Policy in Brief

MACPAC

High-Cost Drugs and the Medicaid Program: MACPAC Evidence and Recommendations

Summary

While Medicaid drug spending is growing, it is increasingly driven by high-cost specialty drugs.

In fiscal year (FY) 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. In FY 2021, drugs over $1,000 per claim accounted for less than 2 percent of utilization but more than half of Medicaid spending. From 2018 to 2021, the average cost of a brand drug has increased almost 50 percent from $430.51 to $631.16, reflecting the introduction of new, high-cost specialty drugs (Table 1). In December 2023, the U.S. Food and Drug Administration (FDA) approved two cell-based gene therapies, Casgevy and Lyfgenia, for the treatment of sickle cell disease. These two therapies have prices of $2.2 and $3.1 million respectively for the course of treatment (Kolata 2023).

MACPAC's work has been focused on how to address states' concerns about the growing costs associated with specialty drugs, as well as how to ensure that beneficiaries who could benefit from these new therapies would still have access to them.

TABLE 1. Medicaid Gross Drug Spending, by Brand versus Generic Status, FYs 2018–2021

Fiscal year	Gross brand drug spending per claim	Gross generic drug spending per claim	Gross total drug spending per claim
2018	$430.51	$17.77	$83.76
2019	486.71	18.68	92.88
2020	553.38	19.91	100.75
2021	631.16	20.82	111.10

Notes: Includes federal and state funds. Gross expenditures are before the application of rebates. Does not include Medicare Part D clawback payments. To assign brand and generic status, we linked the state drug utilization data to the Medicaid drug product data from the Centers for Medicare & Medicaid Services using the National Drug Code, the universal product identifier for drugs.

Source: MACPAC, 2022, analysis of Medicaid state drug rebate utilization and product data as reported by states as of September 2022.

By the numbers...

In FY 2021

$80.6 billion

was spent on outpatient prescription drugs

$42.5 billion

was collected in rebates, equaling a total of

$38.1 billion

in net spending

Background

Under the Medicaid Drug Rebate Program (MDRP), drug manufacturers must provide Medicaid rebates, but in exchange, states must generally cover all FDA-approved drugs. This coverage requirement can limit states' ability to manage utilization and spending for high-cost drugs.

Additionally, an increasing number of high-cost drugs are being approved through the accelerated approval pathway on the basis of a surrogate endpoint that has not yet demonstrated a clinical benefit. States have concerns about having to cover and pay high prices for drugs that do not have a verified clinical benefit.

Key Points

- Many high-cost specialty drugs are likely to have lower rebates than other brand drugs. Many of these high-cost drugs launch at a high price but do not increase prices substantially over time, so they are likely to have lower inflationary rebates.
- Cell and gene therapies are a subset of specialty drugs that are receiving significant attention due to their high costs and potential as durable (i.e., having long-term benefit) or curable treatments. Many cell and gene therapies have list prices over $1 million for the course of treatment.
- A MACPAC pipeline analysis in 2020 identified 45 cell or gene therapies indicated for pediatric populations and 61 therapies indicated for adults in Phase III or later (e.g., a new drug application submitted).
- Because more than two out of every five children in the U.S. are Medicaid beneficiaries, high-cost pediatric products are of particular importance for Medicaid. While Medicaid is not likely to be the largest payer for gene and cell therapies indicated for adults, any use of these high-cost products may strain Medicaid budgets.
- The high up-front cost of cell and gene therapies can create significant budget volatility for states. Additionally, there are still uncertainties about whether these therapies will produce the long-term benefits anticipated by manufacturers. MACPAC discussed technical considerations for a new national drug benefit for cell and gene therapies that could address these issues and allow for new coverage, payment, or rebate requirements without disrupting the structure of the MDRP for all other outpatient drugs.
- The Center for Medicare and Medicaid Innovation is exploring a new Medicaid cell and gene therapy model to help states negotiate outcomes-based contracts with manufacturers that would provide states greater rebates should a therapy not achieve the expected outcomes over time. This model is expected to begin in 2025.

Reference

Kolata, G. 2023. F.D.A. approves sickle cell treatments, including one that uses CRISPR. *New York Times*, December 8. https://www.nytimes.com/2023/12/08/health/fda-sickle-cell-crispr.html.

Further reading

Addressing High-Cost Specialty Drugs

Strengthening Evidence under Medicaid Drug Coverage

Trends in Medicaid Drug Spending and Rebates

Priority Pipeline Specialty Drugs for Medicaid

Recommendations

MACPAC has examined the issue of how to balance states' concerns about the high cost of drugs without a verified clinical benefit with patient access to them. Increasing the Medicaid rebates on accelerated approval drugs until the clinical benefit has been verified strikes a balance between addressing state concerns about paying high prices for these products while maintaining access for beneficiaries. Additionally, allowing states to implement a Medicare coverage with evidence requirement can help develop additional evidence on the clinical benefits of a drug for populations prevalent in Medicaid. MACPAC's recommendations make no changes to the obligation to cover these drugs.

MACPAC has made the following recommendations related to high-cost drugs:

- (March 2023) The Commission recommends that Congress amend sections of the Social Security Act to allow states to exclude or restrict coverage of outpatient drugs based on coverage with evidence development requirements implemented under a Medicare national coverage determination. In addition, the Commission recommends that Congress amend to the Social Security Act to require that managed care contracts 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.
- (June 2021) The Commission recommends that Congress increase the minimum rebate percentage on drugs approved through the accelerated approval pathway until the manufacturer has verified the clinical benefit. The Commission also recommends an increase in the additional inflationary rebate on drugs that receive approval from the FDA through the accelerated approval pathway. Once the FDA grants traditional approval, the Medicaid rebates would revert back to the standard amounts.