Quantifying Impact of Accelerated Approval Drugs on Medicaid Spending

De Minimus Impact, Maximum Attention

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KEY POINTS: States seek to limit coverage of drugs approved through the FDA's accelerated approval pathway 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 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.**
- **These data support preserving access to accelerated approval drugs for the seriously ill.**

ABSTRACT

State policymakers are seeking CMS approval to limit coverage of drugs approved through the Food and Drug Administration's (FDA) accelerated approval pathway designed to speed availability of medications that treat serious or life-threatening conditions. The National Governors Association and several states are targeting these therapies because of concerns over the perceived level of impact on Medicaid spending. This analysis identified accelerated approval drugs between 2007 and 2018 and estimated the impact they had on the growth in Medicaid spending. The analyses show that accelerated approval drugs accounted for less than one percent of annual Medicaid spending consistently year over year. These data support preserving access to accelerated approval drugs for the seriously ill as a part of Medicaid reform efforts.

INTRODUCTION

New medicines approved through FDA's accelerated approval pathway have made novel therapeutics for serious or life-threatening conditions available more quickly to the benefit of patients, particularly those battling life-threatening cancers or rare diseases with limited to no treatment options. These new therapeutics often are the first available treatments for a given disease and may spur development of even more effective treatments. Increasingly, Medicaid and other payers are implementing significant coverage barriers or refusing to cover these medicines outright claiming limited or insufficient evidence of effectiveness or inaccurately suggesting that accelerated approval treatments are subject to lower FDA efficacy and safety standards for approval. Such coverage decisions force patients to wait longer to access FDA-approved treatment options, undermining the intent and urgency associated with the accelerated approval pathway, and potentially disincentivizing innovation. Since expressed reluctance to cover these medicines includes financial concerns about costs of accelerated approval drugs, the analysis included calculating the total costs of accelerated approval medicines to Medicaid from 2007 through 2018 and revealed that these medicines comprised less than one percent of Medicaid annual spending with no significant increase over this timeframe.

FDA's Accelerated Drug Approval Program

The Food and Drug Administration has developed five distinct programs for making drugs for serious or life-threatening conditions available safely and rapidly: fast track designation, breakthrough therapy designation, Regenerative Medicine Advanced Therapy (RMAT) designation, priority review designation and accelerated approval pathway.[1] Importantly, none of the programs compromises FDA's long-standing standards for safety and efficacy. Of the five, only accelerated approval provides an alternate pathway to approval. The analysis focuses on accelerated approval drugs for this reason.

The FDA created the accelerated approval pathway through regulations in 1992 in response to the growing HIV-AIDS epidemic[2] where immune cell response was used as a surrogate endpoint to predict survival. Using a surrogate endpoint that is reasonably likely to predict long-term clinical benefits, which would otherwise take years to measure, saves valuable time for patients whose critical illness does not afford time to wait. Congress first codified the accelerated approval pathway in 1997[3] and then modernized and enhanced it in 2012[4] to expand the pathway's use beyond treatments for HIV/AIDS or oncology to include rare diseases.

The accelerated approval pathway allows drugs for serious conditions that fill an unmet medical need to be approved based on a surrogate or intermediate clinical endpoint that is reasonably likely to predict a clinical benefit. The FDA relies upon scientific support to determine acceptability of a given endpoint for this purpose. Studies demonstrating a drug's effect on a surrogate or intermediate clinical endpoint must also meet legal standards demonstrating the study is "adequate and well controlled."[2]

In weighing 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.[4] Under accelerated approval, the FDA's continued approval is contingent on the sponsor's completion of a longer-term, confirmatory study after approval. A streamlined withdrawal process exists for drugs where benefit is not confirmed, drugs are found to be unsafe, or the sponsor fails to conduct confirmatory trials.

A number of payers have singled out accelerated approval drugs for coverage limits and exclusions, arguing that accelerated approval drugs have insufficient evidence and should be considered experimental, despite these drugs meeting FDA's safety and efficacy standards. For example, Tennessee and Massachusetts Medicaid agencies have recently petitioned CMS for the ability to not cover accelerated approval drugs, citing cost and effectiveness concerns. Specifically, TennCare's Medicaid proposal states "much of the current volatility in prescription drug prices is driven by new drugs coming to market through the FDA's accelerated approval pathway… [though] many of them have not yet demonstrated actual clinical benefit."[6] A recent Kaiser Family Foundation study[7] indicates that a majority of states are developing strategies related to new high-cost therapies, particularly those approved on an accelerated pathway, a high priority.

The analysis below examines utilization and spending of accelerated approval drugs and their proportion of total Medicaid spending and proportional contribution to spending growth, as well as the risks of upending the long-standing accelerated approval pathway and Medicaid Drug Rebate Program agreement.

METHODS AND DATA

The analysis started with the growth in Medicaid spending nationally from 2007-2018 and then involved estimates on how much of the growth in Medicaid spending is associated with each source of care (hospital services, physician and clinic services, nursing home, home health, prescription drugs overall, and accelerated approval drugs specifically). The analysis does not take into account any prescription drug rebates, which in 2017 accounted for 55 percent of state Medicaid drug spending.[8] Spending includes Medicaid managed care as well as fee-for-service spending.

Accelerated approval drugs were identified from the FDA Center for Drug Evaluation and Research (CDER) drug and biologic accelerated approvals document.[9] These data informed calculations of the Medicaid amount reimbursed for each year. Total Medicaid drug reimbursements for the year were also tabulated.[10]

Data on Medicaid spending for each year 2007-2018 were collected from the National Health Accounts tabulated by the Centers for Medicare and Medicaid Services (CMS). These data also include Medicaid specific spending on hospital care, physician and clinic services, nursing homes, home health, prescription drugs among other covered services.[11] The results are presented below.

RESULTS

Exhibits 1 and 2 present data on total Medicaid spending and the distribution of spending by source of treatment. Between 2007 and 2018 total Medicaid spending increased from $326 billion to over $597 billion, an average increase of 5.7 percent per year. Hospital spending accounts for the largest share of Medicaid spending, though hospital spending as a percentage of total declined from 36 percent in 2007 to under a third in 2018. Physician and clinic services accounted for a growing share of Medicaid spending, increasing from 10 percent in 2007 to 13 percent by 2018. Total retail drug spending accounted for just over 5 percent of Medicaid spending in 2007 to over 10 percent in 2018, not including drug rebates.

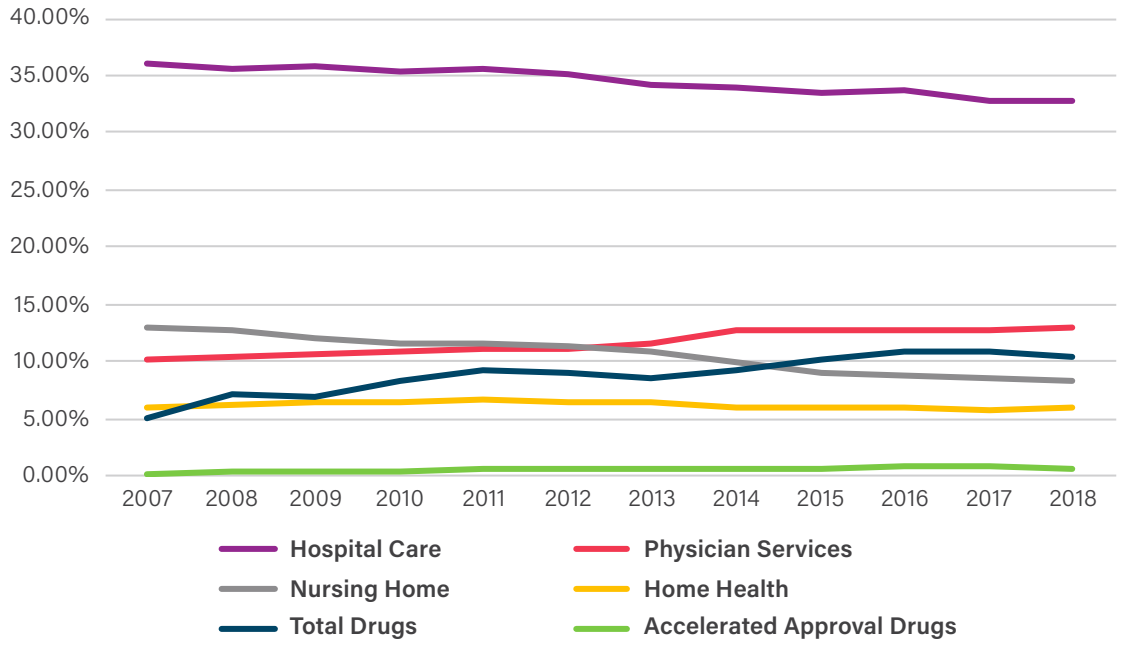
Exhibit 1: Total Medicaid Spending by Source of Care and by Accelerated Drugs Approval Status, 2007-2018 (in billions US $)

Year	Total	Hospital Care	Physician Clinic Services	Nursing Home	Home Health	Total Drugs	Accelerated Approval Drugs
2007	325.9	117.2	33.5	42.3	19.6	17	0.95
2008	344.4	122.7	35.9	44.0	21.6	24.6	1.456
2009	374.7	133.6	40.2	45.5	24.0	26	1.702
2010	397.4	141.8	43.3	46.3	25.9	33	2.173
2011	406.7	143.7	45.2	47.6	27.1	37.8	2.479
2012	422.9	150.0	47.0	47.7	27.7	37.9	2.773
2013	445.2	156.1	51.5	48.1	28.7	37.8	2.883
2014	497.8	169.8	63.4	49.1	30.3	46.4	3.392
2015	542.6	183.9	69.1	49.4	32.4	55.7	3.927
2016	565.4	189.2	72.2	49.8	33.8	62.1	4.428
2017	590.1	192.7	75.2	50.0	35.0	64.3	4.538
2018	597.4	196.6	77.4	49.9	35.9	62.3	4.377

Source: See Data and Methods on Spending

Accelerated approval drugs account for a small percentage of overall Medicaid spending during 2007-2018, as shown in **Exhibit 3**. In 2007, these drugs accounted for 0.3 percent of Medicaid spending rising to 0.7 percent by 2012 and remaining relatively constant through 2018. Notably, 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 (FDASIA), which codified the pathway and encouraged its use for conditions in addition to oncology and HIV/AIDS.

Exhibit 2: Distribution of Medicaid Spending by Source and by Accelerated Drug Approval Status, 2007-2018



Source: See Data and Methods on Spending

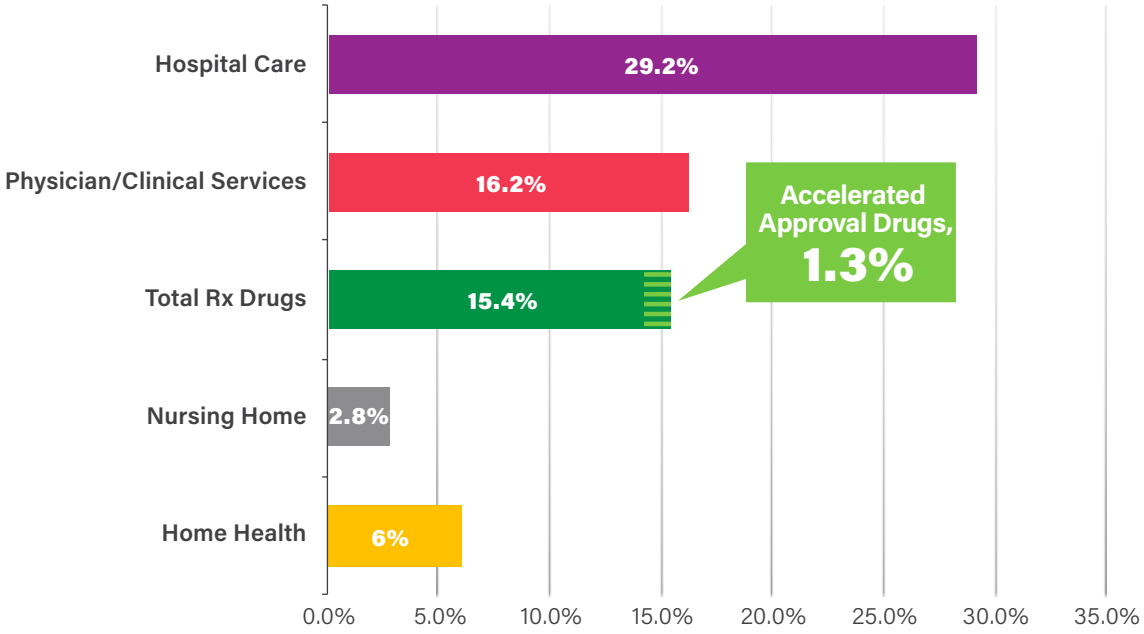
Exhibit 3: Distribution of Medicaid Spending by Source and by Accelerated Drug Approval Status, 2007-2018

Year	Hospital Care	Physician Clinic Services	Nursing Home	Home Health	Total Drugs	Accelerated Approval Drugs
2007	36%	10.3%	13.0%	6.0%	5.2%	0.3%
2008	35.6%	10.4%	12.8%	6.3%	7.1%	0.4%
2009	35.7%	10.7%	12.1%	6.4%	6.9%	0.5%
2010	35.3%	10.9%	11.7%	6.5%	8.3%	0.5%
2011	35.5%	11.1%	11.7%	6.7%	9.3%	0.6%
2012	35.1%	11.1%	11.3%	6.6%	9.0%	0.7%
2013	34.1%	11.6%	10.8%	6.4%	8.5%	0.6%
2014	33.9%	12.7%	9.9%	6.1%	9.3%	0.7%
2015	33.5%	12.7%	9.1%	6.0%	10.3%	0.7%
2016	33.7%	12.8%	8.8%	6.0%	11.0%	0.8%
2017	32.9%	12.7%	8.5%	5.9%	10.9%	0.8%
2018	32.9%	13.0%	8.4%	6.0%	10.4%	0.7%
Average	**34.5%**	**11.7%**	**10.7%**	**6.7%**	**8.8%**	**0.6%**

Source: See Data and Methods on Spending

Exhibit 4 examines the sources of care that account for the growth in Medicaid spending 2007-2018. The analysis examines four time periods, 2007-2010, 2010-2015, 2015-2018 and 2007-2018. As shown in Exhibit 5, over each of these periods, the growth in hospital spending accounted for the largest share of the overall increase in Medicaid spending. Over the 2007-2018 period, the growth in hospital spending accounted for nearly 30 percent of the growth in Medicaid spending. Over the same period, increased spending on drugs accounted for 16.7 percent of the growth in Medicaid spending, similar to increased spending on physician and clinical services. The growth in total retail drug spending has accounted for a declining share of the growth in Medicaid overall. Between 2010 and 2015, rising spending on drugs accounted for 15.6 percent of the growth in overall Medicaid spending. However, in the most recent period, increased spending on retail prescription drugs overall only accounted for 10 percent of rising Medicaid spending.

Exhibit 4:



Source: See Data and Methods on Spending

Note: A variety of smaller contributors not illustrated account for the remaining percentage of spending increases.

Exhibit 5: Percent of Increase in Medicaid Spending by Source of Care and by Accelerated Drugs Approval Status, 2007-2018

Year	Hospital Care	Physician Clinic Services	Nursing Home	Home Health	Total Drugs	Accelerated Approval Drugs
2007-2010	35.7%	10.9%	11.7%	6.5%	8.3%	0.5%
2010-2015	29.0%	17.8%	2.1%	4.5%	15.6%	1.2%
2015-2018	20%	20%	0.1%	10%	10%	0.1%
2007-2018	29.2%	16.2%	2.8%	6.0%	16.7%	1.3%

Source: See Data and Methods on Spending

Increased spending on drugs approved through the accelerated approval process accounts for a small fraction of the growth in Medicaid spending. Between 2007 and 2018, increased spending on these medications accounted for only 1.3 percent of the growth in overall Medicaid spending. In the most recent period, these medications only accounted for 0.1 percent of the overall increase in Medicaid spending.

DISCUSSION

Unlike the federal government, governors and state legislatures are responsible for spending within balanced budgets. Like other large purchasers, states are concerned about rising health care costs. The perennial focus on lowering health care spending, the current political debate about prescription drug costs and the slow pace of federal drug pricing legislation has spurred states into action. The negative impact COVID-19 is having on state budgets has added urgency to cut spending. Many states have pursued a variety of policies to manage Medicaid drug spending, including two states – Massachusetts and Tennessee – that have sought specific federal permission to not cover accelerated approval drugs under Medicaid.

This approach undercuts the Food and Drug Cosmetic Act and the Medicaid Drug Rebate Program (MDRP), through which Congress sought to provide rapid access to drugs likely to help patients with serious, often fatal, medical conditions and to provide Medicaid beneficiaries coverage to drugs for which companies provide a deep discount. Massachusetts and Tennessee have requested CMS waivers that would have allowed each state to not cover drugs approved by the FDA's accelerated approval pathway, but remain eligible to receive the rebates required under the MDRP.

In support of its request to CMS, TennCare, Tennessee's Medicaid program, argued that it be allowed the flexibility to exclude accelerated approval medicines to "avoid exorbitant spending on high-cost drugs that are not medically necessary, which do not provide additional clinical benefit, and/or which actually pose health risks for members when prescribed without sufficient medical evidence…".[6] MassHealth, Massachusetts' Medicaid program sought to exclude from its closed formulary drugs with "limited or inadequate clinical efficacy," defined to include drugs where "only surrogate endpoints have been reported."[12] CMS ultimately denied Massachusetts' request but expressed a willingness to consider a request from Massachusetts to exclude coverage if they would be willing to forego the mandatory rebates for all drugs and negotiate with individual drug companies. Tennessee's Medicaid waiver request[6] that could include coverage restrictions for accelerated approval medicines was approved by the Trump Administration in January 2021.[13] Its future under the Biden Administration is uncertain. A Kaiser Family Foundation Issue Brief described the potential patient impact: "Because seniors and people with disabilities often need complex acute and long-term care services, which are high

cost and unavailable through other coverage sources, these groups may be particularly vulnerable to financing models that incentivize lowering costs to realize savings."[14]

The National Governors Association's 2019 Principles for Federal Health Care Action to Address Health Care Costs included formulary exclusion or additional rebates for accelerated approval drugs in their state flexibility agenda.[15] The analysis on drivers of Medicaid spending data, however, shows that accelerated approval drugs have a *de minimus* impact on spending while addressing significant unmet medical needs.

Drugs approved via accelerated approval are typically intended for small populations of patients who lack other options and, as such, are costly. However, their total costs represent less than one percent of total Medicaid spending, and in the most recent period, only accounted for 0.1 percent of the overall increase in Medicaid spending. In fact, the portion of overall Medicaid spending accounted for by accelerated approval drugs has remained below 1 percent and essentially flat for more than a decade. The medicines are neither a primary driver of state Medicaid spending or spending growth, nor an effective target for reform.

Indeed, the Congressional Budget Office (CBO) recently evaluated the savings potential of a proposal by the Medicaid and CHIP Payment and Access Commission (MACPAC) to "give states a set period of time to evaluate the clinical evidence for new drugs and determine appropriate coverage criteria." 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.[16] In FY2018, federal spending on Medicaid amounted to 62 percent[17] of total Medicaid spending, so the state share of savings over the 10 year window as estimated by the CBO would be less in total 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 proposed six-month delay in access to treatment could have a profound impact on patients.

The Medicaid Drug Rebate Program (MDRP) generates rebates that reduce Medicaid drug costs by more than half.[18] By comparison, rebates in the Medicare Part D program, which allows payors significant discretion in establishing formularies, are less than 25 percent.[19] Net prices for specialty drugs in Medicare Part D are much higher than in Medicaid, because manufacturer rebates are substantially lower for Medicare.[20] Allowing 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.

Nevertheless, the pressures on state and federal budgets resulting from the COVID-19 pandemic will increase states' demand for drug pricing reform, including MDRP flexibility for Medicaid formularies. To maintain the ability of this important FDA approval pathway to offer patients quicker access to medicines for serious or life-threatening medical conditions, the FDA should use its existing authority

to enforce the conditions of accelerated approval and work with manufacturers to increase the rigor of interactions and monitoring from study design through completion of post-approval confirmatory studies. Collection and consideration of real world evidence in supporting post-approval commitments would boost understanding of drug effectiveness within real-world settings while potentially addressing some uncertainties held by payors. By exercising its existing post-approval authority, the FDA could help to bolster confidence in the accelerated approval program and prevent payers from undermining FDA authority by raising doubts as to its clinical standards required to demonstrate safety and efficacy.

Limitations

The lack of 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. 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 percent per year. Though spending on all categories of care increased during Medicaid expansion, the percentage of total Medicaid spending attributable to accelerated approval drugs remained stable from 2010 to 2018.

CONCLUSION

Medicaid programs looking for ways to lower drug costs will not find success in severely restricting coverage or eliminating coverage altogether for accelerated approval drugs. This analysis of Medicaid claims data from 2007 to 2018 shows that such efforts and the rationale used are misguided. Accelerated approval drugs account for less than one percent of overall Medicaid spending, while representing the only treatment options available for beneficiaries with serious or life-threatening conditions. During the COVID-19 pandemic, in particular, people living with serious conditions face increased vulnerability. This situation places paramount importance on facilitating access to treatments for serious or life-threatening conditions. Greater enforcement of FDA authority on confirmatory studies could reinforce confidence in the rigor of the accelerated approval pathway. Limiting Medicaid coverage for accelerated approval drugs, however, would appear to have a *de minimus* impact on spending and a devastating impact on patients benefiting from these treatments. Though the analysis is limited to Medicaid, the conclusions on the limited spending impact associated with accelerated approval drugs should inform coverage decisions for Medicare and private payers.

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