Chapter 3:

Strengthening Evidence under Medicaid Drug Coverage

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Recommendations

3.1 Congress should amend § 1927(d)(1)(B) of the Social Security Act to allow states to exclude or otherwise restrict coverage of a covered outpatient drug based on coverage with evidence development requirements implemented under a Medicare national coverage determination.

3.2 Congress should amend Section 1903(m)(2)(A)(xiii) to require the managed care contract conform to the state's policy with respect to any exclusion or restriction of coverage of a covered outpatient drug based on coverage with evidence development requirements implemented under a Medicare national coverage determination.

Key Points

- Under Medicare Part A and Part B, the Centers for Medicare & Medicaid Services can link coverage of an item or service to participation in an approved clinical study or the collection of additional clinical data. This policy is referred to as coverage with evidence development (CED).
- Under the Medicaid Drug Rebate Program, state Medicaid programs generally must cover all of a participating manufacturer's drugs when prescribed for a medically accepted indication. Unlike Medicare Part A and Part B, Medicaid is not allowed to link drug coverage to the collection of additional evidence through a clinical trial or comparative study.
- States have expressed concerns about paying for prescription drugs that have yet to verify a clinical benefit. Allowing states to follow Medicare's CED requirement to link coverage of a particular drug to participation in a clinical trial or a comparative study would help ensure that evidence of the clinical benefit can be developed in a timely manner.
- Extending a Medicare CED policy to Medicaid would help provide additional evidence on the clinical benefits of a drug for populations prevalent in Medicaid and whether there are occurrences of adverse events that need to be monitored and managed.
- Requiring managed care organizations follow the state's decision on whether to implement a CED requirement would apply a consistent coverage policy across all beneficiaries, whether they receive services through fee for service or managed care.
- These recommendations would not automatically apply current or future Medicare CED requirements to the Medicaid program. States would have the option to follow Medicare requirements, but nothing in these recommendations would prohibit a state from providing broader coverage than allowed under Medicare.

CHAPTER 3: Strengthening Evidence under Medicaid Drug Coverage

Introduction

In fiscal year 2021, Medicaid spent approximately $80.6 billion on outpatient prescription drugs and collected $42.5 billion in rebates, bringing net drug spending to $38.1 billion. This net spending on outpatient prescription drugs accounted for about 5.3 percent of Medicaid benefit spending (MACPAC 2022a). The Centers for Medicare & Medicaid Services (CMS) Office of the Actuary projects Medicaid drug spending to increase between 5 and 6 percent annually over the next several years (OACT 2022).

While Medicaid drug spending is growing overall, it is increasingly being driven by high-cost specialty drugs. From 2010 to 2015, net spending on specialty drugs in Medicaid almost doubled, growing from $4.8 billion (25 percent of total net drug spending) to $9.9 billion (35 percent of total net drug spending) (CBO 2019). According to Magellan Rx Management, a leading Medicaid pharmacy benefit administrator, the net cost per claim for traditional drugs in fee-for-service Medicaid increased 5.8 percent from 2020 to 2021, while the net cost per claim for specialty drugs increased 13.0 percent over the same period (Magellan 2022). In 2021, high-cost specialty drugs accounted for less than 2 percent of drug utilization but more than half of Medicaid pharmacy spending (MACPAC 2022a, Magellan 2022).

States have expressed concern about paying high prices for drugs approved through the accelerated approval pathway (CMS 2022a, 2019a, 2017). These drugs have been approved by the U.S. Food and Drug Administration (FDA) on the basis of surrogate endpoints that are reasonably likely to predict a clinical benefit but are not a verified measure of a clinical benefit.[1] The FDA typically requires that manufacturers conduct confirmatory trials to verify the clinical benefit of a drug receiving accelerated approval, but these trials are often delayed beyond the scheduled completion date, and some trials can take more than 10 years to complete (Chen 2018, Naci et al. 2017).[2] The U.S. Department of Health and Human Services Office of the Inspector General estimated that Medicaid spent $3.6 billion between 2018 and 2021 on drugs approved through the accelerated pathway that had at least one confirmatory trial past its original planned completion date (OIG 2022). In its June 2021 report to Congress, the Commission raised these concerns about accelerated approval drugs and made recommendations to increase the Medicaid statutory rebates on these products until the manufacturer has demonstrated the clinical benefit and received traditional approval from the FDA. To date, Congress has not acted on these recommendations.

The approval of Aduhelm (aducanumab) for the treatment of Alzheimer's disease in June 2021 drew attention to the concerns over paying for a drug that has yet to verify a clinical benefit. The FDA's decision to grant accelerated approval of Aduhelm was considered controversial by many in the scientific, medical, and health policy communities after the almost unanimous recommendation against traditional approval from the FDA advisory committee based on its determination that there was insufficient evidence of a clinical benefit (Belluck 2021). Many stakeholders expressed concern with the price and potential cost to the health care system, particularly in light of the uncertain clinical benefit (Joseph and Cohrs 2021). Due to these concerns, CMS initiated a Medicare national coverage determination (NCD) to establish coverage parameters for monoclonal antibodies targeted against amyloid (antiamyloid monoclonal antibodies) for the treatment of Alzheimer's disease (e.g., Aduhelm) and in April 2022 decided to allow Medicare Part B coverage only under a coverage with evidence development (CED) policy that requires that the beneficiary participates in a clinical trial or other approved comparative study (CMS 2022b). Unlike Medicare Part A and Part B, state Medicaid programs are generally required by the Medicaid Drug Rebate Program (MDRP) to cover all of a participating manufacturer's drugs when prescribed for a medically accepted indication and are not allowed to link drug coverage to participation in a clinical trial or comparative study. Medicaid does not have to cover a drug for full-benefit dually eligible beneficiaries if it is excluded or limited by Medicare Part A or Part B, such as under an NCD (CMS 2022b).

This chapter presents the Commission's recommendations on allowing states to exclude or otherwise restrict coverage of a drug for Medicaid-only beneficiaries based on CED requirements included in a Medicare NCD. Specifically, the Commission recommends the following:

- Congress should amend § 1927(d)(1)(B) of the Social Security Act to allow states to exclude or otherwise restrict coverage of a covered outpatient drug based on coverage with evidence development requirements implemented under a Medicare national coverage determination.
- Congress should amend Section 1903(m)(2)(A)(xiii) to require the managed care contract conform to the state's policy with respect to any exclusion or restriction of coverage of a covered outpatient drug based on coverage with evidence development requirements implemented under a Medicare national coverage determination.

The recommendations would provide statutory authority for states, at their option, to link coverage of a particular drug to participation in a clinical trial or comparative study following CED requirements that have been implemented under a Medicare NCD. Allowing states to link coverage of a particular drug to the collection of additional clinical data would help ensure that evidence of the clinical benefit can be developed in a timely manner and provide additional information on the benefits and risks of treatment in the Medicaid population. The recommendations would also require Medicaid managed care organizations (MCOs) to follow the state's decision on whether to implement any CED requirements to ensure that coverage is consistent across all beneficiaries, whether they receive services through fee for service or managed care.

This chapter begins with an overview of drug coverage under Medicaid and Medicare. It provides background on the different coverage requirements under the MDRP and Medicare Part A and Part B. The chapter then presents the rationale for the Commission's recommendations for Congress to allow states to implement coverage criteria that follow CED requirements implemented under a Medicare NCD. The chapter concludes by outlining the Commission's future work on prescription drugs.

Medicaid Drug Coverage

The MDRP was created under the Omnibus Budget Reconciliation Act of 1990 (P.L. 101-508) with the purpose of ensuring that Medicaid pays a net price that is consistent with the lowest or best price that manufacturers charge other payers for the drug. Under the program, a drug manufacturer must enter into a Medicaid national drug rebate agreement with the Secretary of the U.S. Department of Health and Human Services (the Secretary) for states to receive federal funding for using the manufacturer's products (§ 1927(a)(1) of the Social Security Act (the Act)).[3] In exchange for the rebates, state Medicaid programs generally must cover all of a participating manufacturer's drugs when prescribed for a medically accepted indication, although the states may limit the use of some drugs through preferred drug lists (PDLs), prior authorization, and quantity limits.[4]

Under the MDRP, a drug meets the definition of a covered outpatient drug if its manufacturer has in place a rebate agreement with the Secretary and the drug has been approved by the FDA (§ 1927(k) of the Act). Although a state can use prior authorization, clinical criteria, or other utilization management tools to manage the use of a particular drug, the effect of these limitations "should not result in the denial of access to effective, clinically appropriate, and medically necessary treatments" (CMS 2015, p. 3).

States must follow a prescribed process to publish and implement formal coverage criteria. The statute requires that the PDL and other coverage criteria (e.g., prior authorization) must be developed by a committee consisting of physicians, pharmacists, and other appropriate individuals appointed by the governor of the state (§ 1927(d)(4)(A) of the Act). To fulfill this requirement, states typically use a pharmacy and therapeutics (P&T) committee to develop their PDLs and make recommendations on appropriate utilization protocols, such as prior authorization, for each drug.[5] The process of P&T committee deliberations varies from state to state. P&T committee meetings are typically open to the public for comment and testimony, and states may require public notice and the publication of the meeting agenda a few weeks in advance of the meeting.

The statutory requirement for Medicaid to cover essentially all FDA-approved drugs makes the program unique among payers by limiting states' ability to manage utilization and spending and to negotiate rebates with manufacturers compared with other payers. In general, plans sold on health insurance exchanges and Medicare Part D plans have minimum requirements for drug coverage, but they are allowed to exclude coverage for some drugs.[6] Likewise, self-insured plans, large group plans, and grandfathered health plans not subject to essential health benefit requirements can exclude coverage for some drugs.

Additionally, the coverage requirement under the MDRP means that a state is generally required to cover all of a participating manufacturer's products as soon as they have been approved by the FDA and enter the market.[7] In contrast, exchange and Medicare Part D plans are allowed a period of time after a new drug's release onto the market to evaluate it and make coverage decisions. Exchange plans are required to make a reasonable effort to review new drugs within 90 days of approval and make coverage determinations within 180 days (HHS 2015). Medicare Part D plans are similarly required to make a reasonable effort to review new drugs within 90 days and make coverage decisions within 180 days of a drug's release onto the market (CMS 2016a).[8]

This statutory requirement to cover new drugs upon market entry creates both operational and fiscal challenges for states.[9] A state must quickly determine under what circumstances coverage is supported by the FDA label. For novel drugs or first-in-class therapies, state officials and providers may not know in advance what uses will be supported by its label or if professional societies will release additional clinical guidelines regarding appropriate dosing, potential drug interactions, or clinical monitoring. Furthermore, new high-cost drugs (e.g., hepatitis C treatments) can be released at any time, but if they were unanticipated at the start of the fiscal year, they can exert fiscal pressures on annual state budgets. Last, states with managed care programs may need to make midyear contract (e.g., carve-out) or capitation rate changes (e.g., kick payment, rate adjustment) to ensure that plans are paid appropriately to cover the cost of the new drug.

Statutory rebates

Medicaid drug rebates are calculated based on average manufacturer price (AMP). AMP is defined as the average price paid to the manufacturer for the drug in the United States by wholesalers for drugs distributed to retail community pharmacies and by retail community pharmacies that purchase drugs directly from the manufacturer (§ 1927(k)(1) of the Act).[10]

The rebate formula for single-source and innovator multiple-source drugs (i.e., brand-name drugs) differs from the formula for non-innovator multiple-source drugs (i.e., generic drugs).[11] For purposes of simplicity, this chapter refers to single-source and innovator multiple-source drugs as brand drugs and refers to non-innovator multiple-source drugs as generic drugs or generics.

The rebate amount for covered outpatient drugs has two components: a basic rebate amount and an additional inflationary component. For most brand drugs, the basic rebate amount is equal to either 23.1 percent of AMP or AMP minus best price, whichever is greater.[12] Best price is statutorily defined as the lowest price available to any wholesaler, retailer, provider, or paying entity, excluding certain governmental payers (§ 1927(c)(1)(C) of the Act).[13] For generic drugs, the basic rebate amount is calculated as 13 percent of AMP with no best price provision.

An additional rebate based on an inflationary component is added to both brand and generic drugs if the increase in a drug's AMP exceeds the increase in the Consumer Price Index for All Urban Consumers (CPI-U) over time. The inflationary component is equal to the amount that the drug's current quarter AMP exceeds its baseline AMP trended to the current period by the CPI-U.[14] This inflationary rebate is designed to limit the increase in the net price of any drug to the rate of inflation.

Until January 1, 2024, the total rebate amount (the sum of the basic and inflationary components) cannot exceed 100 percent of AMP (§ 1927(c)(2)(D) of the Act). This rebate cap can limit the inflationary rebate if the price increases substantially over time and restricts the dollar amount of rebates that Medicaid can receive. The American Rescue Plan Act of 2021 (ARP, P.L. 117-2) removes this cap on Medicaid rebates beginning January 1, 2024 (§ 9816 of ARP).[15]

Supplemental rebates

A state can negotiate with each participating manufacturer to obtain supplemental rebates for one or more of that manufacturer's drugs, which manufacturers provide to ensure that their products are placed on the state's PDL. As of September 2022, almost all states (46 states and the District of Columbia) were receiving supplemental rebates in addition to mandated federal rebates (CMS 2022c).[16] Preferred drugs typically face fewer utilization management requirements (e.g., prior authorization) than therapeutically equivalent drugs that are not on the list, and this results in a shift in market share to the preferred drugs. Some states pursue supplemental rebate agreements on their own, while others have joined multistate coalitions for negotiation purposes (CMS 2022c).

Both the statutory rebates and supplemental rebates are treated as an offset to drug expenditures and are shared by the federal government and state based on each state's current federal medical assistance percentage (FMAP).

Physician-administered drugs

A physician-administered drug is an outpatient drug (other than a vaccine) that is typically administered by a health care provider in a physician's office or other clinical setting. For example, drugs that are infused or injected are typically physician-administered drugs. The provider bills the state Medicaid program for the drug using the appropriate national drug code (NDC) and billing code, such as a Healthcare Common Procedure Coding System code. States may maintain a list of (1) which drugs are considered physician-administered drugs and must be provided in a clinical setting and (2) which drugs are considered outpatient drugs and must be dispensed by a pharmacy.

Physician-administered drugs may also be eligible for the statutory rebate as long as the drug meets the definition of a covered outpatient drug. The statute contains language that limits the definition of covered outpatient drugs to exclude drugs that are billed as part of a bundled service within certain settings (e.g., drugs provided as part of a clinic visit or hospital stay) and are paid for as part of those services (§ 1927(k)(3) of the Act). This means that if a drug is provided as part of services received in one of the settings listed in the statute and is paid as part of those services (i.e., there is not direct payment for the drug), it is not subject to the MDRP rebate. However, if a state authorizes and makes a direct payment for the drug separately from the service in one of those settings, it can claim a rebate for that drug. This means that whether a physician-administered drug is considered an outpatient drug subject to a rebate can vary from state to state, depending on how a state pays for the drug (CMS 2016b).

For states to receive federal matching funds for physician-administered drugs, they are required to collect NDCs to claim rebates (§ 1927(a)(7) of the Act). NDCs identify the drug and manufacturer, which are needed to ensure that the correct manufacturer is billed for a rebate in the event that multiple manufacturers produce the same drug (as is the case for generic drugs). The statute requires states to collect NDCs for all brand drugs and for the 20 generic drugs that have the highest annual dollar value. In practice, however, states typically collect NDC information for all brand and generic physician-administered drugs.

Medicare Drug Coverage

Under Medicare, prescription drugs can be covered under either Part A, Part B, or Part D. Covered Part D drugs are defined as those that may be dispensed only upon a prescription, are defined as a covered outpatient drug under the MDRP, and are otherwise not already covered under Part A or Part B (§ 1860D-2(e) of the Act).[17] This means that the vast majority of prescription drugs—those typically obtained from a pharmacy—are covered under the Part D benefit. Drugs that are not covered under Part D can be covered under Part A or Part B depending on whether it is provided in an inpatient (Part A) or outpatient (Part B) setting.

Medicare Part A and Part B

Medicare Part B covers drugs that are not usually self-administered by the patient and are furnished as part of a physician's services in an outpatient setting (§ 1861(s)(2) of the Act). Drugs administered by infusion or injection in physician offices and hospital outpatient departments are the largest category of Part B drugs (MedPAC 2022a).[18]

Most Part B drugs are paid based on average sales price (ASP). ASP reflects the average price based on manufacturers' sales to most purchasers, net of manufacturer rebates, discounts, and price concessions, with exceptions such as those sales excluded from Medicaid best price (§ 1847A(c) of the Act). Medicare pays ASP plus 6 percent for most Part B drugs (§ 1847A(b) of the Act).[19] Medicare also makes a separate payment to the physician or hospital for administering the drug. The drug administration payment rates are determined under the physician fee schedule or outpatient prospective payment system, depending on the location of the service. For Part B drugs, beneficiaries generally face 20 percent cost sharing, except for preventive vaccines, which have no cost sharing (MedPAC 2022a).

Some drugs could also be covered under Part A if provided as part of an inpatient stay in a hospital or skilled nursing facility. Under Part A, the cost of the drug generally would be included in the payment made under the prospective payment system for inpatient hospitals or skilled nursing facilities.

Medicare Part A and Part B drugs are generally the same as those considered physician-administered drugs in the Medicaid program.

National coverage determination

Medicare Part A and Part B must cover services (unless specifically excluded in statute) included in a Medicare benefit category that are reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member (§ 1862(a)(1)(A) of the Act). This means that Medicare generally covers Part A and Part B drugs approved by the FDA for on-label indications or uses supported in CMS-approved compendia that are considered to be reasonable and necessary for the beneficiary (CMS 2021a, 2019b).[20]

CMS or Medicare administrative contractors can make explicit coverage determinations to evaluate the relevance, usefulness, and medical benefits of an item or service to Medicare beneficiaries (§ 1869(f)(1)(B), (2)(B) of the Act). This process involves a formal review of the medical and scientific evidence and includes a process for public comments. Medicare administrative contractors are responsible for making local coverage determinations, which determine coverage of items and services that apply only in the contractor's regional jurisdiction. The majority of explicit coverage policies are local coverage determinations (MedPAC 2022b). CMS can develop coverage determinations for items and services that apply nationwide through the NCD process. CMS can initiate an NCD internally, or one can be initiated at a stakeholder's request (CMS 2013). To date, fewer than 20 NCDs have been issued for drugs, and these coverage policies have largely aligned coverage with the FDA-approved label indications. In some cases, an NCD has clarified what off-label indications and types of providers Medicare will cover (MACPAC 2022b, MedPAC 2022b).

Coverage with evidence development

Under certain circumstances, CMS can link coverage of an item or service under an NCD to participation in an approved clinical study or the collection of additional clinical data (§ 1862(a)(1)(E) of the Act) (CMS 2022b). This policy is referred to as CED. CED is used when there are outstanding questions about the service's health benefit in the Medicare population, and it allows CMS to gather additional data that would further clarify the effect of these items and services on the health of Medicare beneficiaries. CMS currently applies CED to 21 items and services, but few apply to drug therapies. To date, CED has been used only three times on prescription drugs (MedPAC 2022b).[21]

The most recent example of a Medicare CED for prescription drugs was for the class of antiamyloid monoclonal antibodies for the treatment of Alzheimer's disease after the approval of Aduhelm. CMS limited coverage to participation in a clinical trial or other approved comparative study, depending on the pathway under which the FDA approved the drug (Box 3-1).

BOX 3-1. Accelerated Approval of Aduhelm

On June 7, 2021, the U.S. Food and Drug Administration (FDA) granted accelerated approval to Aduhelm (aducanumab) for the treatment of Alzheimer's disease (FDA 2021a). This approval was granted even though the FDA's Peripheral and Central Nervous System Drugs Advisory Committee recommended against traditional approval (FDA 2021b). The advisory committee decision, made during its November 6, 2020, meeting, was almost unanimous (10 votes against approval, 1 uncertain) against traditional approval, determining that there was insufficient evidence of a clinical benefit due to the conflicting results of the two clinical trials. Subsequent to the advisory committee meeting, further discussion within the FDA raised consideration of the accelerated approval pathway, which had not been presented as a consideration for the advisory committee at the November 2020 meeting (FDA 2021c).

This accelerated approval of Aduhelm has been considered controversial by many in the scientific, medical, and health policy communities. Opponents of the FDA approval highlighted three major concerns:

- **Lack of clinical evidence.** Based on the conflicting results from the two trials, the FDA advisory committee concluded that the totality of the evidence did not amount to the substantial evidence of efficacy required for traditional approval. Several members of the advisory committee commented that the results of studies 301 and 302 did not suggest a reduction of beta-amyloid is reasonably likely to predict a clinical benefit, citing an FDA statistical review that found no evidence that amyloid changes correlated with cognitive or functional changes (Alexander et al. 2021). Additionally, many researchers and clinicians have expressed concern with the potential risks, namely the presence of brain swelling, in light of the limited evidence on efficacy (Belluck 2021, Belluck et al. 2021).
- **Overly broad indication.** The FDA approval stated that the drug was indicated "for the treatment of Alzheimer's disease" with no limitations on severity or restrictions on how the disease should be diagnosed. This indication was broader than the populations included in the clinical trials, which focused on patients with mild cognitive impairment or mild dementia due to Alzheimer's disease (Alexander et al. 2021, Sachs 2021). In July 2021, Biogen (the manufacturer) responded to these concerns by updating the label indication to target patients with mild cognitive impairment or mild dementia stage of disease, the population in which treatment was initiated in clinical trials (Biogen 2021). Even so, many researchers still had concerns that the label did not specify that patients should have verification of elevated beta-amyloid or any other specific biomarker evidence (Alexander et al. 2021).
- **Lengthy timeline for confirmatory trial.** Under the terms of the accelerated approval, Biogen is required to perform a confirmatory trial to verify and describe the clinical benefit. In the approval letter, the FDA has given Biogen until February 2030 for a final report submission, approximately nine years after approval (FDA 2021a). Many stakeholders expressed concern with the lengthy amount of time to complete the clinical trial and noted that many drugs approved under the accelerated approval pathway have not demonstrated meaningful evidence of clinical effectiveness in the confirmatory trial (Alexander et al. 2021, Sachs 2021).

BOX 3-1. (continued)

In July 2021, at stakeholder request, the Centers for Medicare & Medicaid Services (CMS) announced that they would initiate a national coverage determination (NCD) analysis for Medicare, with a 30-day public comment period (CMS 2021b). CMS posted a proposed NCD decision in January 2022, and after another 30-day public comment period, finalized its NCD decision in April 2022 (CMS 2022b). CMS ultimately decided to cover Aduhelm under a coverage with evidence development (CED) policy to allow for the collection of additional clinical data. In addition, CMS made this NCD applicable to the entire class of antiamyloid monoclonal antibodies for the treatment of Alzheimer's disease. Aduhelm was the first approved drug in this class, and another drug, Leqembi (lecanemab), was granted accelerated approval on January 6, 2023 (FDA 2023).[22] At the time of the NCD decision, two other antiamyloid monoclonal antibodies (gantenerumab and donanemab) were undergoing phase three clinical trials (CMS 2022b).[23]

The NCD with CED requirement limited coverage to participation in a clinical trial or other approved comparative study, depending on the pathway under which the FDA approved the drug, as follows (CMS 2022b):

- Antiamyloid monoclonal antibodies approved under accelerated approval—that is, based on a change in a surrogate endpoint—may be covered in a randomized controlled trial conducted under an investigational new drug application.
- Antiamyloid monoclonal antibodies approved under traditional approval—that is, based on a direct measure of clinical benefit—may be covered in CMS-approved prospective comparative studies. The study may be collected in a registry.
- Coverage is also allowed when furnished according to the FDA-approved indication in National Institutes of Health-supported trials.

Medicare will not cover antiamyloid monoclonal antibodies for the treatment of Alzheimer's disease when provided outside of an FDA-approved randomized controlled trial, CMS-approved studies, or studies supported by the National Institutes of Health (CMS 2022b).

Coverage for dually eligible beneficiaries

Under mandatory Medicaid eligibility pathways, referred to as Medicare Savings Programs, beneficiaries dually eligible for Medicare and Medicaid may qualify for assistance with payment of Medicare premiums and, in some cases, Medicare cost sharing.[24] This means that for many dually eligible beneficiaries, Medicaid pays the beneficiary's cost for Part A or Part B drugs through coverage of the Part A or Part B premium and any applicable coinsurance. Under statute, Medicaid does not pay for Part D drugs, or any associated cost sharing, for full-benefit dually eligible individuals (§ 1935(d)(1) of the Act).

When CMS first announced it would proceed with an NCD for Aduhelm, many stakeholders expressed concern that a Medicare coverage decision could potentially shift costs to Medicaid. Because Medicaid must cover all FDA-approved drugs under the MDRP, the concern was that any exclusion of Aduhelm under Medicare Part B would shift that responsibility to Medicaid, as states would be liable to cover Aduhelm for full-benefit dually eligible beneficiaries and pay the full cost of treatment (NAMD 2021). In the April 2022 NCD decision memo on antiamyloid monoclonal antibodies for the treatment of Alzheimer's disease, CMS addressed these concerns by clarifying that when these drugs are not covered under the terms of the NCD, they are considered Part D drugs.

This ties back to the definition of Part D drugs as covered outpatient drugs under the MDRP that are otherwise not already covered under Part A or Part B (§ 1860D-2(e) of the Act). Because Medicaid does not pay for Part D drugs, this means that Medicaid is not a payor of last resort when Part A or Part B drugs are not covered under an NCD, and coverage would not shift from Medicare to Medicaid for full-benefit dually eligible beneficiaries (CMS 2022b).

Commission Recommendations

In this report, the Commission recommends a change to the MDRP to allow states to follow CED requirements that have been implemented under a Medicare NCD. Because full-benefit dually eligible beneficiaries would already be subject to CED requirements under Medicare, the recommendations would apply to Medicaid-only beneficiaries. Additionally, the Commission recommends that Medicaid MCOs be required to follow the state's decision on whether to implement any CED requirements. The recommendations were voted on as a package and should be taken together. The rationale and implications of these recommendations are described in the following sections.

Recommendation 3.1

Congress should amend § 1927(d)(1)(B) of the Social Security Act to allow states to exclude or otherwise restrict coverage of a covered outpatient drug based on coverage with evidence development requirements implemented under a Medicare national coverage determination.

Recommendation 3.2

Congress should amend Section 1903(m)(2)(A)(xiii) to require the managed care contract conform to the state's policy with respect to any exclusion or restriction of coverage of a covered outpatient drug based on coverage with evidence development requirements implemented under a Medicare national coverage determination.

Rationale

Under a Medicare NCD, CMS has gone through a formal process to review the clinical evidence and establish criteria for which coverage is considered reasonable and necessary. This process is similar to the P&T committee process that states use to make recommendations on appropriate utilization protocols, such as prior authorization. However, unlike Medicare Part A and Part B, Medicaid is not allowed to link drug coverage to the collection of additional evidence through a clinical trial or comparative study. In the case of Aduhelm, the National Association of Medicaid Directors asked CMS for the flexibility to apply the same coverage requirements as Medicare—that is, to cover it under a CED policy by limiting its use to persons enrolled in a clinical trial or other comparative study (NAMD 2021). It is conceivable that CMS could exercise its administrative authority and allow states to apply Medicare CED policies as prior authorization requirements for Medicaid, but a CMS policy that limits that application of the statutory MDRP may not stand up to legal challenge by a beneficiary or drug manufacturer. The recommendations would provide statutory authority for states, at their option, to implement CED requirements that have been established under a Medicare NCD.

In its prior work, the Commission has highlighted the need to verify a drug's clinical benefit in a timely manner (MACPAC 2021). State Medicaid officials have expressed concern about the requirement that Medicaid cover accelerated approval drugs that have been approved under surrogate endpoints (CMS 2022a, 2019a, 2017). In particular, they have shared concerns about paying for products that do not have a verified clinical benefit, and in some cases, may have adverse side effects in vulnerable populations. In addition, the length of time it has taken to complete some confirmatory trials means that states may be paying for treatments for several years before the benefit is verified. Allowing states to follow Medicare's requirement to link coverage of a particular drug to participation in a clinical trial or the collection of additional clinical data would help ensure that evidence of the clinical benefit can be developed in a timely manner.

CED has the potential to improve data collection on the outcomes for women, people of color, and low-income populations—groups that historically have been underrepresented in clinical trials (Duma et al.

2018, Unger et al. 2013). Extending the CED policy to Medicaid would help provide additional evidence on the clinical benefits of a drug in the Medicaid population, which may reflect a different mix of health status, demographic, and other socioeconomic characteristics than found in either the initial clinical trial or Medicare populations. For drugs that are more broadly applicable to both Medicare and Medicaid (e.g., oncology treatments), drug manufacturers or CMS may not set priorities for data collection in a manner that considers any differences in the composition of the Medicare and Medicaid populations. Clinical trials and studies can be designed to reflect the diversity of the patient population eligible for treatment beyond the Medicare population. For example, CMS included a requirement in its CED for antiamyloid monoclonal antibodies that the diversity of patients included in each study must be representative of the national population, including racial and ethnic groups (CMS 2022b). CED requirements in Medicaid can encourage drug manufacturers, CMS, and NIH to recruit a more diverse Medicaid population (e.g., individuals with disabilities) in clinical trials and prospective studies. Furthermore, a CED option could spur the negotiation of outcomes-based contracts. Better data collection on the Medicaid population could give states additional leverage to negotiate an outcomes-based contract that provides larger supplemental rebates if the drug does not provide the expected clinical outcomes.

It is important to note that these recommendations would not automatically apply current or future Medicare CED requirements to the Medicaid program. States would have the option to follow Medicare requirements, but nothing in these recommendations would prohibit a state from providing broader coverage than allowed under Medicare.

It is the Commission's belief that the authority to implement CED requirements should be given only to the state. Under the recommendations, the state would be required to have terms in its managed care contract that MCOs follow the state's decision as to whether to implement a CED requirement. This recommendation would apply a consistent coverage policy for any drug subject to CED requirements under a Medicare NCD across all beneficiaries, whether they receive services through fee for service or managed care. Aligning the policy would provide equal coverage across all plans and beneficiaries in the state. A consistent coverage policy would also reduce the administrative complexity for providers who would be required to collect and submit data. Furthermore, states should periodically review the clinical evidence as it is developed and revise their coverage policies to provide appropriate access to effective, clinically appropriate treatments.

Allowing states to follow a Medicare coverage decision is unlikely to affect many drugs. A CED requirement is applicable only to Medicare Part A or Part B drugs, so this option would be available only for drugs administered by a health care provider in an inpatient or outpatient setting. To date, CED has been used only three times on prescription drugs (MedPAC 2022b). Additionally, CMS officials have indicated that Medicare does not expect to implement CED requirements on prescription drugs frequently in the future (Wilkerson 2022). Furthermore, states would have the option to follow each Medicare coverage decision or not.

These recommendations would not address broader concerns states may have with the effect of high-cost drugs on state spending or the accelerated approval pathway. CMS is unlikely to evaluate or implement CED policies for drugs that are not significant to the Medicare population, and therefore, these recommendations likely would not address concerns for many drugs that are significant to Medicaid—for example, treatments for conditions prevalent in childhood, such as cystic fibrosis. Even so, drugs for which Medicare is the primary payer could still create substantial expenditures and corresponding budget pressure for states. MACPAC analysis of the prevalence of Alzheimer's disease in the non-dually eligible Medicaid population indicates that gross spending before rebates could reach as high as $1.7 to $3.3 billion a year, depending on the breadth of the label indication, uptake, and the price of the drugs (MACPAC 2022c). For context, that spending range would be similar to the annual gross spending on hepatitis C drugs.

Drug manufacturers and patient advocates have expressed concern over coverage restrictions that could limit patient access and the potential administrative burden of CED requirements (PhRMA 2022, ASGCT 2019, Twachtman 2019). CED requirements to enroll in a clinical trial might delay or restrict access and might result in beneficiaries not receiving a potentially beneficial treatment. Participation in a clinical trial can introduce additional burdens (e.g., travel) that may disproportionally affect

already underrepresented populations (e.g., low income, rural populations). CED requirements can also be carried out using prospective comparative studies or registries, which would provide broader coverage and are not as burdensome to patients as clinical trials. Drug manufacturers and patient advocates still have concerns that comparative studies or registries could delay access due to the effort it takes to set up the registry and report data.

Manufacturers and patient advocates have the opportunity to express their concerns during the Medicare NCD process. The Medicare NCD process includes formal periods for public comments after the announcement of an NCD consideration and after the publication of the proposed NCD. CMS has acknowledged the need to strike an appropriate balance of providing patient access with the collection of additional information on the clinical benefit and potential harms in the covered population (CMS 2022b). In past NCD decisions, CMS has demonstrated a willingness to alter its proposed criteria in response to concerns over beneficiary access. For example, in its 2019 NCD for chimeric antigen receptor T-cell (CAR–T) therapy, CMS proposed to apply CED that would require the beneficiary be enrolled in a prospective, national, audited registry. However, in response to public comments, it removed the CED requirement and ultimately finalized an NCD that covers CAR–T therapies when they are administered at health care facilities enrolled in the FDA risk evaluation and mitigation strategies and used for an FDA-approved indication or other use that is supported in one or more CMS-approved compendia (CMS 2019c). Upon approval of Leqembi, the second antiamyloid monoclonal antibody for the treatment of Alzheimer's disease, CMS indicated that it would engage with stakeholders and review data on the effectiveness of the drug to determine if it should reconsider the NCD on antiamyloid monoclonal antibodies for the treatment of Alzheimer's disease (CMS 2023).

Furthermore, states would be expected to make the decision to implement CED requirements using the P&T committee process they are required to use in establishing drug coverage criteria. P&T committee meetings are typically open to the public for comment and testimony, so stakeholders would have the opportunity to voice concerns before the state makes its coverage decision.

Implications

Federal spending. Allowing states to follow a Medicare CED requirement would likely reduce federal spending on those drugs. CED requirements would likely reduce utilization for those drugs, and thus, spending would also decrease. The Congressional Budget Office estimates that these recommendations would decrease federal spending by $0 to $5 billion over 10 years compared with the current law baseline.

States. For states that choose to follow a Medicare CED requirement, spending would decrease as use of drugs decreased. States would have another tool to gather evidence of a drug's clinical benefit in the Medicaid population. States could use CED requirements to negotiate outcomes-based contracts that provide larger supplemental rebates when a drug does not provide the desired outcome.

Enrollees. Generally, beneficiaries have been opposed to the CED requirements proposed under Medicare NCDs and are likely to oppose this policy to the extent it reduces access to particular drugs. A requirement to enroll in a clinical trial might restrict the number of people able to access the drug and delay access, which could result in some beneficiaries not receiving a potentially beneficial treatment. A Medicare CED can also require enrollment in a comparative study or registry, which would provide broader access than a clinical trial. A CED requirement could provide additional information about the benefits of treatment in specific subpopulations prevalent in Medicaid and whether there are occurrences of adverse events (e.g., brain swelling) that need to be monitored and managed.

Drug manufacturers. Manufacturers have been opposed to the CED requirements proposed under Medicare NCDs and oppose a policy that allows the extension of CED requirements to the Medicaid population. They argue that CED requirements can substantially restrict access to prescription drugs, and Medicaid coverage should not be restricted further than currently allowed under the MDRP. CED requirements could change manufacturer decisions about the pathway under which they seek FDA approval. For example, the CED requirements applied to the antiamyloid monoclonal antibodies for the treatment of Alzheimer's disease provide an incentive to seek traditional approval because the prospective study requirement allows for broader coverage than the randomized controlled trial requirement under

accelerated approval. Similarly, manufacturers would have an incentive to complete confirmatory trials and verify the clinical benefit in a more timely manner to obtain broader coverage.

Providers. Providers could face an administrative burden in the collection and reporting of data required under a Medicare CED policy. To the extent that these providers also serve Medicare beneficiaries, then they already need to have procedures in place to collect and report data. Including Medicaid beneficiaries in the data collection and reporting process may not be a substantial burden.

Next Steps

The Commission will continue to focus attention on prescription drugs, including physician-administered drugs. Many of the new drug therapies in the pipeline, such as cell and gene therapies, are likely to be administered by a professional in an office or facility setting. The different payment methodologies and administrative processes for physician-administered drugs may require different utilization management tools and payment models than those states currently use for other outpatient prescription drugs. We plan to continue monitoring the development of new proposals for alternative coverage or payment models and to reach out to stakeholders on the strengths and weaknesses of various policy options that could be used to address the challenges of high-cost drugs.

Endnotes

[1] The accelerated approval pathway allows the FDA to grant approval more quickly than the traditional approach because it allows approval based on whether the drug has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit (§ 506(c) of the Federal Food, Drug, and Cosmetic Act). A surrogate endpoint is a marker—a laboratory measurement, radiographic image, physical sign, or other measure—that is thought to predict clinical benefit but is not itself a measure of clinical benefit (FDA 2014).

[2] When the FDA approves a drug through the accelerated approval pathway, it generally requires manufacturers to conduct additional postmarketing studies (sometimes called phase IV studies) to verify that the drug achieves a clinical benefit (21 CFR 314.510, 21 CFR 601.41, FDA 2014).

[3] In addition to executing a Medicaid drug rebate agreement as a condition for Medicaid coverage of their products, drug manufacturers must enter into an agreement that meets the requirements of Section 340B of the Public Health Service Act (P.L. 102-585) and a master agreement with the Secretary of Veterans Affairs (§ 1927(a)(1) of the Act). Additionally, the manufacturer must enter into a Medicaid drug rebate agreement for payment to be made under Medicare Part B. A drug not covered under a rebate agreement may be eligible for federal Medicaid funding in limited circumstances if the state has determined that the drug is essential to the health of its beneficiaries.

[4] A medically accepted indication means any use for a covered outpatient drug that is approved under the Federal Food, Drug, and Cosmetic Act (P.L. 75-717) or that is supported by one or more citations included or approved for inclusion in one of the following three compendia: American Hospital Formulary Service Drug Information, United States Pharmacopeia-Drug Information, or the DRUGDEX Information System (§ 1927(k)(6) of the Act).

[5] The P&T committee examines the scientific literature (e.g., drug labeling, drug compendia, peer reviewed clinical literature, and professional association guidelines) for evidence that supports including a specific drug on the PDL based on the drug's safety, efficacy, and effectiveness relative to other drugs in its class. Price may also be considered once a drug's safety, efficacy, and effectiveness have been evaluated. For instance, inclusion on the PDL may be related to whether the state receives supplemental rebates from the drug's manufacturer. The P&T committee also makes recommendations on the appropriate utilization protocols, such as prior authorization or quantity limits for individual medications or for therapeutic categories.

[6] For Medicare Part D formularies, each drug category or class must include at least two drugs (regardless of the classification system used). Part D plan formularies must include all or substantially all drugs for the following six protected classes: immunosuppressants (for prophylaxis of organ transplant rejection), antidepressants, antipsychotics, anticonvulsants, antiretrovirals, and antineoplastics (CMS 2016a). Exchange plans must cover one drug in every United States Pharmacopeia category and class or the same number of drugs in each category and class as the state benchmark plan (45 CFR 156.122(a)(1)).

[7] A drug manufacturer must have a signed Medicaid drug rebate agreement in place for its products to be covered by Medicaid. If a manufacturer does not have a rebate agreement with the Secretary, a state does not have to cover that manufacturer's products until the rebate agreement is effective.

[8] If a drug is in one of the six protected classes, Medicare Part D plans are required to conduct an expedited review and render a coverage decision 90 days after it comes onto the market. At the end of the 90-day period, the drug must be added to the plan's formulary (CMS 2016a).

[9] In its June 2019 report to Congress, the Commission recommended allowing states to exclude or otherwise restrict coverage of a covered outpatient drug for 180 days after a new drug or new formulation of a drug has been approved by the FDA and entered the market (similar to the requirements for exchange plans and Medicare Part D plans). Congress has not acted on this recommendation.

[10] The covered outpatient drug rule finalized in 2016 includes a separate definition of AMP for the so-called 5i drugs—inhalation, infusion, instilled, implanted, or injectable drugs. These drugs are not generally sold through the same distribution channels as other drugs, so the AMP for 5i drugs includes sales of a type not included in AMP calculations of non-5i drugs.

[11] Generally, an innovator drug is a drug produced or distributed under a new drug application approved by the FDA. Single-source drugs are innovator drugs manufactured by only one company, and innovator multiple-source drugs are innovator drugs that have at least one generic equivalent available. Non-innovator multiple-source drugs are multiple-source drugs that are not innovator drugs—generally, these are drugs that have been approved by the FDA under an abbreviated new drug application.

[12] For blood clotting factor drugs and drugs approved by the FDA exclusively for pediatric indications, the rebate percentage is 17.1 percent of AMP, instead of 23.1 percent of AMP.

[13] Best price excludes certain governmental payers, such as the Indian Health Service, U.S. Department of Veterans Affairs, U.S. Department of Defense, Public Health Service (including 340B), Federal Supply Schedule, and Medicare Part D plans.

[14] The baseline AMP is the AMP during the quarter before the MDRP was started or, for new drugs, the first full quarter after the drug's market date. For generic drugs marketed on or before April 1, 2013, the baseline AMP is equal to the AMP for the third quarter of 2014, and the baseline CPI-U is the CPI-U for September 2014. For generic drugs marketed after April 1, 2013, the baseline AMP is equal to the AMP for the fifth full calendar quarter after which the drug is marketed as a drug other than a brand drug, and the baseline CPI-U is equal to the CPI-U for the last month of the baseline AMP quarter (CMS 2016c).

[15] The Commission recommended removing the rebate cap in its June 2019 report to Congress.

[16] In accordance with Section 2501(c) of the Patient Protection and Affordable Care Act (ACA, P.L. 111-148, as amended), 24 states—Arizona, Arkansas, California, Delaware, Florida, Illinois, Iowa, Kansas, Kentucky, Louisiana, Massachusetts, Michigan, Minnesota, Nebraska, New Hampshire, New York, North Dakota, Ohio, Oregon, Pennsylvania, Texas, Virginia, Washington, and West Virginia—are expanding supplemental rebate collections to include drugs dispensed to beneficiaries who receive drugs through an MCO. Minnesota limits its collection of supplemental rebates for MCO enrollees to direct-acting antivirals for the treatment of hepatitis C (CMS 2022c).

[17] Certain vaccines are considered covered drugs under Part D but are not considered covered outpatient drugs under the MDRP (§860D-2(e) of the Act).

[18] Medicare Part B also covers certain preventive vaccines that are explicitly listed in statute (influenza, pneumococcal, hepatitis B, and COVID-19); certain oral anticancer drugs, oral antiemetic drugs, and immunosuppressive drugs; some home infusion drugs; and clotting factor when self-administered by beneficiaries with hemophilia (MedPAC 2022a).

[19] The Inflation Reduction Act (P.L. 117-169) includes a temporary increase in Medicare Part B payment for certain biosimilars. Qualifying biosimilars may be paid at 100 percent of its own ASP plus 8 percent of the originator's biologic ASP for five years (MedPAC 2022a).

[20] Section 1861(t)(2) requires Part B coverage of anticancer chemotherapeutic regimens for indications not approved by the FDA if the drug's off-label use is supported by selected third-party compendia (MedPAC 2022a).

[21] Most recently, CMS applied CED to coverage of monoclonal antibodies directed against amyloid treatment of Alzheimer's disease (e.g., Aduhelm). In 2005, CMS

applied CED to cover off-label use of colorectal cancer drugs (oxaliplatin, irinotecan, cetuximab, or bevacizumab), linking coverage to participation in nine clinical trials sponsored by the National Cancer Institute. In 2009, Medicare applied CED for pharmacogenomic testing for warfarin response (MedPAC 2022b).

[22] The manufacturer, Eisai, Inc., has completed the confirmatory trial and submitted a supplemental biologic drug application to the FDA for traditional approval on January 6, 2023 (Eisai 2023).

[23] On January 19, 2023, the FDA did not grant accelerated approval for donanemab due to the limited number of patients with at least 12 months of drug exposure data in the phase two trial. Lilly, the manufacturer, has stated that the confirmatory phase three clinical trial is scheduled to be completed in the second quarter of 2023, and it will seek traditional approval after completion of that trial (Lilly 2023). On November 14, 2022, Roche announced that the phase three clinical trials for gantenerumab did not meet their clinical endpoints of slowing clinical decline (Roche 2022).

[24] Individuals who receive assistance only through the Medicare Savings Programs (MSPs), but do not receive full Medicaid benefits, are referred to as partial-benefit dually eligible beneficiaries. In addition, individuals may qualify for full Medicaid benefits under separate non-MSP pathways. Those who qualify for full Medicaid benefits, who may or may not receive assistance through the MSPs, are referred to as full-benefit dually eligible beneficiaries.

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Commission Vote on Recommendations

In its authorizing language in the Social Security Act (42 USC 1396), Congress requires MACPAC to review Medicaid and CHIP program policies and make recommendations related to those policies to Congress, the Secretary of the U.S. Department of Health and Human Services, and the states in its reports to Congress, which are due by March 15 and June 15 of each year. Each Commissioner must vote on each recommendation, and the votes for each recommendation must be published in the reports. The recommendations included in this report, and the corresponding voting record below, fulfill this mandate.

Per the Commission's policies regarding conflicts of interest, the Commission's conflict of interest committee convened prior to the vote to review and discuss whether any conflicts existed relevant to the recommendations. It determined that, under the particularly, directly, predictably, and significantly standard that governs its deliberations, no Commissioner has an interest that presents a potential or actual conflict of interest.

The Commission voted on these recommendations on January 27, 2023.

Medicaid Coverage Based on Medicare National Coverage Determination

3.1 Congress should amend §1927(d)(1)(B) of the Social Security Act to allow states to exclude or otherwise restrict coverage of a covered outpatient drug based on coverage with evidence development requirements implemented under a Medicare national coverage determination.

3.2 Congress should amend Section 1903(m)(2)(A)(xiii) to require the managed care contract conform to the state's policy with respect to any exclusion or restriction of coverage of a covered outpatient drug based on coverage with evidence development requirements implemented under a Medicare national coverage determination.

3.1-3.2 voting results	#	Commissioner
Yes	15	Bella, Bjork, Brooks, Carter, Cerise, Davis, Duncan, Gerstorff, Giardino, Gordon, Heaphy, Johnson, Medows, Scanlon, Weno
No	1	Allen
Not present	1	Herrera Scott