Solving for Access and Affordability: PDABs are Not the Answer

An Evolution of State Trends

For years, states have implemented various legislative and regulatory policies to lower prescription drug costs in an effort to improve affordability. The first wave was pharmacy benefit management (PBM) transparency rules theorizing that greater transparency would promote comparative shopping to reduce prices.[1] Then, the second wave focused on manufacturer price transparency reporting requirements.[2] Legislators believed that requiring manufacturers to report and justify price changes would prevent drug prices from increasing. Unsurprisingly, neither wave has reduced costs nor improved affordability for states or patients because the solutions ignored the workings of the healthcare system.[3]

The third wave started in 2019, when state legislatures started implementing prescription drug affordability boards (PDABs) to lower the price of specified high-cost drugs. While Maryland was the first, as of March 2025, 11 states have legislation in place for PDABs with many more states considering it.[4]

The concept sounds intriguing; create a board that offers state-specific affordability solutions and slashes drug costs. But, again, 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.

While the aim of lowering drug spending and improving patient affordability is laudable, unknowingly states might be creating access issues while not improving affordability. 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.

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Background on Prescription Drug Affordability Boards

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.[5]

Membership on PDABs varies but, in general, they have a mix of five to seven board members that meet regularly (at least quarterly.) Their remits differ; some states seeking to reduce spending for Medicaid, others for state employee health programs and others are broader looking at state and local government purchases or even all payers in the state. In some states, PDABs study prescription drug costs and make recommendations to negotiate additional Medicaid supplemental rebates; in other states they establish spending targets, set UPLs, or employ other strategies.[6]

The funding mechanisms for PDABs vary across the states that have implemented them, reflecting different policy choices and priorities. States like Colorado, Minnesota, Oregon, and Washington have opted for direct financial support through state appropriations.[7] Other states, like Maryland, fund their PDABs through assessments or fees levied on supply chain stakeholders like pharmaceutical manufacturers, wholesalers, and health plans.[8]

Current Status of PDABs

As of early 2025, 11 states have enacted legislation to create PDABs. The activities of PDABs vary depending on their stage of implementation and the specific mandates outlined in their state legislation.

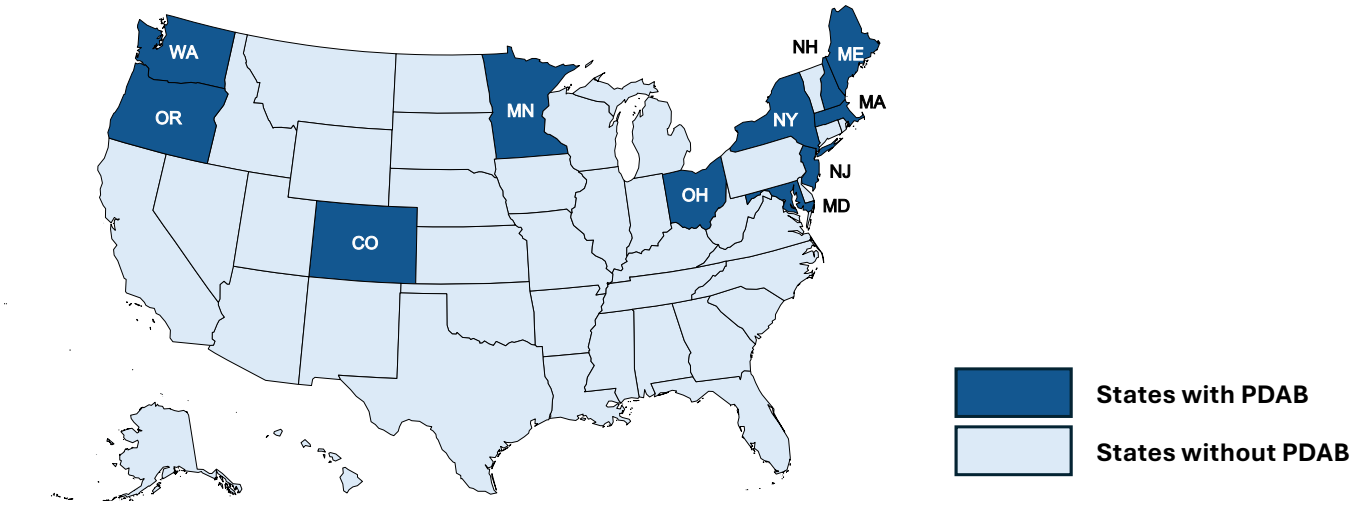
Table 1: Activities of PDABs

State	Year Authorized	Authority to Set Upper Payment Limits (UPLs)	Current Activities
Colorado	2021	Yes	Finalizing data submission guidelines for UPL rulemaking for Enbrel; holding rulemaking hearings in 2025
Maine	2019	No	Setting spending targets and issuing recommendations
Maryland	2019	Yes	Legislation introduced in 2025 to establish UPL process; six drugs selected for cost review in 2024
Minnesota	2023	Yes	Hiring Executive Director; gathering input for drug review process
New Hampshire	2020	No	Introduced bills in 2025 related to PDAB definitions and data collection
New Jersey	2024	No	Board appointments made as of March 2024
Ohio	2019	No	Council established; issued report of recommendations
Oregon	2021	No	Reviewed policies/procedures and data for affordability reviews; legislation introduced for alignment with transparency act; released 2024 report
Washington	2022	Yes	Approved drug selection policy; reviewed data dashboard; discussed shortlist of potential drugs for review in early 2025; released shortlist in Dec 2024
Massachusetts	2020	No (Drug utilization review board with negotiation authority)	Authority to negotiate how much the state pays a manufacturer for a prescription drug
New York	2017	No (Drug utilization review board with negotiation authority	Authority to negotiate how much the state pays a manufacturer for a prescription drug

[i] Prescription Drug Affordability Boards - Aimed Alliance, accessed March 28, 2025, https://aimedalliance.org/prescription-drug-affordability-boards/

Beyond the states that have already established PDABs, several others are actively considering or have legislation pending to implement such boards.

Current Landscape of PDABs[10]



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

In 2024, Connecticut, Illinois, Iowa, Kentucky, Michigan, Nebraska, Rhode Island, South Carolina, Vermont, Wisconsin, and West Virginia all had proposed PDAB legislation.[11] Legislative efforts have continued into 2025, with states like Arizona, Kansas, Michigan, Pennsylvania, Virginia, West Virginia, and Wisconsin considering similar measures.[12]

Assessing Affordability

While there are differences between PDABs in terms of their authority and funding, all are looking to assess prescription drug affordability.

The first step each PDAB takes is to follow statutory criteria to identify eligible drugs. Commonly, PDABs focus on the wholesale acquisition cost (WAC) of a drug. For example, a PDAB might set a WAC of $30,000 or more for a 12-month supply or a course of treatment lasting less than a year as a trigger for affordability review.[13]

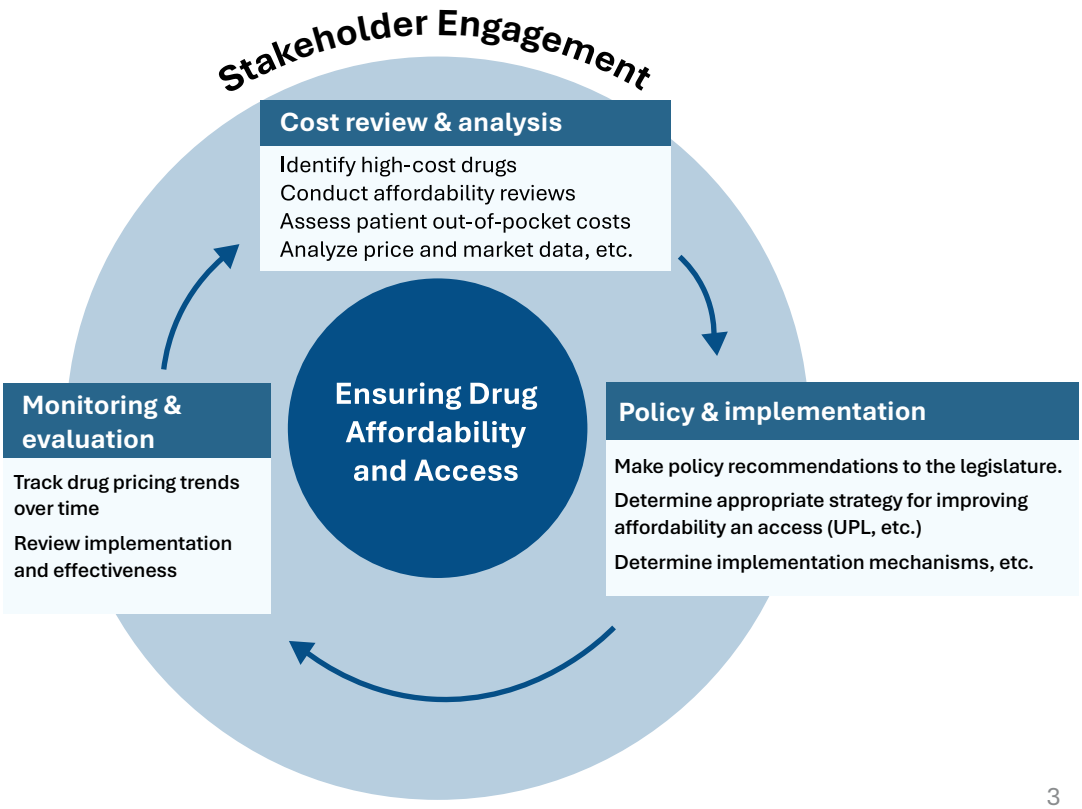
Once the PDAB has selected drugs to study, they then perform an affordability review. There is no single, universally adopted standard or definition of "affordability," meaning each state must produce its own definition of defining affordability. Is it looking at affordability to the state? To the patient? What is affordability to a patient? These costs are highly dependent on what plan individuals are enrolled in and the plan design that they are subject to (copayments, coinsurance, deductibles, etc.).

There is no single, universally adopted standard or definition of "affordability," meaning each state must produce its own definition of defining affordability

In examining affordability, a PDAB also might consider things like the lifecycle of the product, state spending, manufacturer discounts and rebates and whether patient assistance is available. PDABs might look at the overall budget impact; that is the indirect expenditures related to prescription drug spending such as premiums or productivity or lost wages.

States vary in their ability to use metrics like Quality Adjusted Live Years (QALYs.) Colorado's PDAB is permitted to consider QALYs during the affordability review process although it cannot use it during the value determination process as part of its UPL.[14] Patient advocates have been fighting against the use of this metric because it overlooks individual patient needs and preferences and is discriminatory against the disabled and those with rare diseases.[15]

Finally, the board may study the price of the drug against therapeutic alternatives, if there are any. In the case of rare diseases, there are often only one therapy available for the rare condition. However, if there are multiple therapies, the PDAB then decides the weight of one variable over another.



It is up to the PDAB to determine the value of state spending versus patient out-of-pocket spending and balance that against coverage and access. With states using different prioritization, access for patients may be limited based on where they live; PDABs can reach vastly different conclusions about the affordability of the same drugs and what to study to reach those conclusions. For example, none of the five drugs that Colorado chose, were chosen by Oregon. Instead, Oregon examined 15 different drugs.[16]

Each step in this affordability review process requires resources, debate, public input, and decisions. And this is just to get to the point where the PDAB can make recommendations on how to find savings within their remit (Medicaid supplemental rebates, UPLs, etc.). It is slow and resource-intensive; Maryland implemented its PDAB in 2020 and has had no savings to date.[17]

Use of UPLs to Set Prices

Once a PDAB selects a drug, the common next steps include:

- **Policy Recommendations:** PDABs may issue policy recommendations to state legislatures or other relevant bodies on ways to improve drug affordability.
- **Transparency Initiatives:** PDABs may promote transparency in drug pricing by collecting and publishing data on drug costs and manufacturer pricing practices.
- **Cost-Effectiveness Analysis:** PDABs can use cost-effectiveness analysis to evaluate whether the price of a drug aligns with its clinical value and effectiveness.
- **Engagement with PBMs:** PDABs may engage with PBMs to address their role in drug pricing and negotiate better prices.
- **Implement Pricing Initiatives:** PDABs may have the authority to set reimbursement rates, UPLs, for drugs that are determined to be unaffordable.

The intent of UPLs is to establish a maximum price that payer will pay for specific drugs based on their cost-effectiveness and affordability.[18] 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.

The UPL tells drug manufacturers and sellers that the state will not reimburse more than a specified amount for the drug. While UPLs set a ceiling on what payers can pay for a drug, they do not dictate the price that a manufacturer can charge for it.[19] PDAB advocates have stated that they ***hope*** that the UPL will pressure manufacturers to lower price, but the state does not have authority to set the manufacturer's price.[20] This differs from the Inflation Reduction Act's (IRA's) Medicare Drug Price Negotiation where, as a condition of participating in Medicare, pharmaceutical manufacturers must lower the acquisition price to the negotiated price for Medicare beneficiaries.

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. Colorado's PDAB is furthest along in its work; it initially found 604 eligible drugs and conducted an affordability review on five.[21] Of the five, three were found to be unaffordable for patients. This means that the state can move forward with setting a UPL for these drugs, which kicks off another process. The three drugs deemed unaffordable are for arthritis and not the costliest drugs for this therapeutic area. Further, these arthritis drugs have significant patient assistance support and are covered by plans.

Upper Payment Limits (UPLs)

are price caps. UPLs set a ceiling on what payers can pay for a drug, they do not dictate the price that a manufacturer can charge for it.

States with UPLs


Washington


Maryland


Colorado


Minnesota

Like the selection/affordability process, there is no standard methodology across all PDABs for setting the UPL price. PDABs can employ various strategies to determine the specific value for a UPL. These methodologies often draw upon existing price benchmarks and economic analyses.

- **Budgetary Thresholds:** PDABs can consider the drug's impact on overall budgets.[22] This could involve setting a UPL to limit the drug's contribution to increases in health insurance premiums or utilizing a budget impact analysis to set savings targets.

- **Reference Pricing:** PDABs could use internal reference pricing. For example, a PDAB might set a UPL based on the lowest price of another drug that it considers to be therapeutically equivalent to UPL drug.

  External reference pricing involves benchmarking the drug's price against prices negotiated by other entities. For example, the IRA's Medicare Negotiated Drug Program (Maximum Fair Price or MFP), or the prices negotiated by the Department of Veterans Affairs.

  Minnesota requires that if a drug that they select for the UPL has an MFP, the UPL be set at the MFP. But MFP is for drugs taken by Medicare enrollees which may, or may not, be the same ones that the state is selecting.

  It is possible that with federal attention on Most Favored Nation (MFN) pricing, which is the lowest price available in a comparator set of countries, states may use this price as a benchmark for UPLs.

- **Average Net Price:** PDABs could also set UPL to the average net price which would reflect rebates and discounts negotiated between the manufacturer and PBMs/health plan.

  For provider-administered drugs, PDABs might consider setting UPLs based on publicly available Average Sales Price (ASP) data and/or the Medicare rate given that Medicare often reimburses at a far lower rate than commercial plans.[23]

  In setting UPLs, PDABs can use drug pricing data available through pricing files available for purchase from databanks. This data includes information on WAC, often known as list price. This is the amount that wholesalers and other direct purchasers pay to acquire the drug from pharmaceutical manufacturers without factoring in any discounts, rebates, or other price reductions. PDABs do not have direct access to discounts and rebates that pharmaceutical manufacturers provide to commercial payers; they can purchase estimates of these rebates from companies like SSR Health.

  Focusing on the net price aims to ensure that the payment limit reflects the actual cost of the drug as experienced by payers. Of course, this entire process requires considerable resources to get to an average price which payers are already benefiting from. The state can go below the average price with discounts and rebates if it feels that this is still unaffordable, but lowering the average price has unintended consequences that we will discuss below.

PDABs are often statutorily required to consider other factors when setting UPLs, such as the cost of administering and delivering the drug, whether the drug has orphan drug status and whether it is on the Food and Drug Administration's drug shortage list.

Which payers have access to the UPL price varies by state. In general, the UPLs apply to state-regulated markets.[24] PDAB laws do **not** include employer self-funded plans covered under the Employee Retirement Income Security Act of 1974 (ERISA), but these plans may access the UPL if they choose. As a federal program, Medicare is governed by federal law and regulations and is exempt from the UPLs.

Medicaid already receives lower pricing through Best Price, but a state sets UPL below Medicaid Best Price, this would create a new nationwide Best Price. This is a problem discussed later in the paper.

Table 1: Activities of PDABs

State	Payers/Markets Subject to Upper Payment Limits	Key Exclusions
Colorado	All payers for prescription drug purchases/payments made within Colorado, including state agencies (like state employee plans, potentially Medicaid depending on structure) and state-regulated commercial plans (fully insured market.)	ERISA plans
Maryland	Originally state and local government entities only but in 2025 expanded to include commercial coverage regulated by the state.	ERISA plans
Maryland	State purchasers (including state employee plans, Medicaid fee-for-service & managed care, workers' compensation) and health plans/issuers regulated by the state (fully insured market.)	ERISA plans
Minnesota	All payers and PBMs that operate within the state. This includes state programs (like Medicaid, MinnesotaCare, state employee plans) and the state-regulated commercial market (fully insured plans.)	ERISA plans

UPLs Impact on Patient Access and Affordability

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. Interestingly, the potential challenges of UPLs mirror the concerns that are coming up as potential consequences of the IRA's Medicare drug price negotiation program with its MFP. We could be trading the perception of not receiving adequate discounts and rebates for the creation of other issues in the supply chain that will impact patient access and affordability.

Patient Access

Concerns about Drug Availability: UPLs assume that manufacturers would voluntarily sell the product to wholesalers at the UPL. While Washington and Colorado have legislated penalties for manufacturers that do not comply with the UPL and/or restrict patient access, manufacturers could decide that the penalties are more beneficial than offering the UPL or may find workarounds. If UPL were based on reference pricing or budget assessment, a major concern would be the possibility of drug shortages or limited access to medicines if manufacturers decide to withdraw their products from states where the UPLs are deemed too restrictive.[25]

Another possibility is that the UPL might be below Medicaid Best Price. State Medicaid programs require that pharmaceutical manufacturers provide Medicaid programs with the "Medicaid Best Price" for their drugs. This rebate is the greater of 23.1% of the Average Manufacturer Price or the difference between Average Manufacturer Price and Best Price. Best Price is determined by comparing the prices offered by drug manufacturers to various U.S. purchasers, including wholesalers, retailers, and healthcare providers (with exceptions for things like 340B pricing.) While federal policy can exempt its programs from applying to Medicaid Best Price, state programs cannot.

If the UPL was lower than Best Price, then the PDAB's UPL would be the new Medicaid Best Price in all states. This would increase a pharmaceutical manufacturer's liability nationwide. The implications of this are far reaching; manufacturers could be less inclined to do innovative research for the Medicaid population and/or change the types of drugs they bring to market. While unlikely, they could also decide not to participate in the Medicaid program. Some states like Maryland specifically prohibit the UPL from being impacting Best Price but there is the concern that not all states will.

At the pharmacy. It is possible that wholesalers may not be willing to buy the drug if their acquisition cost is higher than the amount the state will reimburse them or if they determine that it is no longer profitable to distribute these products in that state. That, in turn, means that pharmacies in that state may have difficulty keeping inventory of these products.

Even if pharmacies can stock the product, being able to be profitable or to break-even may be difficult. Pharmacies depend on a spread between the purchase price and the reimbursement price to survive. If the wholesaler decided to not provide a discount off the UPL to the pharmacy or decided to provide a smaller discount than they do currently or if the PBM decides to increaseits fees to make up for lost revenue, the pharmacy could lose money every time they dispensed the medicine. Already a survey found that over half of community pharmacists were losing money on at least 30% of their prescriptions due to fees from PBMs called direct or indirect remuneration fees (DIR) fees, with a substantial percentage considering closing their businesses.[26]

UPLs might be especially challenging for independent pharmacies in underserved communities that do not have the purchasing power of larger chains like Walmart and CVS. Reduced reimbursement rates may also force pharmacies to limit their stock of certain medications, particularly high-cost specialty drugs, potentially leading to delayed treatment and reduced access for patients.[27] Primary research with payers found that 60% of respondents believe that pharmacies may not stock UPL drugs.[28]

60% of respondents believe that pharmacies may not stock UPL drugs

These changes are happening concurrently with the difficulties pharmacies are having with the implementation of the IRA's Medicare negotiated drugs. As background, starting in January 2026, pharmacies will acquire Medicare Part D negotiated drugs at their normal rate and then reimbursed by health plans at MFP when pharmacists dispense the medications to Medicare patients. Pharmacy associations have raised concerns about the potential for "underwater reimbursements," where the MFP is set below the price at which pharmacies acquire the drugs, and they must wait to be repaid the delta between MFP and their acquisition price.[29]

The IRA also introduces operational complexities and increased administrative burdens for community pharmacies and there are growing concerns that the financial pressures and operational burdens might lead some pharmacies, particularly independent ones, to choose not to stock certain drugs with negotiated prices.[30],[31] A recent NCPA survey indicated that a significant percentage of independent pharmacists are either considering not stocking or have already decided not to carry one or more of the first 10 Medicare Part D drugs selected for price negotiation.[32]

Pharmacies could face similar challenges with UPLs. Limited reimbursement along with the administrative burden of managing inventory, might not be enough to make it financially viable for pharmacies to stock and dispense UPL drugs.[33] **Instead of availability at a community pharmacy, patients may have to contact specialty pharmacies for mail order or drive to pharmacies further from their homes to get their medicines.**

At the provider's office. Providers, like pharmacies, must purchase the product and store it in anticipation of dispensing the drug—this is referred to as "buy and bill." While Medicare reimburses outpatient drugs at the ASP plus 6%, commercial plans typically reimburse providers at a much higher rate.[34]

Lowering provider reimbursement could affect the financial viability of providing certain treatments. Reimbursement not only covers the cost of drugs, but it also pays practice costs associated with drug administration, such as purchasing variability, shipping fees, and overhead. It also pays for the administration of the drug and additional services tied to patient care. Providers count on commercial health plan's higher reimbursement to provide services that health plans do not directly pay for. **The price of the drug does not equal the cost of treatment** and reimbursing based on UPL would significantly alter the revenue of providers. Providers might encounter challenges in acquiring medications at prices that align with the established UPLs, potentially leading to financial losses.[35]

Another effect of reimbursing based on UPL would be a decrease in the ASP because the new UPL price would factor into average sales and providers **around the country** would feel the impact of the lower reimbursement rate. ASP is a widely used benchmark and, as said earlier, used by Medicare for reimbursement. If ASP drops, Medicare reimbursement (and potentially commercial reimbursement) would drop for thousands of providers nationwide that would not have the benefit of a lower acquisition cost (the UPL.) These providers could be "underwater" on provider-administered prescriptions.

The interaction between PDABs, UPLs, and the 340B program creates a complex set of challenges. The 340B Drug Pricing Program is a federal program established in 1992. It requires drug manufacturers participating in Medicaid to provide outpatient drugs at discounted prices to eligible healthcare organizations that serve a high proportion of low-income or vulnerable patients. These "covered entities" include hospitals serving a disproportionate share of low-income patients, federally qualified health centers, and other safety-net providers. The 340B program aims to help these providers stretch their resources, enabling them to provide more comprehensive care to underserved populations. Discounts from 340B range from 20% to 50% off the ASP, although the discount can be higher for certain drugs.[36]

While both PDABs and the 340B program aim to improve affordability and access to medications, their mechanisms may end up conflicting. Providers at 340B covered entities, who benefit from a wide difference between the 340B price and their commercial reimbursement, would now be limited to the spread between 340B and the UPL. This reduction in revenue could limit the ability of these providers to offer comprehensive services, such as specialized clinics, medication management programs, and other support services that are essential for vulnerable populations. It could also force providers to reduce the number of patients they serve or to cut back on essential programs, potentially leading to reduced access to care for those who rely on safety-net providers.

Changes to Health Plan Formularies: There is also concern about patient access through their health plans. If PDABs institute UPLs, manufacturers could stop giving PBMs/plans additional rebates.

Payers prefer high cost, high rebate prescription drugs. Low cost, low rebates generate less revenue because cost-sharing is often based on the cost of the drug, not net rebate. A UPL would reduce revenue for PBMs/plans, and plans might encourage utilization away from UPL drugs to competitor products through formulary changes and/or utilization management barriers like prior authorization or step therapy.

In fact, 50% of surveyed payers said they believe that utilization management would increase on drugs with UPLs.[37] Formulary changes resulting from UPLs could disrupt stable treatment regimens if plans force patients to switch medications due to coverage changes. For rare patients there may be no switch options at all.

Patient Affordability

Drugs with a UPL need enough utilization (volume) to produce savings to make the PDAB worth the investment. It could translate to lower out-of-pocket costs for patients although this is not always a direct outcome of UPLs.

Patients may not see a difference in their out-of-pocket costs unless the state mandates that payers pass the savings on to patients. Overall, UPL drugs represent a fraction of overall spending so individual health plan savings will be limited. It is likely that the savings will get lost in the shuffle of costs.

In fact, primary payer research found that:

- 67% of payers thought that patient out-of-pocket spending will either stay the same (17%) or increase (50%).[38]
- And more than half of payers (57%) thought that health insurance premiums would increase with the use of UPLs.[39]

In summary, patients could see pharmacies close, an inability to get their drugs at their pharmacies, a push toward therapeutic alternatives, reduction in provider services, premium increases, and no reduction in out-of-pocket costs for their drugs.

The Unique Impact of UPLs on Patients with Rare Disease

For rare disease patients, the unintended consequences are like those for other patients, but more dire. 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.

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 states 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.[40] 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.[41] This could lead to a disproportionate impact on this patient group.

Federal and state policy typically recognize the fragility of this market. The 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. Due to the economic consequences of the IRA on rare research and development programs, there are efforts to expand that exemption to include any additional orphan designations that the product may receive and to "start the clock" on negotiation with the first non-orphan designation.

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.[42]

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.[43]

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.

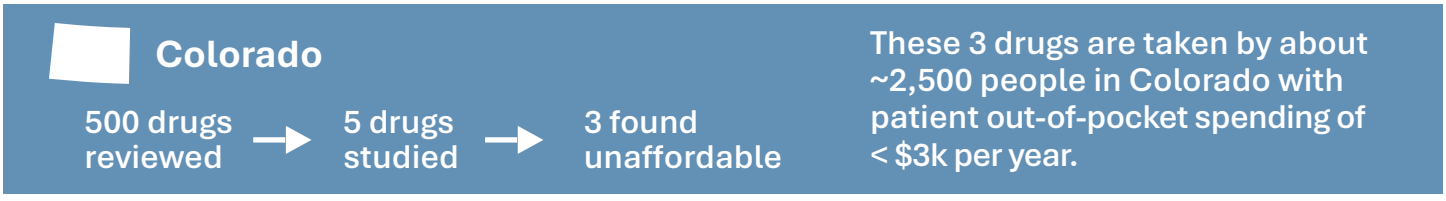
PDABs and UPLs are Not the Path Forward

After considering the potential consequences of UPLs, it is difficult to see their value against the potential drawbacks and understanding the resources (both financial and otherwise) that go into implementing UPLs. The establishment and operation of PDABs and the implementation of UPLs do not lower costs for patients and could create barriers to access for drugs subject to UPLs and others in the same drug class.

Pharmaceutical manufacturers pay discounts, rebates and fees that bolster the supply chain; they create the margins that drive the entire supply chain. PBMs and plans secure discounts and rebates from pharmaceutical manufacturers that may not be evident by simply looking at the list price of a drug or even the cost-sharing associated with a product. Industry representatives also question the effectiveness of PDABs in addressing the root causes of high drug costs, pointing to the role of PBMs and health insurers in determining what patients pay at the pharmacy counter.[44]

UPLs may not create enough savings that patients notice them. With just a few drugs subject to the UPL, the savings to payers and thus passed on to patients, will be minimal if any. It is a fraction of overall spending that factors into calculating benefit design decisions like premiums and deductibles. **Much of the cost of drugs is diverted to other stakeholders in the supply chain (i.e., wholesalers, PBMs, plans, etc.), with about 50% going to the pharmaceutical manufacturer. Going back to the preference for high cost, high rebate drugs, by focusing on the price, we need to be thinking more holistically about the cost.**

And, if none of the savings are going back to patients, what was the purpose of considering affordability? Is affordability even the right question? No two patients pay the same price for a drug; it can vary widely depending on their health coverage; what is affordable to one patient may not be affordable to another. The Colorado PDAB has reviewed five drugs and found that three drugs, Enbrel, Stelara and Cosentyx, are unaffordable for Coloradans. According to its own data, there are about 2,500 total people in the state taking these drugs and the out-of- pocket cost is less than $3,000 per year.[45]



Ultimately, PDABs are unlikely to help patients with their affordability in a meaningful way and they face a long road of legal challenges.[46] Already Amgen filed a lawsuit against Colorado, challenging the constitutionality of imposing such payment limits on Enbrel.[47] This case was dismissed in late March 2025 because the court said Amgen lacked standing to bring a lawsuit against the state of Colorado because no damage had yet been done to Amgen.[48] There will be more lawsuits as PDABs begin their implementation and, by then, the pharmaceutical companies may be able to demonstrate damages.

Solutions that Matter to Patients

Rather than implement processes that are time-consuming and costly without delivering on savings for patients, states should consider more meaningful reforms that address the drivers of healthcare that impact patients' out-of-pocket costs and truly help patient affordability. These suggestions can provide real benefit to patients in terms of lower costs, and increase certainty of access to treatments, including rare therapies:

- Work towards policy changes that remove barriers to access such as banning non-medical switching and step therapy.
- Streamline (or eliminate) prior authorization requirements, particularly for rare disease therapies to reduce administrative burdens for a medically necessary therapy.
- Adopt affordability changes that have a greater impact on reducing patient costs, such as out-of-pocket cost caps, copay accumulator and maximizer bans and eliminating alternative funding programs.
- Encourage the use of copayments rather than coinsurance so that the out-of-pocket cost is more consistent and manageable. Some states require plans to offer capped co-payment plans as part of their Exchange plans.
- Implement PBM reforms that require rebate pass-throughs to consumers at the pharmacy counter and banning claw backs and spread-pricing.
- Consider creating a state-level (or multi-state) rare disease reinsurance program where payers (like insurance companies) transfer the financial risk associated with treating rare diseases, which are often expensive and unpredictable, to a third-party insurer (a reinsurer). This helps protect payers from potentially catastrophic costs and ensures rare disease patients have access to treatments.
- Permit and encourage Medicare Part B Medigap plans for Medicare-eligible patients under the age of 65. This would permit disabled patients to purchase supplemental coverage that could reduce their out-of-pocket cost sharing.

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.

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