PARTNERSHIP TO FIGHT CHRONIC DISEASE

April 7, 2021

Medicaid and CHIP Payment and Access Commission (MACPAC)
1800 M Street, NW, Suite 650 South
Washington, DC 20036

Submitted via comments@macpac.gov
Re: MACPAC Considerations on Accelerated Approval Drugs

Dear MACPAC Commissioners:

The Partnership to Fight Chronic Disease and other undersigned organizations share grave concerns about the potential recommendations MACPAC is considering that threaten to undermine access to medicines and continued innovation for individuals living with serious illnesses. We urge you to reject the recommendations that would impose a new, differential Medicaid rebate for accelerated approval drugs. We draw your attention to a recent economic analysis that supports preserving access to accelerated approval drugs for the seriously ill and continuing to fulfill Medicaid's mission of providing health care coverage to America's most vulnerable.

Recently, Kenneth E. Thorpe, PhD, PFCD Chair and Chair of the Department of Health Policy & Management for the Rollins School of Public Health at Emory University and Douglas Holtz-Eakin, PhD, President of the American Action Forum and former CBO Director, completed an economic analysis of Medicaid spending and the growth in Medicaid spending 2007 through 2018 by cost component, including accelerated approval drugs. *Limiting Medicaid Access to Accelerated Approval Drugs: Costs and Consequences* appears online in the *American Journal of Managed Care* (copy attached). An accompanying commentary can be found here.

The analysis examines the utilization and spending of accelerated approval drugs and their proportion of total Medicaid spending and proportional contribution to spending growth. The authors also discuss the risks of upending the long-standing accelerated approval pathway and Medicaid Drug Rebate Program agreement to enable facilitate for Medicaid's most vulnerable populations. We strongly encourage you to consider the entire analysis and point to several key conclusions drawn from it:

- Accelerated approval drugs accounted for less than 1 percent of Medicaid spending consistently every year.

- Medicaid spending on accelerated approval drugs remained steady at 0.6-0.8 percent a year after 2012 passage of the Food and Drug Safety and Innovation Act, which encouraged accelerated approval use for rare and other serious conditions in addition to oncology and HIV/AIDS treatments.
- Accelerated approval drugs accounted for 1.3% of the growth in Medicaid spending 2007-2018.

Medical and prescription drug coverage provided through Medicaid is a lifeline for millions of individuals and families. Protecting that coverage and the access to comprehensive medical services and treatment is of paramount importance and should dominate discussions of reforms that place that access at risk.

New medicines approved through FDA's accelerated approval pathway have made novel therapeutics, developed in response to significant unmet medical needs, available to people with serious or life-threatening cancers, HIV/AIDS and related conditions, and rare diseases with limited or no available treatment options. These new therapeutics often are the first available treatments for a given disease. We are troubled by the commentary at the last MACPAC meeting that insinuates that these drugs somehow evade FDA scrutiny and are unsafe or ineffective. People living with serious or life-threatening conditions dependent on Medicaid for access to accelerated approval medications are arguably among Medicaid's most vulnerable populations and reforms aimed at restricting access to treatments should be viewed with those significant vulnerabilities foremost in mind.

Also, consideration of the total burden of illness on patients is critically important particularly when making policy changes that could dramatically affect access to treatments that can extend life or otherwise lessen the burden of illness. As detailed in EveryLife Foundation's burden of illness study for rare diseases, the costs of rare disease exceed $1 trillion a year with indirect costs, non-medical and uncovered expenses exceeding medical expenses. The availability of the accelerated approval pathway provides hope to reduce the burden of illness for many living with rare diseases and other serious illnesses – often positively affected these indirect costs, such as facilitating independence, increasing productivity, or reducing caregiver burden.

As the Thorpe-Holtz Eakin economic analysis shows, accelerated approval drugs have a *de minimus* impact on Medicaid spending and spending growth. The ostensible basis for this policy proposal is that accelerated approval drugs are driving spending, but there is no evidence that that is the case. However, there is substantial evidence that these drugs are delivering effective treatments and cures to vulnerable populations. The Commission should not advance a policy that would deter manufacturers from pursuing the accelerated approval pathway,

would undermine access to critical drugs in Medicaid, and have a devastating impact on individuals living with serious or life-threatening illnesses.

We urge you to consider the harm presented by proposed policy changes and vote against the proposed policy changes on Medicaid rebates and reimbursement for accelerated approval drugs.

Respectfully submitted,

Kenneth E. Thorpe, PhD
Chair

And the undersigned organizations:

ACCSES - The Voice of Disability Service Providers
Alliance for Aging Research
American Autoimmune Related Diseases Association
American Brain Coalition
Association of University Centers on Disabilities (AUCD)
Barth Syndrome Foundation
Beyond Type 1
Caregiver Action Network
The Coelho Center for Disability Law, Policy and Innovation
The COSHAR Healthy Communities Foundation
Cystic Fibrosis Research Institute
EveryLife Foundation for Rare Diseases
Genetic Alliance
Global Liver Institute
HealthyWomen
HIV + Hepatitis Policy Institute
ICAN, International Cancer Advocacy Network

PARTNERSHIP TO FIGHT
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LEAD Coalition (Leaders Engaged on Alzheimer's Disease)
Lupus and Allied Diseases Association, Inc.
Lupus Foundation of America
Mental Health America
The Migraine Diva
Multiple Sclerosis Foundation
NBIA Disorders Association
National Kidney Foundation
National Task Group on Intellectual Disabilities and Dementia Practices
NTM Info and Research
Organic Acidemia Association
Patients Rising Now
PXE International
VHL Alliance
Volunteers of America

COMMENTARY

Limiting Medicaid Access to Accelerated Approval Drugs: Costs and Consequences

Kenneth E. Thorpe, PhD; and Douglas Holtz-Eakin, PhD

State budget shortfalls resulting from the COVID-19 pandemic will likely amplify efforts to cut Medicaid spending. In advising on targets for Medicaid cuts, the National Governors Association[1] has identified Medicaid coverage of "selected fast-tracked, first-in-class drugs," such as those approved through the FDA's accelerated approval pathway, as ripe targets. Two states, Massachusetts[2] and Tennessee,[3] have sought federal waivers from Medicaid coverage requirements to limit patient access to these medicines. The efforts raise serious patient and provider concerns about access to medicines for a Medicaid population living with serious or life-threatening, often rare, conditions without treatment options—individuals for whom the accelerated approval pathway was developed.

By pursuing access limitations in Medicaid, states presume that any savings to be had are worth restricting access for these patients. An analysis of Medicaid spending on accelerated approval drugs between 2007 and 2018 and their impact on the growth in Medicaid spending shows that accelerated approval drugs accounted for less than 1% of annual Medicaid spending consistently year over year. These data support preserving Medicaid access to accelerated approval drugs for the seriously ill.

Background

New medicines approved through FDA's accelerated approval pathway[4] have made novel therapeutics for serious or life-threatening conditions available more quickly to the benefit of patients, particularly those battling cancers or rare diseases with limited to no treatment options.[5] Congress first codified the accelerated approval pathway in 1997.[6] Accelerated approval drugs changed the course of disease for individuals with HIV/AIDS and many cancers by driving innovation for these previously underserved populations.[5] Congress modernized and enhanced the pathway in 2012 to expand its use for rare diseases.[7]

For accelerated approval applications, the FDA must also consider the severity, rarity, or prevalence of the condition treated and the availability or lack of existing treatments.[7] FDA approval is contingent on the sponsor's completion of a longer-term, confirmatory study. A streamlined withdrawal process exists for drugs that are found to be unsafe, or for which benefit is not confirmed, or for which no confirmatory trials are conducted by their sponsor.

Increasingly, Medicaid and other payers are implementing significant coverage barriers or refusing to cover these medicines outright, arguing that accelerated approval drugs have insufficient or limited evidence despite these drugs meeting the FDA's safety and efficacy standards. A recent Kaiser Family Foundation study[8] indicates that a majority of states are developing strategies related to new high-cost therapies, particularly those approved on an accelerated pathway. Notably, the National Governors Association has called for federal action "[a]llowing state Medicaid programs to exclude…select fast-tracked, first-in-class drugs that lack sufficient data on safety and efficacy, until such evidence is produced."[1]

Delaying Medicaid coverage of FDA-approved treatment options undermines the intent and urgency of the accelerated approval pathway, as access delays can mean irreversible harm. Because expressed reluctance to cover these medicines includes financial concerns about costs of accelerated approval drugs, understanding the extent to which accelerated approval drugs affect Medicaid spending overall and spending growth is important to these deliberations.

TAKEAWAY POINTS

States seek to limit coverage of drugs approved through the FDA's accelerated approval pathway, which is designed to accelerate availability of medications that treat serious or life-threatening conditions. Analysis of Medicaid spending from 2007 to 2018 shows:

- Accelerated approval drugs accounted for less than 1% of Medicaid spending consistently every year.
- Medicaid spending on accelerated approval drugs remained steady at 0.6% to 0.8% a year after 2012 passage of the Food and Drug Safety and Innovation Act, which encouraged accelerated approval use for rare conditions in addition to oncology and HIV/AIDS.
- These data support preserving access to accelerated approval drugs for the seriously ill.

Estimating Impact on Medicaid Spending

To estimate the impact of accelerated approval drugs on overall Medicaid spending and spending growth, data on Medicaid spending for each year from 2007 to 2018 were collected from the National Health Accounts tabulated by CMS.[9] These data also include Medicaid-specific spending on hospital care, physician and clinic services, nursing homes, home health, and prescription drugs, among other covered services. Spending includes Medicaid managed care and fee-for-service spending. Accelerated approval drugs were identified from the FDA Center for Drug Evaluation and Research drug and biologic accelerated approvals document as of December 31, 2019.[10] These data informed calculations of the Medicaid amount reimbursed for each year. Total Medicaid drug reimbursements for the year were also tabulated.[11]

Between 2007 and 2018, total Medicaid spending increased from $326 billion to more than $597 billion, an average increase of 5.7% per year. As shown in **Figure 1**, hospital spending consumed the largest share of Medicaid spending year over year, followed by physician and clinical services and prescription drugs. Accelerated approval drugs accounted for a small percentage of overall Medicaid spending between 2007 and 2018. In 2007, these drugs accounted for 0.3% of Medicaid spending, rising to 0.7% by 2012 and remaining relatively constant through 2018. Notably, the portion of Medicaid spending for accelerated approval drugs did not increase and remained steady at 0.6% to 0.8% a year after the 2012 passage of the Food and Drug Safety and Innovation Act,[7] which codified the pathway and encouraged its use for rare diseases.

Similarly, as **Figure 2** illustrates, hospital spending contributed the most to Medicaid spending growth. Over the 2007-2018 period, hospital spending accounted for nearly 30% of the growth in Medicaid spending. During the same period, increased spending on prescription drugs accounted for 16.7% of the growth in Medicaid spending. Between 2007 and 2018, increased spending on accelerated approval medications accounted for only 1.3% of the overall growth in Medicaid spending.

It is important to note that the analysis does not take into account any prescription drug rebates, which in 2017 accounted for 55% of state Medicaid drug spending.[12] The lack of available detailed information on Medicaid rebates obscures information about rebates specific to accelerated approval drugs and other on-patent medicines. That limits the analysis and ability to quantify how rebates relating to both accelerated approval and other on-patent medicines affect net drug spending, although spending net of rebates would decrease the total spent on prescription drugs, as well as the percentage of Medicaid spending growth attributable to prescription drugs overall.

During the study period, Medicaid also underwent significant changes largely driven by Medicaid expansion as a part of the Affordable Care Act. Most notably, after 2010, enrollment in the program increased by an average of 3.9% per year.[13] Although spending on all categories of care increased during Medicaid expansion, the percentage of total Medicaid spending attributable to accelerated approval drugs remained stable between 2010 and 2018.

FIGURE 1. Distribution of Medicaid Spending by Source and by Accelerated Drug Approval Status, 2007-2018

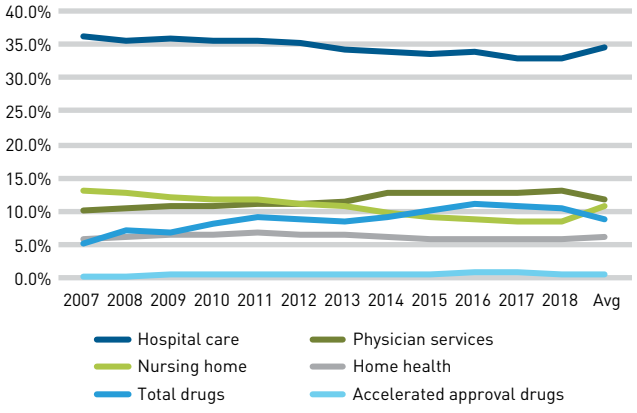
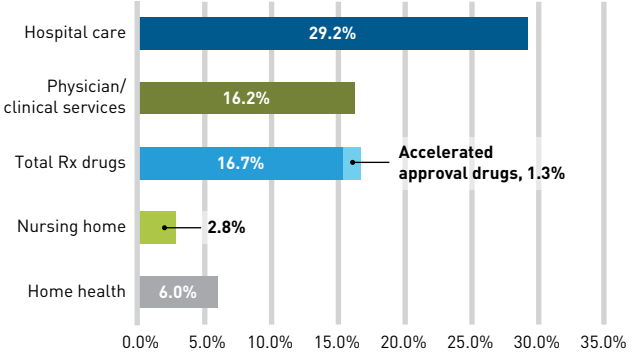


FIGURE 2. Percentage of Increase in Medicaid Spending by Source of Care and by Accelerated Drug Approval Status, 2007-2018[a]



[a]A varied assortment of smaller contributors not illustrated account for the remaining percentage of spending increases.

Policy Implications

Drugs approved via accelerated approval are novel treatments that address urgent and unmet medical needs involving serious and life-threatening diseases. Since inception, the accelerated approval pathway has resulted in tremendous advances, most notably for HIV/AIDS and many cancers. Despite cost concerns, the analysis on drivers of Medicaid spending shows that accelerated approval drugs have a de minimis impact on spending while addressing significant unmet medical needs. The potential human costs far outweigh potential savings from coverage restrictions.

Relatedly, the Congressional Budget Office (CBO) recently evaluated the savings potential of a proposal by the Medicaid and CHIP Payment and Access Commission to "give states a set period of time to evaluate the clinical evidence for new drugs and determine

appropriate coverage criteria."[14] CBO estimated that proposal would decrease federal spending by less than $25 million over 10 years, with savings primarily resulting from delaying the start of the coverage period and shifting some spending to another budget window. In fiscal year 2018, federal spending on Medicaid amounted to 62% of total Medicaid spending,[15] so the state share of savings estimated by the CBO would be less overall and then divided among the states. In contrast, given that accelerated approval drugs must address serious or life-threatening conditions with significant unmet medical needs, a 6-month delay in access to treatment could have a profound, irreversible impact on patients.

States seeking to limit access to accelerated approval drugs must obtain approval from CMS to waive drug coverage requirements that enable states to collect substantial rebates under the Medicaid Drug Rebate Program (MDRP). The MDRP generates rebates that reduce Medicaid drug costs by more than half.[13] Allowing such exceptions to the MDRP could open it up to further changes that weaken Medicaid's ability to provide affordable access to therapeutics for serious unmet medical needs that FDA's accelerated approval pathway was designed to address. CMS has declined Massachusetts' Medicaid waiver request[2] for drug coverage exceptions, noting that accelerated approval drugs "must be covered by State Medicaid programs" if the manufacturer participates in the MDRP.[16] States can use utilization management mechanisms to ensure appropriate use, and they have greater coverage discretion for drugs from manufacturers not participating in the MDRP. Tennessee's Medicaid waiver request[3] that could include coverage restrictions for accelerated approval medicines was approved by the Trump administration in January 2021.[17] Its future, given the change in administration, is uncertain.

Conclusions

Medicaid programs seeking ways to meaningfully mitigate budgetary impacts from rising health care costs will not find success in restricting or eliminating coverage of accelerated approval drugs. This analysis of Medicaid claims data from 2007 to 2018 shows that such efforts are misguided. Accelerated approval drugs account for less than 1% of overall Medicaid spending while often representing the only treatment options available for beneficiaries. During the COVID-19 pandemic, in particular, individuals living with serious conditions face increased vulnerability. This reality places paramount importance on facilitating access to treatments for patients with serious or life-threatening conditions. Limiting Medicaid coverage for accelerated approval drugs would have a devastating impact on patients benefiting from these treatments while having a de minimis impact on spending. ■

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