# Medicaid Medical Directors Network:

## Perspectives On The FDA Accelerated Approval Program

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### Executive Summary

Federal and state health policymakers are engaged in an emerging conversation regarding payment for prescription drugs with limited clinical evidence. Policymakers are concerned about the high premium for medications that lack demonstrated improvement of patient health outcomes. Central to this discussion are drugs approved under the U.S. Food and Drug Administration's (FDA) **Accelerated Approval Program**, which grants early-stage approval to drugs that treat serious conditions to fill an unmet medical need based on a surrogate endpoint, most often an improvement in a laboratory result. The drugs in the accelerated pathway, by definition, do not yet have a proven clinical outcome. Despite these reduced evidentiary standards for products approved under the FDA's Accelerated Approval Program, state Medicaid programs are legally obligated to pay for these drugs in the same way as drugs approved through traditional pathways because of the Medicaid Drug Rebate Program (MDRP)[1].

Medicaid programs need the ability to steward scarce resources in ways that protect the health of the country's most vulnerable and disadvantaged. However, this flexibility is challenged by the firm budgetary constraints of states, which can limit the critical services that address social determinants of health, preventive services, and emergency and acute care. Giving states the ability to target limited funds toward evidence-based population health interventions that are often under-resourced (e.g., cancer screening) would help to ensure access to quality health care for all Medicaid enrollees.

The fiscal impact of these high-cost drugs, with limited demonstrated clinical benefit, must be balanced by the needs of the program to provide services to the entire Medicaid population: achieving the correct balance is the essence of stewardship. Collectively, the Medicaid Medical Directors Network (MMDN) proposes the following drug approval policy recommendations. These recommendations represent the synthesis of many facilitated conversations among MMDN members, and in consultation with other state colleagues.

### Policy Recommendations

1. That the FDA hold drug approvals to high evidentiary standards and address issues related to transparency and cost shifting in the accelerated approval process.

2. That Medicaid programs have their federal obligation in the pharmacy benefit limited to coverage of only proven, effective, non-experimental, and non-investigative drugs; if coverage of accelerated approval drugs is required, Medicaid programs should have added federal financial support.

### Background

High-cost drugs present a growing problem to the safety-net programs that sustain our country's low-income and underserved populations. Medicaid spending on drugs increased from $43.2 billion in FY 2014 to $66.7 billion in FY 2019[2]. Much of the recent growth in drug spending has been attributed to high-cost specialty drugs (defined as those drugs exceeding $1,000 per claim)[3]. While the overall share of high-cost drug prescriptions remained relatively stable, the share of spending on these drugs has increased substantially, as they accounted for only 31 percent of total spending in FY 2014 but accounted for almost 44 percent of spending in FY 2017 (*Table 1*). Average spending for these drugs has increased from $2,600 per claim in FY 2014 to over $3,100 in FY 2017, reflecting price inflation for existing drugs as well as the introduction of new high-cost drugs. There are several drugs in the "high-cost" category planning to be released within the next 18 months[4].

Additionally, states are often devoting increasingly limited financial resources toward high-cost specialty drugs that may ultimately have no real clinical benefit. Specifically, during the first half of 2020, the FDA granted 16 distinct oncology accelerated approvals, with a mean price of $18,198 per month[5]. Medicaid alone spent at least $1.2 billion on drugs that received accelerated approvals in 2019[6].

### About this Paper

This paper is informed by an advisory committee of the Medicaid Medical Directors Network (MMDN). It is intended to alert policymakers, elected officials, health care providers, patients and the public-at-large, to the increasingly negative fiscal impact of high-cost drugs vetted through the FDA's Accelerated Approval Program and the ways these costs jeopardize the sustainability of other essential Medicaid health benefits. It does not represent the position of any individual Medical Director or any one state, or its Medicaid agency. It serves to reflect the diversity of thought and opinion among MMDs on this subject. Every state is well-advised to consider its own unique population needs, geography and challenges due to ever-changing environments.

**Table 1: Medicaid Drug Claims and Gross Spending for Drugs over $1000/claim, 2014-2017[11]**

| Fiscal Year | Drug Claims ($M) | Gross Spending ($B) | Spending per Claim | Total Claims % | Total Spending % |
|---|---|---|---|---|---|
| 2014 | 5.2 | 13.4 | 2,597 | 0.9% | 31.1% |
| 2015 | 6.3 | 18.4 | 2,822 | 1% | 34.4% |
| 2016 | 7.3 | 23.9 | 3,252 | 1% | 39.2% |
| 2017 | 8.8 | 27.8 | 3,174 | 1.2% | 43.7% |

## The FDA Accelerated Approval Program

The Centers for Medicare and Medicaid Services (CMS) policy states that Medicaid funding cannot be directly used for experimental or unproven treatments. However, the FDA's Accelerated Approval Program, while well-intentioned, is predicated upon surrogate endpoints which are often theorized but not proven to link to clinical outcomes. The process thus highlights the fiduciary tension when a given therapy transitions between "experimental" to "established".

The FDA instituted its Accelerated Approval Program to facilitate a more rapid approval of drugs that treat serious conditions, and that meet a pressing medical need based on a surrogate endpoint. A surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure. Oftentimes these surrogate endpoints are theorized to result in clinical benefit, but ultimately require further study—for example, in the case of Duchenne Muscular Dystrophy, dystrophin production may be used as a surrogate endpoint, but preservation of muscle function and mobility is the ultimate desired outcome. As such, the accelerated approval process often cites unpublished clinical studies in its decision-making[7]. This absence of peer-reviewed published literature makes medical appropriateness decision-making by state Medicaid programs extremely difficult to perform. The accelerated approval pathway is the only FDA approval pathway where institutional approval might be interpreted as an "interim" because the approval can be based on studies using surrogate or intermediate endpoints rather than clinical benefit.

The FDA has awarded over two hundred and fifty drugs accelerated approval status since inception and the program continues apace. Some drug classes, including those related to oncology, now obtain most of their drug approvals using this pathway[8]. While many accelerated approval drugs ultimately convert to full approval, several recent examples, which made national headlines, raise increasing concerns about reduced evidentiary standard for drug approvals.

Additionally, accelerated approval status is linked to aftermarket studies of clinical efficacy. However, in practice, aftermarket studies are frequently delayed, and early-stage approval often compromises the ability to study a comparison population that is not taking the drug[9]. Furthermore, label approval may be granted to drugs on a population far broader than the community studied. Approvals have historically been infrequently revoked based on the absence of confirmatory trials. Presently, this places Medicaid in the conflicted position of paying for high-cost, high-resource therapies still under active investigation for clinical benefit.

## Considerations for drug coverage in Medicaid: The Medicaid Drug Rebate Program (MDRP)

Medicaid programs are unique in their drug coverage decisions because they are tied to FDA approval by the Medicaid Drug Rebate Program (MDRP). The MDRP was created in 1990 by the Omnibus Reconciliation Act and was most recently amended by the Affordable Care Act. By design, the MDRP allows drug manufacturers to obtain Medicaid coverage of their drugs by entering into an agreement with the U.S. Department of Health and Human Services. Under this agreement, the manufacturer agrees to rebate a portion of the amount paid by Medicaid for drug costs back to the states, who must also share the rebates with the federal government. In exchange for entering the MDRP, the drug manufacturer guarantees that Medicaid will cover nearly all the manufacturer's FDA-approved drugs[10]. This level of universal drug coverage is unlike that in commercial insurance and even in other developed nations, who still utilize market leverage and feature a closed drug formulary, that lists which drugs are covered by the insurance program. Therefore, while other public payers (e.g., Medicare and VA) can make their own drug approval decisions, Medicaid drug coverage is linked to FDA approval by federal statute.

The MDRP is most effective when states have robust market competition in a specific drug class. As manufacturers compete in a drug class (e.g., statins) among the non-Medicaid marketplace based on price, Medicaid as a state-funded program serving the underserved, benefits from the drug rebates available to it. Drug manufacturers also benefit by receiving universal coverage of their drug portfolio (e.g., all statins are still covered by Medicaid). However, the MDRP is ineffective when a unique drug enters the market and has no competing manufacturer upon its entry (e.g., a novel Hepatitis C treatment). In such a scenario, Medicaid is

compelled to cover the drug within its formulary regardless of set price; a scenario that differs from commercial insurers. As such, drug manufacturers are also able to set disproportionately high market prices for unique drugs knowing that the Medicaid program, covering 20 percent of the U.S. population, half of its children, and a high proportion of individuals with complex medical conditions requiring these drugs, will have to cover them. MDRP rebate amounts are not set by statute and represent a percentage of drug list prices set by manufacturers making these rebates largely irrelevant for specialty drugs. While states feature low or no Medicaid co-payments for their covered and underserved populations by design, state budgets bear the brunt of this cost. The limited tools, such as prior authorization or preferred drug listings, are available to focus drug spending on appropriate use but have limited value when drugs have a unique indication.

## Policy principles moving forward

Medicaid Medical Directors (MMDs) intimately understand the importance of providing every patient with the best possible medical care. The clinicians that make up the Network membership have treated patients with severe, rare conditions and have counseled families about treatments that may or may not be beneficial. MMDs have the lived bedside experience of working with families and patients on their journeys and understand how important these therapies may seem. Oftentimes the evidence does not bear out clinical benefit for high-cost/accelerated approval therapies. FDA accelerated approval grants broad access to therapies that may unfortunately facilitate false hope and misdirected resources that could be channeled to more effective interventions for these same individuals.

Moving forward, several policy principles should be considered for accelerated approval drugs in Medicaid. While these recommendations cannot stand on their own, nor can they individually solve the complex problem of high-cost specialty drug pricing in the U.S., they are critical pieces of the broader puzzle of rising drug costs.

First, the Medicaid Medical Directors urge *the FDA to hold drug approvals to high evidentiary standards and address issues related to transparency and cost shifting in the accelerated approval process*. Drugs for rare diseases should receive labels that reflect their immature status and accelerated approval based on small clinical trials with proxy endpoints. Aftermarket studies should be held to defined timelines to prove clinical effectiveness, as running trials for many years after approval effectively shifts the cost of research and experimentation onto states and the federal government. Clinical trial registries for gene therapies should be more robust and research processes in these registries should be transparent throughout the research period and analysis.

Second, we propose that *Medicaid programs have their federal obligation in the pharmacy benefit limited to coverage of only proven, effective, non-experimental, and non-investigative drugs.* This could be accomplished either through enhanced Federal Medical Assistance Percentage (FMAP) for accelerated approval drugs, direct federal purchasing, or federal negotiating of drug prices. States with smaller populations have less of a cushion than larger states in balancing budgets—even relatively small changes in utilization of high-cost drugs can have a profound impact on a state's ability to provide other critical services. Barring federal intervention, high-cost drug pools across states may offer relief but face significant logistical and regulatory challenges, as do risk corridors. Allowing waivers to the MDRP may enable states to enact evidence-based formularies rather than solely rely on FDA approval decisions.

## Conclusion

Medicaid Medical Directors are the state lead clinicians with responsibility for overseeing clinical care and stewardship of resources. We are acutely aware of the potential benefits these medications offer Medicaid enrollees amidst the competing challenges their current approval and pricing create for the stewardship of state and federal resources.

This issue that has come to the attention of the **National Association of Medicaid Directors (NAMD)**, the **National Governors Association (NGA)** and the **Medicaid and CHIP Payment and Access Commission (MACPAC)**. By adding our perspective as clinicians immersed in Medicaid policy, we aim to promote efforts to address these concerns. Our recommendations track with solutions recently proposed by MACPAC, which seek to promote the clinical advances that science is producing while being mindful of the financial stewardship required to provide the best outcomes for all Medicaid clients.

Resolving this issue will require federal legislative action. We are interested in working collaboratively with other partners to find solutions to improve clinical outcomes and steward resources

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## About the Medicaid Medical Directors Network (MMDN)

The MMDN seeks to advance more equitable, high-quality, accessible health care for all Medicaid beneficiaries by providing a forum for senior clinical leaders to discuss their most pressing needs and evidence-based solutions. As the professional home of the MMDN, AcademyHealth maintains a strategic partnership with more than 40 state Medicaid Medical Directors (MMDs) – committed to participating in multi-state data projects on pressing policy topics, hosting yearly convenings, and leveraging their collective experience to bolster both state and national Medicaid program initiatives. With support from several partner organizations including the Agency for Healthcare Research and Quality (AHRQ), the Patient-Centered Outcomes Research Institute (PCORI), and the Centers for Disease Control and Prevention (CDC), the MMDN is committed to synthesizing and disseminating relevant findings to policymakers in a timely and translatable manner.

## About AcademyHealth

AcademyHealth is a leading national organization serving the fields of health services and policy research and the professionals who produce and use this important work. Together with our members, we offer programs and services that support the development and use of rigorous, relevant, and timely evidence to increase the quality, accessibility, and value of health care, to reduce disparities, and to improve health. A trusted broker of information, AcademyHealth brings stakeholders together to address the current and future needs of an evolving health system, inform health policy, and translate evidence into action.

Learn more at www.academyhealth.org and follow us on Twitter @AcademyHealth.

## Endnotes


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