely confirmatory trial completion, CMS could enable improved access to post-market safety and efficacy data.



Beneficiary Population: Medicare fee-for-service (FFS) beneficiaries.

Model Participants: Mandatory participation for applicable Medicare Part B fee-for-service providers.

Test Question: Do targeted adjustments on payments for AAP drugs accelerate confirmatory trial completion, provide timely information on the safety and effectiveness of AAP drugs on the market, facilitate earlier withdrawals of AAP drugs when appropriate, and reduce Medicare spending on drugs that do not have confirmed clinical benefit?

Background

The accelerated approval pathway was established by the FDA in 1992 to expedite access to drugs that fill an unmet medical need and offer improvements (relative to other available treatments) to patients with serious conditions, but would take longer to evaluate if full evidence of clinical outcomes was required.⁷¹ Congress added provisions to the FD&C Act related to accelerated approval first through the Food and Drug Administration Modernization Act (FDAMA) of 1997 (P.L. 105-115). The statutory provisions were revised by Congress in 2012 via the Food and Drug Administration Safety and Innovation Act (FDASIA) (P.L. 112-144). These provisions were again revised by Section 3210 of the Consolidated Appropriations Act, 2023 (P.L. 117-328), which, among other things, provided FDA new authorities to require confirmatory trials be ongoing at the time of accelerated approval or within a specified time period after the date of approval, and streamlined the process for withdrawal.

To receive an accelerated approval, a drug must address an unmet clinical need and meet the same safety and effectiveness standard as required for traditional approval, but can rely on surrogate or intermediate clinical endpoints that are reasonably likely to predict clinical benefit (e.g., tumor size, gene expression) instead of clinical endpoints that represent evidence of clinical benefit (e.g., survival, improvement in symptoms).⁷² Following accelerated approval by the FDA, manufacturers have been required to complete a confirmatory trial to verify the drug provides clinical benefit.

The AAP is likely attractive to manufacturers and patients with unmet medical needs as it allows a product or a new indication for an approved product to receive expedited approval based on surrogate (or intermediary clinical) endpoints. However, incomplete and delayed data from confirmatory trials may result in ongoing utilization of drugs that subsequently fail to confirm effectiveness, which is concerning for patients and payers.^{73,74,75}

The AAP has been criticized because manufacturers may fail to complete confirmatory trials by the date to which they committed at the time of accelerated approval. As of May 2022, 104 out of the 278 drug applications approved through AAP have incomplete confirmatory trials, 35 (34%) of which have at least one trial past their originally planned confirmatory trial completion date.⁷⁶ From 2018 to 2021, Medicare and Medicaid together spent an estimated \$18 billion on AAP therapies that were past their originally scheduled confirmatory trial completion date.⁷⁷ Given the increasing number of AAP approvals, some experts are concerned the number of past-due trials may continue to increase.⁷⁸

From 2018 to 2021, Medicare and Medicaid together spent an estimated \$18 billion on AAP therapies that were past their originally scheduled confirmatory trial completion date.

Although the FDA has authority to issue civil money penalties (CMPs) against sponsors for “failure to conduct” a confirmatory study required under accelerated approval and can withdraw an accelerated approval if a required confirmatory study is not conducted with due diligence or fails to verify clinical benefit, imposing CMPs and withdrawing approval both involve lengthy procedures. In addition, once a product is on the market and established

in clinical practice, physicians and patient groups may oppose the FDA’s recommendation for a withdrawal, even once there is a failure of a confirmatory trial.⁷⁹ The lack of confirmatory evidence creates a dilemma. These factors support the Secretary’s consideration of changes to payment that might encourage evidence development via timely completion of confirmatory trials.

The AAP by design must ultimately balance the *benefit* of faster drug approvals for patients with serious and life-threatening conditions facing unmet medical needs, with the *risk* of bringing therapies to market for which clinical benefit ultimately may not be confirmed. The Accelerating Clinical Evidence Model would test whether targeted Medicare payment adjustments incentivize timely manufacturer trial completion, facilitating earlier availability of clinical evidence. The completion of confirmatory trials on a timelier basis could provide earlier confirmation of benefit for AAP drugs that succeed in their trials or support earlier withdrawal of AAP drugs that are unable to confirm clinical benefit, contributing to improved clinical care for Medicare beneficiaries and a reduction in costs for CMS.

Rationale

1) Addresses Coverage and Payer Concerns

Private and public payers are beginning to evaluate the effectiveness of AAP drugs and consider methods for limiting coverage. State Medicaid agencies, which generally must cover FDA-approved drugs under the Medicaid Drug Rebate Program (MDRP), with limited exceptions, in order to receive federal matching funds and to receive statutory rebates, have requested CMS waivers to exclude coverage of drugs where confirmatory trials are delayed and where the state considers the available clinical efficacy data to be limited.⁸⁰ CMS has also narrowed Medicare coverage for certain AAP drugs through its Coverage with Evidence Development (CED) process (e.g., aducanumab, trade name Aduhelm), providing coverage only to beneficiaries enrolled in qualifying clinical trials.⁸¹

CMS’s approach for designing the Accelerating Clinical Evidence Model would be informed by the recommendations from the Medicare Payment and Advisory Commission (MedPAC), the Medicaid and CHIP Payment and Access Commission (MACPAC), and others who are studying the AAP process, as well as in consultation with FDA, and attempt to find the appropriate balance between the more rapid availability of new medications that show promise and the

longer time needed for those medications to be evaluated through traditional pathways.^{82,83} Although this Model would initially address Medicare Part B payments for AAP drugs, the manufacturer incentive to complete confirmatory trials could ultimately benefit other payers offering coverage of accelerated approval drugs as well.

2) Addresses Misaligned Manufacturer Incentives

Although clinical trials can fall behind schedule for many reasons, for AAP confirmatory trials, misaligned manufacturer incentives may also contribute. Because the AAP offers a faster path to revenue, manufacturers may preferentially seek accelerated approval (provided a suitable surrogate or intermediate clinical endpoint is available). Once a drug receives accelerated approval, a product is on the market, potentially priced at or above other drugs used to treat the condition. While the FDA does have statutory authority to withdraw an approval if the sponsor fails to conduct a required trial with the required due diligence, this is a difficult path to take as there may be no evidence to say the drug is ineffective and the available evidence is that which the FDA relied upon for approval. Manufacturers could also seek to extend their trial completion to delay the possibility of obtaining a negative result that could result in an FDA or self-initiated market withdrawal. The Consolidated Appropriations Act, 2023, among other things, provided FDA new authorities to require confirmatory trials be ongoing at the time of accelerated approval or within a specified time period after the date of approval, and streamlined the process for withdrawal. However, instances of delayed confirmatory trials and incomplete clinical data may result in beneficiaries taking ineffective drugs, resulting in excess cost and delaying a patient's progress towards alternative therapies or treatments, if available.

Evaluation

To monitor and evaluate this Model, CMS could consider metrics to assess quality and financial impacts. These metrics may include an assessment of the rate of study completion within the proposed study duration of confirmatory trials, total costs to the Medicare program, and beneficiary-focused metrics around access, safety, health outcomes, and quality of care. Price trends of accelerated approval drugs and Part B drug spending could also be considered.

Implementation & Timeline

Although drugs with multiple indications make up a large portion of accelerated approvals, CMS Part B fee-for-service drug payments are not tied to specific indications, making a variable, indication-based pricing scheme difficult to implement. To overcome the limitation, the Secretary will consider the need to treat certain AAP drugs differently (e.g., drugs with multiple indications). The Secretary will also explore options to appropriately address cases where a drug may have multiple confirmatory trials in progress for multiple indications.

Given the recent enactment of the Consolidated Appropriations Act, 2023, the Secretary directs CMS to begin consultation with FDA to explore the Accelerating Clinical Evidence Model in 2023, and if determined appropriate, continue development thereafter with a targeted launch as soon as feasible. To promote stakeholder engagement, the Secretary may direct CMS to publish an Advance Notice of Proposed Rulemaking after Model development and before engaging in rulemaking.

Additional Areas of Research

The Secretary has directed the Innovation Center to continue to evaluate potential models in the areas below.

1) Accelerating Biosimilar Adoption

The biosimilar approval pathway was enacted in the Biologics Price Competition and Innovation Act of 2009 (BPCI Act).⁸⁴ In the 12 years since, 40 biosimilar products have received FDA approval, and only 21 products have commercially launched.⁸⁵ As of 2021, only half of the 11 reference products with biosimilar competition have given up over 50% of their market share to biosimilars, and biosimilar acceptance in the U.S. currently lags the OECD average.⁸⁶ Forecasts from MedPAC and RAND estimate that price reductions due to competition from biosimilars could save the federal government more than \$9 billion per year over the next decade.^{87,88}

Research from NORC finds that over 70% of patients would consider a biosimilar if their physician recommended it and over 50% of physicians would prescribe a biosimilar if the financial incentives were comparable to the reference product.⁸⁹ Medicare Part B drug payment methodology for biological products (other than biosimilars) provides a 6% add-on of the Average Sales Price (ASP) of the biological. For biosimilars, Medicare Part B provides an add-on of 6% (or 8% in the case of a qualifying biosimilar) of the ASP of the biosimilar's reference biological product. The percentage-based add-on for Part B drugs and biologics, for which Part B providers 'buy-and-bill', may disincentivize physicians or other Part B providers from choosing lower cost options, often the biosimilar. In addition, beneficiaries may only see a partial reduction in their out-of-pocket costs when choosing a biosimilar due to factors such as coinsurance benefit design and supplemental Medigap insurance. Although the IRA encourages use of biosimilars by temporarily increasing the Part B add-on payment for certain biosimilars (those with an ASP less than the reference product) from 6% to 8% of the ASP of the reference biological product, early feedback from some provider groups and hospital systems have suggested that additional actions could be taken to encourage such adoption.⁹⁰

To address this opportunity, the Secretary directs the Innovation Center to continue investigating options to improve biosimilar adoption. Areas of consideration include: 1) aligning biosimilar cost-sharing and payment incentives for providers and beneficiaries; 2) creating shared savings arrangements and/or payment bundles for therapeutic classes; and 3) adjusting payment methods to increase competition and promote investment in biosimilar development.

2) Data Access Changes to Support Price Transparency

The Secretary directs the Innovation Center to continue exploring opportunities to encourage price transparency for prescription drugs, building on efforts made in the CMS Interoperability and Patient Access Final Rule (CMS-9115-F, 85 FR 25510) and Transparency in Coverage Final Rule (CMS-9915-F, 85 FR 72158). These rules, along with other efforts by CMS, are intended to strengthen patient access to health information, reduce administrative burden for clinicians so they can focus on direct care, and support interoperability across the health care landscape. Through its data transparency and interoperability efforts, CMS has made substantial progress to help ensure beneficiaries have access to the data they need to make informed decisions about their health care. To build on these efforts and improve transparency and interoperability of prescription drug data, the Secretary directs the Innovation Center to explore models or other activities (e.g., challenge.gov proposals, algorithm improvements, personalized recommendations, etc.) that would allow beneficiaries and providers to use prescription drug data to consider alternatives, assess utilization management review requirements, compare price by fulfillment locations, and shop plan options.

3) Cell and Gene Therapy Access in Medicare Fee-for-Service

The Secretary directs the Innovation Center to consider potential Medicare fee-for-service options to support CGT access and affordability, to complement the Medicaid-focused Cell and Gene Therapy Access Model discussed earlier in this report. Opportunities may exist to test alternative payment approaches, such as bundled payments, that would replace traditional fee-for-service billing during extended care episodes associated with CGT. Payment could be structured to incentivize high-quality care in the most appropriate setting for a patient through site neutrality, enhanced patient-centered care through a quality adjustment, and better care coordination by incentivizing providers to optimize outcomes and manage negative side effects that result in additional acute care utilization. This payment approach could potentially serve as a model for other payers as well as have applicability to other CGT products in the future. In addition, a bundled payment approach may increase manufacturer competition, which could lead to reduced manufacturer prices. CMS would study the impact of this type of program on its ability to reduce beneficiary costs, improve beneficiary access and quality of care, and reduce overall Medicare spending.

Conclusion & Next Steps

HHS is committed to helping build a health system that achieves equitable outcomes through high-quality, affordable, and person-centered care. The EO builds on the IRA and directs the Secretary of HHS to further address prescription drug affordability and access challenges for beneficiaries.

In this report, the Secretary has selected for testing by the Innovation Center three models to address beneficiary needs in Medicaid and Medicare Parts B and D. All three of these models would test hypotheses that could inform future policy to further increase the availability and affordability of prescription drugs for people in the United States. The Secretary has directed CMS to explore three additional research areas for potential model development.

As directed by the Secretary, the Innovation Center will take appropriate actions to further develop and test the three models enumerated in this report and will continue to engage with beneficiary and caregiver advocacy groups, manufacturers, health insurers, health care providers, academic institutions and researchers, and other interested parties for feedback and input.

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