Confronting State Medicaid Drug Spending Pressures

Across the country, state Medicaid budgets are under increasing strain, driven by factors including the high and increasing prices of prescription drugs. Nearly 30% of state spending supports Medicaid programs, and Medicaid programs spent more than $29 billion on prescription drugs in 2017 after accounting for manufacturer discounts. Perhaps most concerning is the increasing share of spending devoted to a few high-cost drugs. In 2017, drugs costing more than $1000 per claim represented just 1.2% of claims but accounted for 43.7% of all Medicaid drug spending, which is up from 31.1% in 2014.2

These problems will become even more challenging in the next few months because the coronavirus disease 2019 (COVID-19) pandemic has exacerbated state budget crises through lower tax revenues and higher Medicaid enrollment for newly uninsured individuals in the US. However, federal laws and regulations around drug coverage and pricing may have unintentionally contributed to strained Medicaid budgets and access challenges for patients.

One potentially large driver of drug spending pressure on state Medicaid budgets arises from the interaction between US Food and Drug Administration (FDA) regulations with the US Centers for Medicare & Medicaid Services (CMS) Medicaid Drug Rebate Program, which was established under federal law in 1990. The Medicaid Drug Rebate Program required state Medicaid programs that choose to cover prescription drugs (as all do today) essentially to cover all FDA-approved drugs, but it also required pharmaceutical companies to provide Medicaid with rebates for drugs sold under the program. Today, brand-name drug manufacturers must provide a minimum rebate of 23.1% of their drug’s average manufacturer price or the difference between the average manufacturer price and the lowest price given to any commercial payer, whichever is greater. This arrangement helped ensure that states could provide access to medically necessary drugs for their Medicaid beneficiaries, while also somewhat insulating the safety net program’s finances. Unlike other health insurers, Medicaid programs cannot control costs through strategies like steering beneficiaries to lower-cost drugs by requiring cost sharing for more expensive products.

Challenges with this arrangement that guaranteed coverage of all drugs quickly emerged. In 1990, FDA approval for a new drug required the completion of clinical trials demonstrating that the product was safe and effective for its intended use. But in 1992, largely in response to the HIV/AIDS crisis, the FDA created an accelerated approval program in which drugs intended for the treatment of “serious or life-threatening illnesses” aiming to provide “improved therapeutic benefit compared to existing treatment” could obtain early approval on the basis of a surrogate end point rather than a true clinical end point.3

The accelerated approval program enabled the FDA to speed up access to potentially effective new medications for patients with HIV/AIDS, cancer, or other serious conditions, many of which are quite expensive. These newly approved products would be subject to continued study after approval to ensure that their initial benefits translated to improved patient health and not just improvements in surrogate outcomes. Over the years, the program has received support from both the FDA and legislators, and Congress codified the program in 2012. But the program may not be serving its intended purpose because pharmaceutical companies often do not complete the required postapproval trials or they continue to use surrogate end points in those trials and regulators are unable to evaluate the true clinical benefits of a drug.4

Particularly when many accelerated approval drugs are priced in the hundreds of thousands of dollars, it is not clear that drug companies are charging prices that are merited by existing clinical evidence. As one example, the FDA granted accelerated approval in September 2016 to eteplirsen (Exondys 51) for the treatment of Duchenne muscular dystrophy. The pivotal trial for eteplirsen, which involved just 12 patients, evaluated whether the drug increased patients’ levels of dystrophin rather than whether the drug resulted in clinical improvement.5 Although the approval documents for eteplirsen expected a confirmatory trial to be completed in 2020, the manufacturer (Sarepta Therapeutics) reportedly does not plan to complete the trial until 2024.6 In the meantime, the annual cost of the drug can reach $1 million per patient, and even with a 23.1% rebate for state Medicaid programs, it is enormously expensive.

Despite reduced evidentiary standards for products approved under the FDA’s accelerated approval program, state Medicaid programs are legally obligated to cover these drugs on the same terms as drugs approved through traditional FDA approval pathways.7 As a result, states are often devoting increasingly limited financial resources toward expensive drugs that may ultimately have no real clinical benefit. The number of products approved under the accelerated approval pathway, as well as their prices, have increased substantially over time, putting significant financial pressure on Medicaid programs. During the first half of 2020 alone, products for 16 distinct oncology indications were approved through the accelerated approval pathway with a mean price of $18,198 per month.8

States have begun to challenge the requirement to cover all FDA-approved drugs, seeking to exclude drugs for which a clinical benefit has not yet been established. In 2017, Massachusetts submitted a waiver request to the CMS to close its Medicaid formulary to such products until the relevant clinical trials were...
completed; however, the CMS denied the waiver request in 2018 without providing an explanation. In 2020, the CMS publicly took the position that such formulary closures were legally permitted following a 2019 application from Tennessee for such a formulary closure in the context of a block grant application. Reducing spending for such products until the completion of confirmatory trials could increase financial incentives for companies to complete the postapproval trials of clinically important outcomes in addition to surrogates.

To date, many questions remain unanswered about the scale and implications of the FDA’s accelerated drug approval program. But with states publicly expressing concern about the costs of drugs approved in the accelerated program and their effect on the sustainability of Medicaid programs, it is important to understand these issues and identify targeted solutions for states. The goal is to help states financially while also ensuring vulnerable beneficiaries retain access to medically necessary products. Because Medicaid is jointly administered and funded by the federal government and the states, there are several potential policy options for addressing these goals.

One way to balance the dual goals of early access to novel therapies with financial sustainability for state Medicaid programs could be through modifying the Medicaid statutory rebate. For example, the federal government might seek to impose a much larger mandatory minimum Medicaid rebate for accelerated approval products until the required postapproval studies are completed and clinical benefits are established. If a 50% rebate was required, for instance, states could reduce spending on a $1 million drug to $500 000 until clinical benefits are confirmed compared with spending today of approximately $750 000. This strategy could maintain a relatively high level of patient access while also assisting states financially, although some affordability concerns would likely remain, given the high prices of some products.

Another set of interventions would enable the federal government to assume greater responsibility for the cost of accelerated approval medications. Products have been marketed under the accelerated approval pathway for conditions affecting fewer than 200 000 individuals in the US (conditions Congress deemed sufficiently rare to qualify for special incentives and approval benefits under the Orphan Drug Act), such as agalsidase beta and migalastat for Fabry disease, eteplirsen and golodirsen for Duchenne muscular dystrophy, and voxelotor for sickle cell disease. For these conditions, the level of risk and unpredictability at the state Medicaid level could be pooled and managed more effectively by the federal government. Even beyond Medicaid, a national rare disease fund could be created and supported through a tax on all insurance plans, allowing for concentrated price negotiations from a single payer, reducing the risk for an individual state, employer, or health plan. The CMS has already implemented a similar policy around chimeric antigen receptor (CAR) T-cell therapy. When a Medicare Advantage beneficiary receives CAR T-cell therapy, fee-for-service Medicare pays for that therapy to remove the risk for Medicare Advantage plans. This idea could be extended to benefit Medicaid programs.

As state Medicaid programs face looming budget crises related to COVID-19, they may look to the legal linkage between FDA approval standards and Medicaid coverage requirements as one potential source for policy reform and fiscal savings. Policy makers should consider options for balancing Medicaid financial sustainability, patient access, and reasonable pricing of prescription drugs.

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