Executive Summary

Solving for Access and Affordability: Prescription Drug Affordability Boards are Not the Answer

For years, states have implemented various policies to lower prescription drug costs in an effort to improve affordability. While this has taken various iterations, in recent years, state legislatures began implementing prescription drug affordability boards (PDABs) to lower the price of specified "high-cost drugs." While Maryland was the first in 2019, as of July 2025, 10 states have legislation in place for PDABs with many more states considering it.[1]

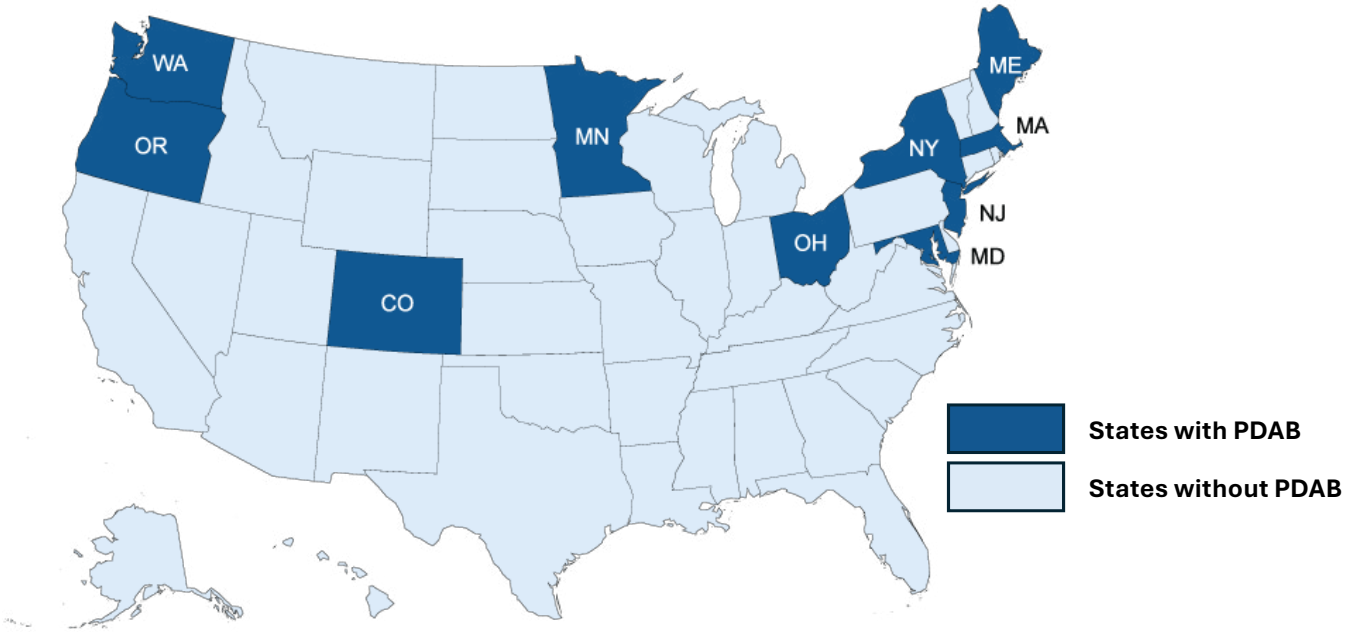
Theory has not matched reality and the implementation of PDABs has revealed difficulties in achieving its lofty goals and has led to concerns about unintended consequences. ***A quick legislative win creating a PDAB can, overall, mean little to the state or its residents because the healthcare system is incredibly complex, and savings may prove illusive.*** New Hampshire, for example, enacted an PDAB in 2020 and repealed it in 2025.

PDABs are not necessarily equipped to foresee the unintended consequences of their actions, particularly for patients with rare diseases, providers, hospitals, health centers and community pharmacies. Nowhere is this more apparent than with the PDABs that are moving towards implementation of Upper Payment Limits (UPLs). While the return on investment for PDABs is questionable overall, the challenges of implementation are considerable and the pay-off for patients is limited at best – particularly as we consider the use of UPLs.

PDABs and UPLs

A PDAB is a state-based oversight entity created to evaluate and manage the cost of prescription drugs. Equipped with legislative authority that vary by state, PDABs are tasked with monitoring drug prices, implementing price controls, conducting data analysis, reporting on pricing trends and drug markets, and formulating policy recommendations to improve consumer prescription drug affordability.[2]

Current Landscape of PDABs[3]



Source: https://naspa.us/blog/resource/pdab/

Once a PDAB determines what drugs are unaffordable, it has to work within its regulatory powers to determine next steps. Four states – Colorado, Maryland, Oregon, and Minnesota - have the authority to set UPLs for drugs that their PDABs deem unaffordable although no program has yet been fully implemented one.

The intent of UPLs is to establish a maximum price that payer will pay for specific drugs based on their cost-effectiveness and affordability.[4] More simply put, a UPL is a price cap. It represents the maximum amount that payers (such as state employee insurance programs or state licensed commercial health insurers) will reimburse for a particular drug to any stakeholder that dispenses or administers the drug. By setting this limit, the PDAB aims to exert downward pressure on drug prices within the state and hopes that manufacturers lower their price within the supply chain in order.

Arguments for UPLs emphasize the need to control escalating drug costs, make medications more affordable for the state and potentially patients, and provide a mechanism to negotiate for better prices. Proponents believe that UPLs can curb excessive pricing and ensure access to essential medicines without significantly hindering innovation.

But there is concern that UPLs ignore the realities of the pharmaceutical supply chain and could unleash a cascade of unintended consequences for patients that negate any savings that the state might realize.

States with UPL Authority


Washington


Maryland


Colorado


Minnesota

The Unique Impact of UPLs on Patients with Rare Disease

Patients with rare diseases face unique considerations within the context of PDABs and UPLs. If PDABs sets a UPL at a rate that does not work financially for the supply chain, rare disease patients are not just out of luck – they may be out of options.

Potential Patient Harms

Access issues from implementation of a UPL may go beyond potential inconvenience for patients with rare diseases. There is an assumption among supporters of UPLs that manufacturers will readily participate and lower their prices, but that remains an unproven theory and the consequences of being wrong risk patient access. If the UPL is significantly lower than WAC, then the manufacturer may not be able to offer it. Manufacturers that market orphan drugs sometimes count on a few patients to succeed. The difference between one or two patients in a quarter can throw a manufacturer for an exceedingly small, rare population into financial ruin.

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Site of care is often a concern unique to rare disease patients. Centers of Excellence are the emerging model for rare diseases, so patients may be traveling out of state for their care, and for those who require infusions, access to their therapies. This could lead to those institutions being unwilling to dose patients when their acquisition costs could be higher than the UPL.

Similarly, many rare medicines are only available through a sole source specialty pharmacy, often located out of state. Products, because of smaller populations, are drop-shipped just in time for patients or their care providers. Those products, again, are acquired and enter commerce outside of the UPL but because of the patient's insurance, may be reimbursed at the UPL price.

In either example rare patients may face an increase in costs because they could be required by the provider or specialty pharmacy to make up the difference between the UPL and the acquisition cost. Some providers may resort to brown bagging where the patient acquires the medicine and brings it to their appointment with them. Brown bagging is a hassle for patients and increases the risk of mishandling therapies that require special handling.

Like other patients, patients with rare disease may face formulary restrictions and/or utilization management barriers. However, patients with rare diseases typically have limited or no therapeutic alternatives, making any restriction in access particularly concerning.

There are also concerns that broadly targeting rare disease products through UPLs could negatively impact innovation in this area, as the development of these treatments requires substantial investment, and price controls might discourage pharmaceutical companies from pursuing such research.

State Exemptions

UPLs are ill-suited for orphan drugs. Orphan drugs treat populations of less than 200,000 and sometimes less than 500 patients eligible in U.S. When looked at on a state level, there is likely to be a low volume of drug utilization of orphan drugs in the state. A UPL would not be worth the effort and resources put forward by a state and assessing affordability for rare disease patients is not possible given the business model and that any attempt to do so will discriminate against that class of patient.

As mentioned earlier, most PDABs are permitted to use QALYs in their affordability and value determination process. QALYs are ill-quipped for calculating value when it comes to rare diseases and are viewed as discriminatory.[5] Also, due to the often-high cost of medications for rare diseases, these products may be more likely to meet the eligibility criteria for review by PDABs, even though they serve smaller patient populations.[6] This could lead to a disproportionate impact on this patient group.

Federal and state policy typically recognize the fragility of this market. The Inflation Reduction Act (IRA) excludes drugs with only one orphan designation from Medicare price negotiation because of concerns of harming innovation and access for patients with rare diseases. This was expanded through the One Big Beautiful Bill to exempt all orphan indications from negotiation. This was critical as the economic consequences of the IRA had a chilling effect on rare research and development programs. The first non-orphan indication would now be the trigger to "start the clock" on negotiation.

In the 340B program, certain covered entities are excluded from receiving 340B discounts on drugs with orphan designations. And on a state level, some state legislation purposefully excludes drugs for rare diseases. For example, California and Oregon have laws that exempt orphan drug manufacturers from certain price reporting requirements.[7]

In contrast, most PDABs do not have this limitation. A state may deem orphan drugs unaffordable and threaten patient access even though these patients have limited treatment options available. Currently, Washington State's PDAB is the only one that explicitly exempts treatments for rare diseases from UPLs, while other states like Colorado require the PDABs to "consider" the orphan status of a drug without specifying how this consideration affects the implementation of activities.[8] Oregon currently exempts orphan drugs from PDAB review although the legislature is considering removing that exemption.

The variability in PDAB processes can create regional access issues for patients, which is particularly ***troublesome*** considering the challenges patients with rare disease already face. Orphan drugs should be exempt from PDAB consideration.

PDABs and UPLs are Not the Path Forward

Patients, particularly those with rare diseases, need policies that improve access and affordability – not put them at risk. State-level drug pricing efforts, including UPLs or other efforts to use reference-based pricing like "most favored nation" policies or MFP pose significant threats to patients – particularly those with rare diseases. These policies, while aiming to reduce healthcare costs, can inadvertently stifle the innovation crucial for developing therapies for conditions affecting small populations. Rare disease drugs inherently face higher development costs due to limited patient pools for clinical trials and specialized research, making their profitability highly dependent on pricing.

Ultimately, such policies could lead to fewer new treatments, withdrawal of existing drugs from state markets, and reduced patient access to life-changing therapies, leaving those with rare diseases with even fewer, or no, viable options.

Apteka was commissioned for this white paper; editorial control was maintained by RAAP.

Sources

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