The Role of Accelerated Approval Drug Spending on Medicaid Spending to Treat Rare Diseases, 2017-2022

EVERYLIFE FOUNDATION FOR RARE DISEASES

Abstract

Why research this

The accelerated approval pathway expedites the Food and Drug Administration's (FDA's) rigorous review of safety and efficacy to facilitate access for patients whose lives depend on timely access to therapies. In the 30 years since the pathway's inception, the FDA has approved more than 250 drugs or biologics via the accelerated approval pathway, providing new, innovative treatment options, and hope for patients and their families.[1]

Despite meeting the same clinical safety and efficacy standards, both private and public payers, including state health plans, have taken steps to avoid or severely restrict coverage of accelerated approval medicines due to the incorrect assumption that these therapies are experimental and too costly for state Medicaid programs. This assumption directly undermines the purpose of the pathway and federal patient protections required of Medicaid, severely limiting patient access to necessary life-altering medications.[2]

Exploring the spending impact of rare disease accelerated approval therapies on state Medicaid programs will help state Medicaid Directors make informed decisions about accelerated approval coverage policies and allow advocates to better champion policies that support access to innovative treatments.

What did we do

To explore the cost impacts of non-oncology rare disease drug spending state Medicaid programs, we examined pre-rebate state spending and changes in spending on the medications overall and as a percent of total state Medicaid spending in 2017 and 2022, using public data on state Medicaid spending.[3] From these data, we narrowed the spending based on a list of non-oncology, non-infectious disease rare disease accelerated approvals from FDA's drug approval database.

What did we find

This study found that accelerated approval therapies are neither a primary driver of state Medicaid spending or spending growth, nor an effective target for reform. Notably, we found that in pre-rebate spending:

- Adjusting for inflation, total spending on accelerated approval drugs to treat rare disease increased slightly between 2017 and 2022, from $1.77 billion to $1.86 billion, a 5.2% increase. However, inflation adjusted spending on non-accelerated approval drugs increased sharply from $69.5 billion to $83.8 billion in 2022, a 20.7% increase, 4 times higher than the accelerated approval drugs.
- Across all jurisdictions, spending on accelerated approval drugs to treat non-oncology rare diseases accounted for only 0.67% of state Medicaid spending in 2017 and 0.84% in 2022.
- In 2022, the total range of spending was 0.09% in Washington DC, to 2.05% in Indiana.

How to use this information

This evidence can support advocates in their outreach with state Medicaid programs, as they advocate for coverage determination policies that support access to accelerated approval therapies. This might be discussing changes in spending on non-oncology rare diseases in your state in a comment letter or testimony before a DUR or P&T Committee, in comments on proposed Medicaid waiver applications or in outreach to state legislators as they consider policies that impact access to life-saving medication.

Key Takeaways

Across all states, pre-rebate spending on accelerated approval drugs to treat non-oncology rare diseases accounted for only 0.67% of state Medicaid spending in 2017 and 0.84% in 2022.

Adjusting for inflation, total spending on accelerated approval drugs to treat rare diseases increased slightly between 2017 and 2022, from $1.77 billion to $1.86 billion, a 5.2% increase. This is compared to a 20.7% increase in spending for non-accelerated approval drugs in the same period.

These medicines are neither a primary driver of state Medicaid spending or spending growth, nor an effective target for reform.

Total spending increase between 2017 and 2022

5.2% accelerated approval drugs

20.7% non-accelerated approval drugs

Percentage of state Medicaid spending
on accelerated approval drugs to treat non-oncology rare diseases

0.67% 2017

0.84% 2022

[1]Accessed on March 3, 2023 at https://www.fda.gov/media/151146/download

[2]FDA Guidance for Industry: Expedited Programs for Serious Conditions—Drugs and Biologics. May 2014. Available from: https://www.fda.gov/files/drugs/published/Expedited-Programs-for-Serious-ConditionsDrugs-and-Biologics.pdf.

[3]*state reported drug utilization for covered outpatient drugs paid for by state Medicaid agencies data downloaded from https://www.medicaid.gov/medicaid/prescription-drugs/state-drug-utilization-data/index.html

Background

Created in 1992 to facilitate expedited access to potentially life-saving products for patients whose diseases warranted a novel approach to regulatory review, the accelerated approval pathway expedites the Food and Drug Administration's (FDA's) rigorous review of safety and efficacy to facilitate access for patients whose lives depend on timely access to therapies. In the 30 years since the pathway's inception, the FDA has approved more than 250 drugs or biologics via the accelerated approval pathway, providing new, innovative treatment options, and hope for patients and their families.[4] Millions of patients have benefitted significantly from therapies approved through accelerated approval (AA)–initially people diagnosed with HIV/AIDS, then people with various cancers, and today people with several rare diseases, many of whom are children. These medicines are reviewed and approved using the FDA's same rigorous scientific review standards as with traditional approval. These standards are just applied to earlier endpoints to expedite access for seriously ill patients, creating hope and opportunity for these patients and their families.

These medicines (accelerated approval) are reviewed and approved using the FDA's same rigorous scientific review standards as with traditional approval.

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.[5] Studies demonstrating a drug's effect on a surrogate or intermediate clinical endpoint must meet standards demonstrating the study is "adequate and well controlled." The FDA created the accelerated approval pathway 30 years ago in response to the HIV/AIDS epidemic where the surrogate endpoint of immune cell response was used to predict survival. By not waiting years to confirm survival as an outcome, people with HIV/AIDS gained earlier access to FDA-approved therapies. Using a surrogate endpoint reasonably likely to predict long-term clinical benefits, which could 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 1992 and then modernized and enhanced it in 2012 to expand the pathway's use beyond treatments for HIV/AIDS or oncology to include rare diseases[6], leading to the approval of over 250 novel therapies.[7, 8, 9]

The FDA created the accelerated approval pathway 30 years ago in response to the HIV/AIDS epidemic where the surrogate endpoint of immune cell response was used to predict survival. Congress first codified the accelerated approval pathway in 1992 and then modernized and enhanced it in 2012 to expand the pathway's use beyond treatments for HIV/AIDS or oncology to include rare diseases[6], leading to the approval of over 250 novel therapies.

The accelerated approval process has come under criticism from some public and private insurers who have sought to exclude paying for medications approved through this process. For example, Oregon had submitted a waiver to the Centers for Medicare and Medicaid Services (CMS) to do so but that component of the application was ultimately dropped following community input. At least one private insurance company has issued a coverage policy that would deny coverage for non-oncology therapies approved using the accelerated approval pathway for at least 18 months. Efforts to restrict access to therapies approved using the accelerated approval pathway do not target the significant drivers of the rare disease economic impact. The 2022 National Economic Burden of Rare Disease Study in the U.S found the overall economic impact of rare diseases in 2019 exceeded $966 billion.[10] Of the costs assessed, non-medical and indirect costs accounted for nearly 60% of overall costs underscoring that significant costs are shouldered by families.[11] Of the $449 billion assessed in direct medical costs, hospital inpatient care was the largest cost category representing 32% of the direct costs, prescription medications represented 18% of direct medical costs, however this 18% reflects all treatments associated with rare diseases (approved both through accelerated approval and the traditional FDA process), of which only a fragment have been approved using accelerated approval.[12] This breakdown reflects the reality that 95% of rare diseases still lack an FDA-approved treatment.[13]

[4]Accessed on March 3, 2023 at https://www.fda.gov/media/151146/download

[5]FDA. Accelerated Approval. Jan. 4, 2018. Available from: https://www.fda.gov/patients/fast-trackbreakthrough-therapy-accelerated-approval-priority-review/accelerated-approval.

[6]Accessed on March 3, 2023 at https://www.fda.gov/media/151146/download

[7]Food and Drug Administration Regulatory Modernization Act of 1997.

[8]Food and Drug Administration Safety and Innovation Act of 2012 (FDASIA)

[9]Accessed on March 3, 2023 at https://www.fda.gov/media/151146/download

[10]Grace Yang, Inna Cintina, Anne Pariser, Elisabeth Oehrlein, Jamie Sullivan, Annie Kennedy. The national economic burden of rare disease in the United States in 2019, Orphanet Journal of Rare Diseases, April 2022

[11]Grace Yang, Inna Cintina, Anne Pariser, Elisabeth Oehrlein, Jamie Sullivan, Annie Kennedy. The national economic burden of rare disease in the United States in 2019, Orphanet Journal of Rare Diseases, April 2022

[12]Grace Yang, Inna Cintina, Anne Pariser, Elisabeth Oehrlein, Jamie Sullivan, Annie Kennedy. The national economic burden of rare disease in the United States in 2019, Orphanet Journal of Rare Diseases, April 2022

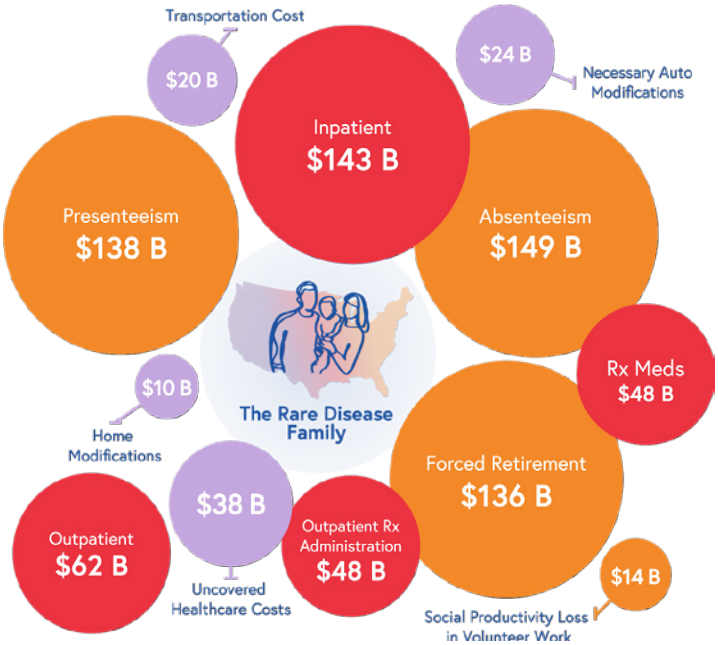
[13]National Center for Advancing Translational Sciences. (2023, February 24). Delivering Hope for Rare Diseases. https://ncats.nih.gov/sites/default/files/NCATS_RareDiseasesFactSheet.pdf

What is the Impact on the Average Rare Disease Family?

$418 Billion
DIRECT MEDICAL COSTS

$437 Billion
INDIRECT COSTS: PRODUCTIVITY LOSS

$111 Billion
NON-MEDICAL & UNCOVERED HEALTHCARE COSTS



Total Economic Burden of Rare Disease in the U.S in 2019:
$966 Billion

Estimated prevalence of 379 RDs
15.5 million

Among the drugs targeted for exclusion or include rare and orphan disease drugs. These drugs are generally defined as treatments for medical conditions that affect fewer than 200,000 patients in the US[14]. To address the "high costs" of accelerated approval drugs, a technical advisory committee to Congress on Medicaid has proposed requiring higher rebates on the drugs until confirmatory studies were completed.[15] Other efforts and proposals have emerged that would further differentiate reimbursement and access to drugs approved via the accelerated approval pathway.

These proposals often pose restricting coverage for accelerated approval therapies based on misconceptions of "limited efficacy" and proposes undermining the authority of the FDA by relaying decisions of safety and efficacy to the state, despite 98% of the first 105 accelerated approval therapies being converted or withdrawn by 2022.[16]

Among the drugs targeted for exclusion from state Medicaid programs or higher rebates include rare and orphan disease drugs.

Data and Methods

To examine the role that non-oncology rare disease drug spending assumes in state Medicaid programs, we examined state spending and changes in spending on the medications overall and as a percent of total state Medicaid spending in 2017 and 2022. We use public data on state Medicaid spending.[17] From these data we identified a list of accelerated approvals that were identified as non-oncology, non-infectious disease rare disease drug approvals. The data reported for drug spending are before any rebates. Because most accelerated approval drugs convert to traditional approval[18], the analysis considers conversion status and categorizes the spending contribution from an accelerated approval drug to prescription drugs generally (non-accelerated drugs in our tables) once a drug completed confirmatory trials and FDA changed its approval status to traditional approval. Total state Medicaid spending includes drug spending that is post-rebate. As a result, the results reported are an upper estimate of the role this subset of rare drug spending assumes as a share of total state Medicaid spending.

[14] 21CFR Part 316 - Orphan Drugs

[15] Accessed on March 1 at https://www.macpac.gov/publication/high-cost-specialty-drugs-moving-towards-recommendations/

[16] Domike, R., Raju, G.K., Sullivan, J. et al. Expediting treatments in the 21st century: orphan drugs and accelerated approvals. Orphanet J Rare Dis 19, 418 (2024). https://doi.org/10.1186/s13023-024-03398-1

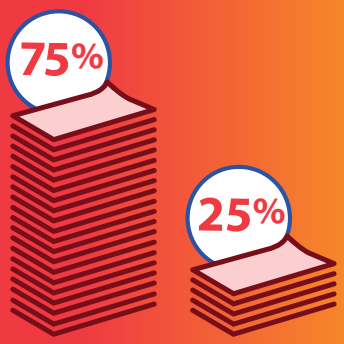
[17] State reported drug utilization for covered outpatient drugs paid for by state Medicaid agencies data downloaded from https://www.medicaid.gov/medicaid/prescription-drugs/state-drug-utilization-data/index.html

[18] Domike, R., Raju, G.K., Sullivan, J. et al. Expediting treatments in the 21st century: orphan drugs and accelerated approvals. Orphanet J Rare Dis 19, 418 (2024). https://doi.org/10.1186/s13023-024-03398-1

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 2022. As of the end of 2022, no orphan designated biological product had yet been approved via the accelerated approval pathway. The list of the rare disease medications included in the analysis to treat non-oncology, non-infectious disease rare diseases is listed in Appendix 1. These data informed calculations of the Medicaid amount reimbursed for each year. Total Medicaid drug reimbursements for the year were also tabulated.[19] Total state Medicaid spending was collected from state financial management reports for 2017 and 2022.[20]

Less than 10% of all products approved via the AA pathway are indicated for non-oncology rare diseases

More than 75% of all products approved via the AA pathway **have been converted to traditional approvals**, with many more still in the process of collecting data to support a full approval.



Results

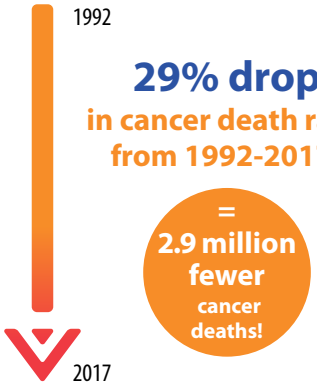
We present three sets of tables. Table 1 tabulates by state (and the District of Columbia (DC)) total accelerated approval drug spending on medications for rare diseases, total non-accelerated approval drugs and accelerated approval spending as a percent of total state Medicaid drug spending. Table 1 reports these in nominal dollars. Table 2 presents the same tabulation except the dollars are adjusted for inflation and reported as spending in 2022 dollars (using the consumer price index-urban). Finally, Table 3 presents the nominal spending on accelerated approval rare disease drugs as a percent of total state Medicaid spending.

1 *As reported in Table 1* the range of spending on the analyzed accelerated approval rare disease treatment drugs as a percent of total drug spending was 0.7 percent in the District of Columbia to a high of 4.1 percent of total drug spending in Alabama in 2017. The average across the 51 states and the District of Columbia was 2.2 percent of total drug spending in 2017. The results are nearly identical in 2022 though lower at 1.9 percent of total drug spending. Accelerated approval rare disease drugs ranged from a low of 0.35 percent of drug spending in Rhode Island and Vermont and even lower in the District of Columbia (0.32 percent) to a high of 5.1 percent in Kansas in 2022.

2 *Adjusting for inflation in Table 2,* total spending on accelerated approval drugs to treat rare disease increased slightly between 2017 and 2022, from $1.77 billion to $1.86 billion, a 5.2 percent increase. However, inflation adjusted spending on non-accelerated approval drugs increased sharply from $69.5 billion to $83.8 billion in 2022, a 20.7 percent increase, 4 times higher than the accelerated approval drugs.

3 *Table 3 presents* tabulations of the share of analyzed accelerated approval drug spending of total state Medicaid spending. On average nationally, pre-rebate spending on accelerated approval drugs to treat non-oncology rare diseases accounted for only 0.67 percent of state Medicaid spending in 2017 and 0.84 percent in 2022. In 2017 this ranged from a low of 0.008 percent in Delaware to a high of 1.7 percent in Indiana. The results were nearly identical in 2022. Accelerated approval drug spending to treat non-oncology, non-infectious disease rare diseases as a share of total state Medicaid spending ranged from a low of 0.09 percent in the District of Columbia to a high of 2.05 percent in Indiana in 2022. **Across all jurisdictions, the average percent of the state Medicaid budget was similar in 2022 as 2017, 0.84 percent.** Accounting for rebates would reduce those contributions to state Medicaid spending even further.

Accelerated Approval Works



Inflation adjusted spending on non-accelerated approval therapies yields a 4 times higher spending than accelerated approval therapies from 2017-2022

[19] data downloaded from https://www.medicaid.gov/medicaid/prescription-drugs/state-drug-utilization-data/index.html

[20] These data are available from: https://www.macpac.gov/wp-content/uploads/2022/12/MACSTATS_Dec2022_WEB-508.pdf and https://www.macpac.gov/wp-content/uploads/2018/12/December-2018-MACStats-Data-Book.pdf

Conclusion

These medicines are neither a primary driver of state Medicaid spending or spending growth, nor an effective target for reform. Meaningful policy solutions must focus on the total cost of patient care. Policy reforms that focus on only one aspect of healthcare costs and that threaten access to the few treatment options available to those with rare diseases will result in negative repercussions for patient health and have detrimental economic outcomes for families and society overall.

During the EveryLife Foundation's Annual Scientific Workshop in December 2021, rare disease scientific, clinical, patient and regulatory experts examined the use of the accelerated approval pathway in rare disease to identify common challenges and opportunities for optimizing the pathway to transform rare disease care in the way that is has been transformational for HIV/AIDS and oncology.

Based on this workshop and subsequent studies, we urge policymakers to consider engaging in efforts that ensure the optimization of the accelerated approval pathway and its ability to transform rare disease outcomes, while applying the relevant principles suggested within the Rare Disease Community Statement on Drug Pricing Policy Priorities for all future decisions. These actions include;

- The need for payer entities such as Medicaid programs to engage early and often with patient, scientific, clinical and regulatory stakeholders to develop the knowledge and evidence available for rare disease biomarkers. As payers engage early in the process, the understanding of the biomarkers being used and the rationale for their use will produce outcomes that payers can meaningfully incorporate into their decision-making process.
- Create early, timely, and transparent processes for patients to engage with payors in their coverage determinations practices, to allow for robust considerations for patient experience data and real world experiences.
- Reject policies that treat all medications approved using the accelerated approval pathway as experimental, thus exacerbating health inequality among communities eligible for life-saving medications.
- Support the robust collection of real-world outcomes to enhance the evidence for therapies approved via the accelerated approval pathway in the post-market setting.
- Seek new and alternative payment models, including outcomes-based contracting, that allow rare disease patients to have access to novel therapies and ensure reimbursement policies encourage development of future disease modifying therapies.
- Exclude rare disease therapies from policy experiments or demonstrations, until or unless it is proven that the changes do not threaten patient access and medical innovation.

Quite clearly, accelerated approval medications for non-oncology drugs to treat rare diseases are not contributing to the level and growth in Medicaid spending.

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EVERYLIFE FOUNDATION FOR RARE DISEASES

About the EveryLife Foundation

The EveryLife Foundation for Rare Diseases is a 501(c)(3) nonprofit, nonpartisan organization dedicated to empowering the rare disease patient community to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments and cures. We do not speak for patients. We provide the training, education, resources and opportunities to make their voices heard. By activating the patient advocate, we can change public policy and save lives.
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Table 1. Rare Drug State Medicaid Spending as Percent of Total Drug Spending, Nominal Dollars, 2017-2022

	2017			2022		
State	Medicaid Non-Accelerated Rx	Medicaid Accelerated Rx	% of State Medicaid	Medicaid Non-Accelerated Rx	Medicaid Accelerated Rx	% of State Medicaid
AK	133,047,085	2,071,263	1.53%	203,482,175	3,120,549	1.51%
AL	669,811,723	28,672,277	4.10%	974,574,474	21,235,212	2.13%
AR	376,024,134	7,474,452	1.95%	494,761,814	7,503,590	1.49%
AZ	1,199,589,546	26,663,237	2.17%	1,726,813,636	50,151,045	2.82%
CA	7,851,172,807	202,694,746	2.52%	11,050,383,098	288,718,646	2.55%
CO	895,415,128	14,625,793	1.61%	1,389,020,919	15,621,678	1.11%
CT	1,196,547,790	15,038,496	1.24%	1,721,193,087	16,732,858	0.96%
DC	267,611,312	1,854,833	0.69%	240,076,443	770,392	0.32%
DE	26,340,185	68,710	0.26%	266,416,852	5,368,026	1.98%
FL	2,807,672,222	106,647,670	3.66%	3,593,360,159	126,208,612	3.39%
GA	1,108,304,832	44,032,049	3.82%	1,342,163,340	37,201,625	2.70%
HI	200,136,478	3,549,394	1.74%	215,265,723	3,481,418	1.59%
IA	352,810,698	11,340,548	3.11%	741,374,072	14,635,626	1.94%
ID	187,817,844	3,297,585	1.73%	532,505,781	10,332,580	1.90%
IL	1,916,623,108	56,194,616	2.85%	2,888,829,014	71,208,000	2.41%
IN	1,467,177,308	55,933,307	3.67%	2,380,027,693	81,934,472	3.33%
KS	354,920,288	9,814,519	2.69%	265,320,232	14,287,414	5.11%
KY	1,226,985,481	14,363,129	1.16%	1,993,529,599	22,454,729	1.11%
LA	1,029,566,746	22,834,910	2.17%	2,396,713,286	35,724,781	1.47%
MA	1,410,629,442	22,185,253	1.55%	2,127,942,062	28,777,999	1.33%
MD	1,107,957,349	17,510,285	1.56%	1,606,950,641	26,324,313	1.61%
ME	217,306,989	2,756,973	1.25%	452,294,704	6,257,270	1.36%
MI	2,150,832,930	50,171,219	2.28%	3,223,190,796	58,042,022	1.77%
MN	951,342,053	28,544,858	2.91%	1,319,090,214	36,312,740	2.68%
MO	1,200,549,758	25,974,522	2.12%	1,484,972,316	38,580,583	2.53%
MS	427,032,050	15,119,517	3.42%	408,158,185	8,318,943	2.00%
MT	201,531,881	5,701,541	2.75%	394,794,601	7,342,098	1.83%
NC	1,740,250,668	40,548,107	2.28%	2,330,222,475	49,740,291	2.09%
ND	51,330,931	811,167	1.56%	97,583,203	509,095	0.52%
NE	175,205,042	2,409,900	1.36%	407,910,324	9,181,513	2.20%
NH	99,189,168	1,155,423	1.15%	252,367,553	6,534,634	2.52%
NJ	1,404,537,336	37,036,092	2.57%	1,631,943,418	42,017,599	2.51%
NM	429,330,157	5,500,046	1.26%	506,186,137	8,597,409	1.67%
NV	473,843,553	13,139,151	2.70%	559,395,326	14,216,542	2.48%
NY	5,829,268,514	144,150,306	2.41%	6,764,122,830	150,821,061	2.18%
OH	3,402,230,358	86,828,604	2.49%	4,176,140,791	103,453,544	2.42%
OK	482,998,932	17,148,816	3.43%	856,691,371	15,744,203	1.80%
OR	692,460,900	15,425,390	2.18%	788,742,202	14,385,851	1.79%
PA	3,773,927,564	90,008,007	2.33%	3,508,116,320	72,142,517	2.02%
RI	216,309,688	3,031,424	1.38%	339,266,810	1,175,048	0.35%
SC	526,662,256	21,495,162	3.92%	703,446,267	20,427,021	2.82%
SD	98,848,284	3,403,779	3.33%	156,941,497	6,293,466	3.86%
TN	1,020,077,285	25,741,369	2.46%	1,437,957,588	37,512,074	2.54%

	2017			2022		
State	Medicaid Non-Accelerated Rx	Medicaid Accelerated Rx	% of State Medicaid	Medicaid Non-Accelerated Rx	Medicaid Accelerated Rx	% of State Medicaid
TX	3,288,329,001	104,535,086	3.08%	3,793,302,258	147,947,276	3.75%
UT	190,228,381	2,136,774	1.11%	477,031,534	9,263,434	1.90%
VA	920,439,229	24,277,717	2.57%	5,077,628,513	68,847,989	1.34%
VT	159,336,717	2,205,689	1.37%	194,264,134	673,315	0.35%
WA	1,133,992,690	27,652,117	2.38%	1,510,240,192	20,737,221	1.35%
WI	1,143,138,974	22,556,271	1.94%	1,929,066,883	15,537,415	0.80%
WV	583,436,427	9,040,465	1.53%	849,891,110	9,347,537	1.09%
WY	35,128,447	848,914	2.36%	45,588,404	792,842	1.71%
	58,192,270,885	**1,481,601,770**	**2.2%**	**75,788,508,629**	**1,624,480,152**	**1.90%**

* State reported drug utilization for covered outpatient drugs paid for by state Medicaid agencies. Data downloaded from https://www.medicaid.gov/medicaid/prescription-drugs/state-drug-utilization-data/index.html

**Accelerated Approval and conversion dates from AA Orphan approval list 1.11.23.xlsx Inflation adjusted using GDP price deflator

Table 2. Accelerated Approval Drugs to Treat Rare Diseases as Percent Total State Drug Spending, 2017-2022 (inflation adjusted)

	2017			2022		
State	Medicaid Non-Accelerated Rx	Medicaid Accelerated Rx	% of State Medicaid	Medicaid Non-Accelerated Rx	Medicaid Accelerated Rx	% of State Medicaid
AK	158,848,296	2,472,933	1.53%	203,482,175	3,120,549	1.51%
AL	799,705,246	34,232,561	4.10%	974,574,474	21,235,212	2.13%
AR	448,944,774	8,923,938	1.95%	494,761,814	7,503,590	1.49%
AZ	1,432,220,456	31,833,917	2.17%	1,726,813,636	50,151,045	2.82%
CA	9,373,714,818	242,002,410	2.52%	11,050,383,098	288,718,646	2.55%
CO	1,069,058,885	17,462,106	1.61%	1,389,020,919	15,621,678	1.11%
CT	1,428,588,828	17,954,842	1.24%	1,721,193,087	16,732,858	0.96%
DC	319,507,950	2,214,532	0.69%	240,076,443	770,392	0.32%
DE	31,448,217	82,035	0.26%	266,416,852	5,368,026	1.98%
FL	3,352,151,245	127,329,365	3.66%	3,593,360,159	126,208,612	3.39%
GA	1,323,233,317	52,570,982	3.82%	1,342,163,340	37,201,625	2.70%
HI	238,948,030	4,237,711	1.74%	215,265,723	3,481,418	1.59%
IA	421,229,662	13,539,768	3.11%	741,374,072	14,635,626	1.94%
ID	224,240,500	3,937,071	1.73%	532,505,781	10,332,580	1.90%
IL	2,288,305,053	67,092,181	2.85%	2,888,829,014	71,208,000	2.41%
IN	1,751,700,288	66,780,198	3.67%	2,380,027,693	81,934,472	3.33%
KS	423,748,356	11,717,803	2.69%	265,320,232	14,287,414	5.11%
KY	1,464,929,162	17,148,505	1.16%	1,993,529,599	22,454,729	1.11%
LA	1,229,225,918	27,263,180	2.17%	2,396,713,286	35,724,781	1.47%
MA	1,684,186,355	26,487,538	1.55%	2,127,942,062	28,777,999	1.33%
MD	1,322,818,449	20,905,975	1.56%	1,606,950,641	26,324,313	1.61%
ME	259,448,339	3,291,620	1.25%	452,294,704	6,257,270	1.36%
MI	2,567,934,121	59,900,694	2.28%	3,223,190,796	58,042,022	1.77%
MN	1,135,831,465	34,080,432	2.91%	1,319,090,214	36,312,740	2.68%
MO	1,433,366,878	31,011,642	2.12%	1,484,972,316	38,580,583	2.53%
MS	509,844,422	18,051,575	3.42%	408,158,185	8,318,943	2.00%
MT	240,614,036	6,807,215	2.75%	394,794,601	7,342,098	1.83%
NC	2,077,729,519	48,411,416	2.28%	2,330,222,475	49,740,291	2.09%
ND	61,285,303	968,473	1.56%	97,583,203	509,095	0.52%
NE	209,181,754	2,877,241	1.36%	407,910,324	9,181,513	2.20%
NH	118,424,469	1,379,489	1.15%	252,367,553	6,534,634	2.52%
NJ	1,676,912,833	44,218,332	2.57%	1,631,943,418	42,017,599	2.51%
NM	512,588,191	6,566,644	1.26%	506,186,137	8,597,409	1.67%
NV	565,733,865	15,687,166	2.70%	559,395,326	14,216,542	2.48%
NY	6,959,711,885	172,104,715	2.41%	6,764,122,830	150,821,061	2.18%
OH	4,062,009,326	103,666,878	2.49%	4,176,140,791	103,453,544	2.42%
OK	576,664,705	20,474,408	3.43%	856,691,371	15,744,203	1.80%
OR	826,746,676	18,416,765	2.18%	788,742,202	14,385,851	1.79%
PA	3,773,927,564	90,008,007	2.33%	3,508,116,320	72,142,517	2.02%
RI	258,257,635	3,619,294	1.38%	339,266,810	1,175,048	0.35%
SC	628,795,457	25,663,621	3.92%	703,446,267	20,427,021	2.82%
SD	118,017,480	4,063,858	3.33%	156,941,497	6,293,466	3.86%
TN	1,217,896,205	30,733,275	2.46%	1,437,957,588	37,512,074	2.54%

	2017			2022		
State	Medicaid Non-Accelerated Rx	Medicaid Accelerated Rx	% of State Medicaid	Medicaid Non-Accelerated Rx	Medicaid Accelerated Rx	% of State Medicaid
TX	3,926,019,598	124,807,098	3.08%	3,793,302,258	147,947,276	3.75%
UT	227,118,500	2,551,149	1.11%	477,031,534	9,263,434	1.90%
VA	1,098,935,797	28,985,784	2.57%	5,077,628,513	68,847,989	1.34%
VT	190,236,158	2,633,428	1.37%	194,264,134	673,315	0.35%
WA	1,353,902,704	33,014,565	2.38%	1,510,240,192	20,737,221	1.35%
WI	1,364,822,684	26,930,505	1.94%	1,929,066,883	15,537,415	0.80%
WV	696,579,583	10,793,641	1.53%	849,891,110	9,347,537	1.09%
WY	41,940,746	1,013,540	2.36%	45,588,404	792,842	1.71%
TOTAL	**$69,477,231,703**	**$1,768,922,021**		**$83,827,252,056**	**$1,862,546,188**	

Table 3. Accelerated Approval Drug Spending to Treat Rare Disease as Percent total State Medicaid Spending, 2017-2022

Note: In calculating percentage of spending, Total Medicaid Spending (denominator) includes drug rebates, but the spending on rare disease AA drugs (numerator) does not include rebates as the information is not available.

	2017	2022
	Rare Drug Accelerated Approval Spending as Percent State Medicaid Spending	
AK	0.321	0.532
AL	1.655	1.346
AR	0.483	0.451
AZ	0.909	1.453
CA	0.562	0.715
CO	0.424	0.379
CT	0.464	0.476
DC	0.236	0.091
DE	0.008	0.583
FL	1.148	1.180
GA	1.295	0.960
HI	0.424	0.388
IA	0.724	0.831
ID	0.593	1.492
IL	0.919	0.781
IN	1.71	2.048
KS	0.667	1.000
KY	0.654	0.891
LA	0.689	1.187
MA	0.272	0.363
MD	0.385	0.536
ME	0.288	0.651
MI	0.614	1.127
MN	0.554	0.630
MO	0.675	1.341
MS	1.043	0.877
MT	1.485	1.648
NC	0.881	1.032
ND	0.161	0.106
NE	0.236	0.916
NH	0.133	0.719
NJ	0.394	0.597
NM	0.473	0.672
NV	1.396	1.225
NY	0.36	0.516
OH	1.156	1.425
OK	0.924	1.250
OR	0.667	0.454
PA	0.665	0.536
RI	0.27	0.110
SC	1.193	1.135
SD	0.917	1.626
TN	0.765	1.177
TX	0.65	0.818
UT	0.274	0.955
VA	0.523	1.200
VT	0.314	0.106
WA	0.6	0.377
WI	0.65	0.428
WV	1.027	1.098
WY	0.278	0.282
Average	**0.67%**	**0.84%**

Appendix 1. List of Accelerated Approval Drugs to Treat Rare Non-Oncology, Non-Infectious Disease Conditions

DEFERASIROX	NOVARTIS PHARMACEUTICALS	1/23/2013	NON-TRANSFUSION DEPENDENT THALASSEMIA (NTDT)
IDURSULFASE	SHIRE HUMAN GENETIC THERAPIES	6/24/2013	HUNTER SYNDROME
DEFERASIROX	NOVARTIS PHARMACEUTICALS	3/30/2015	NON-TRANSFUSION-DEPENDENT THALASSEMIA (NTDT) SYNDROMES
DEFERIPRONE	APOPHARMA	9/9/2015	IRON OVERLOAD DUE TO THALASSEMIA
ETEPLIRSEN	SAREPTA THERAPEUTICS	9/19/2016	DUCHENNE MUSCULAR DYSTROPHY
MIGALASTAT	AMICUS THERAPEUTICS	8/10/2018	FABRY DISEASE
AMIKACIN LIPOSOME INHALATION SUSPENSION	INSMED	9/28/2018	MYCOBACTERIUM AVIUM COMPLEX (MAC) LUNG DISEASE
VOXELOTOR	GLOBAL BLOOD THERAPEUTICS INC	11/25/2019	SICKLE CELL DISEASE
GOLODIRSEN	SAREPTA THERAPEUTICS INC	12/12/2019	DUCHENNE MUSCULAR DYSTROPHY (DMD)
DEFERIPRONE	CHIESI USA INC	5/19/2020	THALASSEMIA
VILTOLARSEN	NS PHARMA	8/12/2020	DUCHENNE MUSCULAR DYSTROPHY (DMD)
DARATUMUMAB AND HYALURONIDASE-FIHJ	JANSSEN	1/15/2021	LIGHT CHAIN (AL) AMYLOIDOSIS
VOSORITIDE	BIOMARIN	11/19/2021	ACHONDROPLASIA
BUDESONIDE	CALLIDITAS	12/15/2021	CROHN'S DISEASE
VOXELOTOR	GLOBAL BLOOD THERAPEUTICS INC	12/17/2021	SICKLE CELL DISEASE
CASIMERSEN	SAREPTA	2/25/2023	DUCHENNE MUSCULAR DYSTROPHY (DMD)