THE IMPACT OF
Prescription Drug Affordability Boards on Rare Disease Therapies

March 6, 2025

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RARE READY

A GENETIC CONDITION COALITION

What We’ll Cover Today

- Understanding Prescription Drug Affordability Boards (PDABs)
- Exploring the unintended consequences for rare disease patients
- Sharing how CF United successfully advocated against the inclusion of a rare disease drug on Colorado’s PDAB
- Hearing from The Bonnell Foundation and their advocacy in Michigan
- Discussing state advocacy strategies to protect access to rare disease therapies

Polling Question

- How familiar are you with Prescription Drug Affordability Boards?
    - **Very familiar** – I have in-depth knowledge and understanding.
    - **Somewhat familiar** – I have heard of them and know a little.
    - **Not very familiar** – I have heard the term but don't know much.
    - **Not familiar at all** – This is new to me.

UNDERSTANDING
PDABs

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PDAB Process Overview

PDABs are state-level entities created to review and set upper payment limits (UPLs) or caps on prescription drugs deemed unaffordable. They analyze drug cost trends, insurance claims data, and patient affordability factors to make pricing recommendations.

Steps After Legislation Establishes a PDAB

- Board Formation – State appoints members with expertise in healthcare, economics, and drug pricing.
- Data Collection & Review – PDAB gathers pricing, cost-effectiveness, and market impact data on selected drugs.
- Public Hearings & Stakeholder Input – Patients, providers, and manufacturers may provide testimony.
- Affordability Review – Board evaluates whether a drug’s cost is leading to financial hardship for patients or the healthcare system.
- Decision on Upper Payment Limits (UPLs) – If deemed unaffordable, the PDAB sets a statewide price cap for insurers and payers.

Who is Appointed

- Typically appointed by the Governor or state agencies.
  - Health economists
  - Public health experts
  - Physicians or pharmacists
  - Insurance representatives
  - Government or policy experts
  - Patient advocates are rarely appointed

How PDABs Affect Drug Pricing and Access

How do PDABs determine which drugs are “too expensive”?

- PDABs review pricing data, often relying on cost-effectiveness analyses that **do not fully consider the unique value of rare disease treatments**.
- They may use **third-party benchmarks**, such as the Institute for Clinical and Economic Review (ICER), which applies **standardized cost-per-QALY (Quality-Adjusted Life Year) metrics** that often undervalue treatments for small patient groups.

What role do insurance companies and PBMs play?

- Insurers and PBMs **control formularies and coverage decisions**, determining which medications patients can access and at what cost.
- PDAB-imposed price controls may lead insurers to **restrict coverage** further or require **more prior authorizations**, delaying treatment for rare disease patients.

How do PDABs differ from federal drug pricing policies?

- **PDABs operate at the state level**, creating **a patchwork of regulations** that can result in drug availability varying by location.
- Unlike federal agencies, most PDABs **lack transparency, accountability, and patient input**, making their decisions more difficult to challenge.
- PDABs **do not regulate PBMs or insurers**, meaning their efforts to control prices may **not directly reduce patient costs** at the pharmacy counter.

The Real Impact of PDAB Policies

While PDABs aim to control drug costs, their **one-size-fits-all** approach risks **limiting access to rare disease treatments**.

PDABs Do

- Restrict access to innovative therapies, especially for rare disease patients.

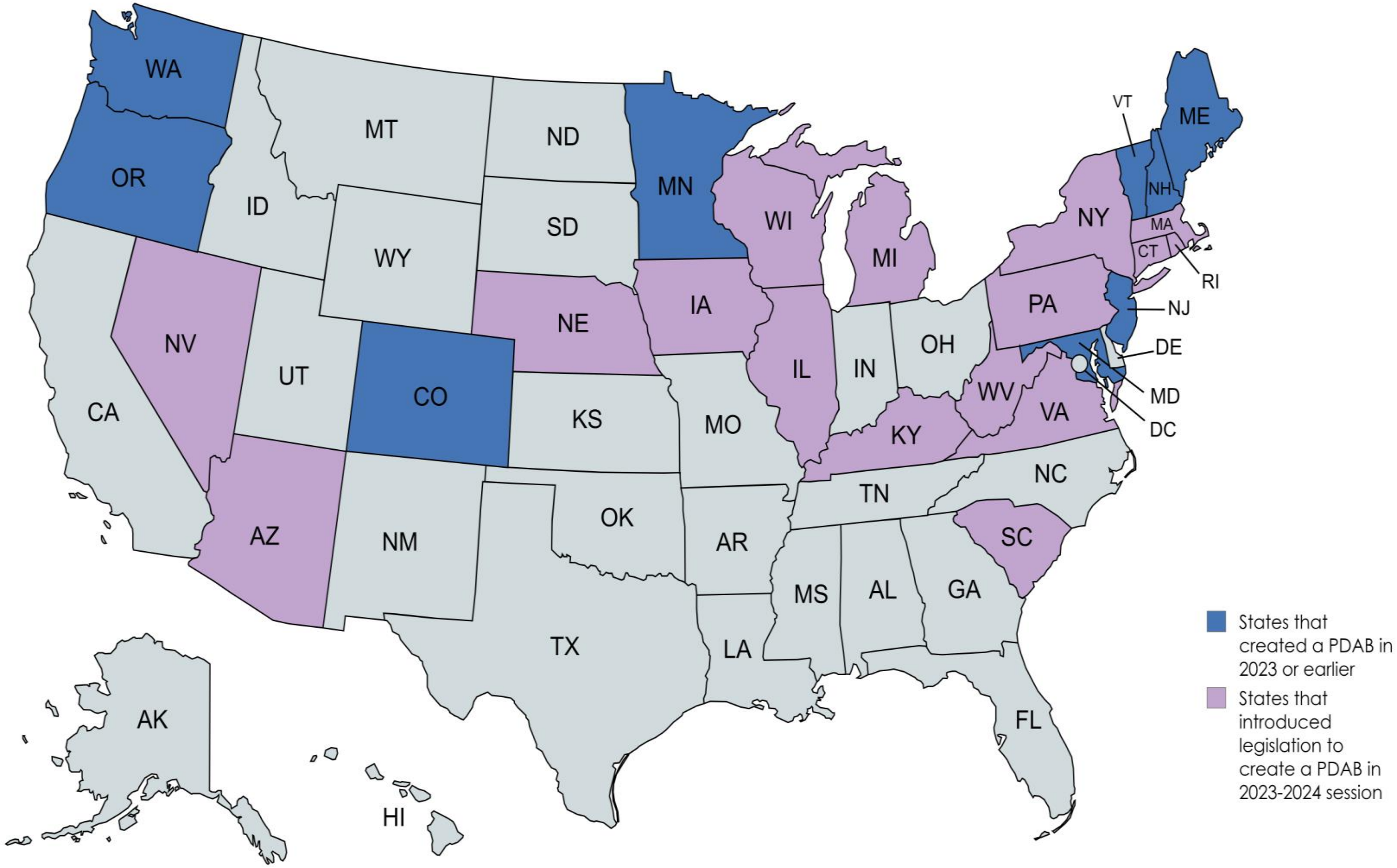
PDABs Do Not

- Lower patient copayments.
- Reduce premiums.
- Create health system transparency.
- Increase access.

PDABs Lead To

- Reduced rare disease research.
- Fewer new treatments.
- Restricted access to essential medicines.

PDAB State Legislative Landscape



🟦 States that created a PDAB in 2023 or earlier

🟪 States that introduced legislation to create a PDAB in 2023-2024 session

- **9 states** have established a PDAB via legislation*
- **4 states PDABs** have UPL authority (Colorado, Maryland, Minnesota, and Washington)
- **2 states PDABs** have exemptions for rare (Oregon, Washington)
- **6 states** have introduced PDAB legislation so far in 2025 (Illinois, Michigan, Virginia, Maryland, Kansas, Iowa)

* New Jersey created a Drug Affordability Council, which has the authority to recommend affordability strategies.

Rare Disease Exemptions – Washington & Oregon

Oregon's Rare Disease Exemption Language - preferred exemption language:

- Oregon 2021 SB 844: Section 2, 2 reads **"A drug that is designated by the Secretary of the United States Food and Drug Administration, under 21 U.S.C. 360bb, as a drug for a rare disease or condition is not subject to review under subsection (1) of this section."**
  - This exemption language could be at risk if Oregon introduces language to give the PDAB authority to set a UPL. That bill didn't come up this session but is expected in the 2026 session.

Washington's Rare Disease Exemption Language - comprise-friendly exemption language:

- Washington 2022 SB 5532: Section 3 reads **"The Board must identify prescription drugs that have been on the market for at least 7 years, are dispensed at a retail, specialty, or mail-order pharmacy, are not designated by the United States food and drug administration under 21 U.S.C. Sec. 30bb as a drug solely for the treatment of a rare disease or condition and meet the following thresholds."**
  - Take note of the word "solely" here. That was a topic of debate as many other companies have drugs that are approved both for a rare disease as well as an additional indication, so this exemption does not apply to their products.

Legislation to Expand and/or Create PDABs

Michigan

- SB 3, SB 4 and SB 5 in Michigan introduced to create a PDAB (no UPL).
- This bill includes **authority for the Governor to appoint members of the PDAB and for the PDAB to set an UPL or price ceiling for drugs.** It is currently in the Senate Committee on Finance, Insurance, and Consumer Protection.

Iowa

- Bill introduced to create a PDAB (no UPL but can make UPL recommendations).
- This Board would consist of 5 members appointed by the Governor, but who also are subject to confirmation by the state Senate.
- This bill also creates a **"Prescription Drug Affordability Stakeholder Council" with 19 members appointed by the Legislature,** but no reference to rare disease.
- This bill is currently referred to a Senate Subcommittee.

Illinois

- IL HB 1443/SB 66 would **establish a new PDAB** and has not been assigned a Committee yet but could be a threat of passage this year.

Virginia

- **HB 1724 passed the legislature.** The Governor will have 30 days to veto it, which we believe he will do.

Maryland

- MD PDAB was established in 2019 for state and local payers and now has UPL authority.
- This year HB 242 / SB 357 was introduced which would **expand the PDAB to all payers.**
- The bill passed the House. In the Senate we are trying to get a rare disease exemption added.
- Rare & Ready letter sent to every member on Senate Finance and the Senate President requesting a rare exemption.

Kansas

- SB 212 introduced to create a PDAB and allow for an UPL.
- This Board would be explicitly called the "Prescription Drug Pricing Board" with 5 members appointed by the Governor representing "healthcare economics, health policy, health equity, and clinical medicine".
- This bill also **creates a "Prescription Drug Affordability Stakeholder Council" with 21 members appointed by the Governor – none of which reference patient care and/or lived experience with rare disease.**
- This bill is currently referred to the Senate Committee on Financial Institutions and Insurance.

WHAT HAPPENED IN
COLORADO

Amanda Boone

Co-Founder

CF United

CYSTIC FIBROSIS
United

Colorado

PDAB

Where are we now?

Cystic Fibrosis United

Who Is Cystic Fibrosis United?

Patient Led

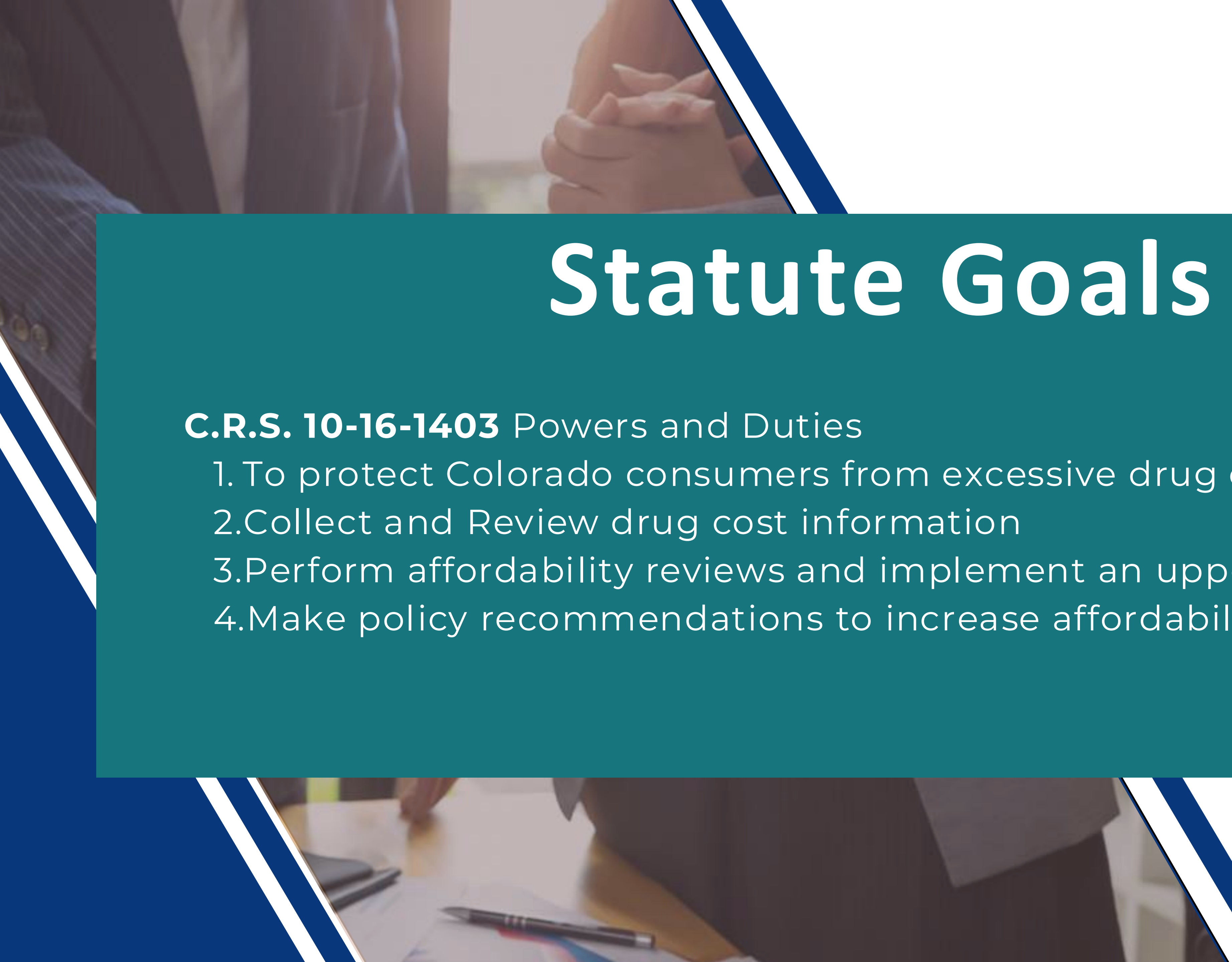
CF United is a patient led group based out of Colorado that helps connect, educate, and support medically complex families and
people with rare diseases.

Community Supported

We are a grassroots organization that has primarily been funded by the community.
Each one of us is a patient, or the caregiver
of a person with a rare disease.

Education

Education is one of the most important parts of our mission. We want patients to learn valuable tools so that they can advocate effectively. We also make sure patients are aware of policies that could impact their access to care.



Statute Goals

C.R.S. 10-16-1403 Powers and Duties

1. To protect Colorado consumers from excessive drug costs
2. Collect and Review drug cost information
3. Perform affordability reviews and implement an upper payment limit
4. Make policy recommendations to increase affordability of medications

Upper Payment Limits

A UPL is a cap on what your insurance company can reimburse your pharmacy or infusion center.

- Does not change out of pocket costs.
- Does not affect what the manufacturer charges.
- Does not impact federal insurance like Medicare or Tricare.
- DOES impact your private insurance policy.
- Savings ARE NOT required to be shared directly with patients.
- Insurance coverage could change.

The pipeline

The U.S. Pharmacy Distribution and Reimbursement System for Patient-Administered, Outpatient Prescription Drugs

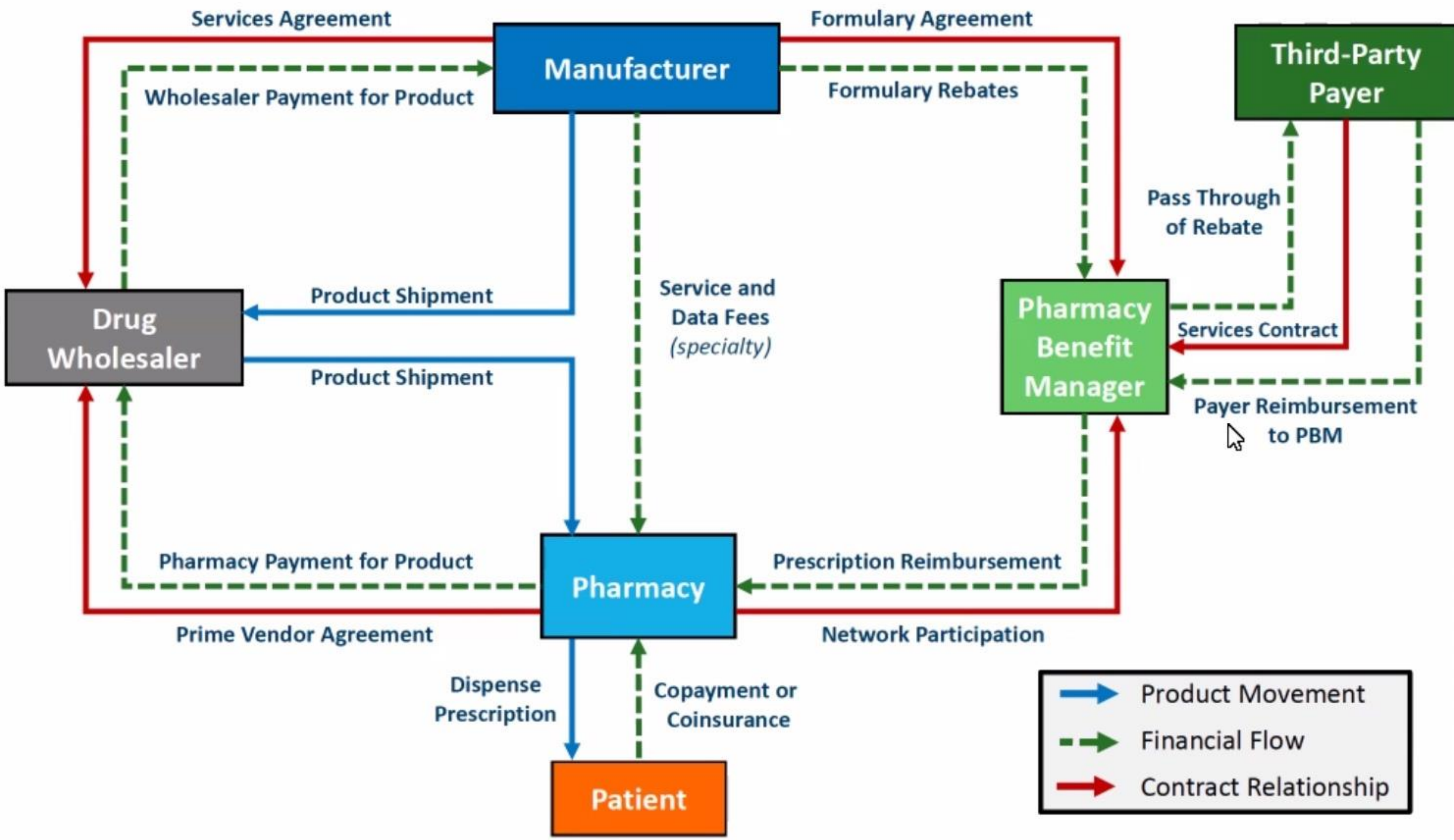


Chart illustrates flows for patient-administered, outpatient drugs. Please note that this chart is illustrative. It is not intended to be a complete representation of every type of financial, product flow, or contractual relationship in the marketplace.

Source: Fein, Adam. J., *The 2016 Economic Report on Retail, Mail and Specialty Pharmacies*, Drug Channels Institute, January 2016.

(Available at http://drugchannelsinstitute.com/products/industry_report/pharmacy/)

Challenges Facing the PDAB

Poor Patient Engagement

The burden of engagement has been placed directly on the patients and there is staggeringly low participation.

Inconsistent Process

Rather than looking at a consistent selection process, the Board can change direction for each set of reviews.

Discriminatory and Incomplete Data

Federally banned discriminatory data along with lackluster surveys are being used to determine affordability.

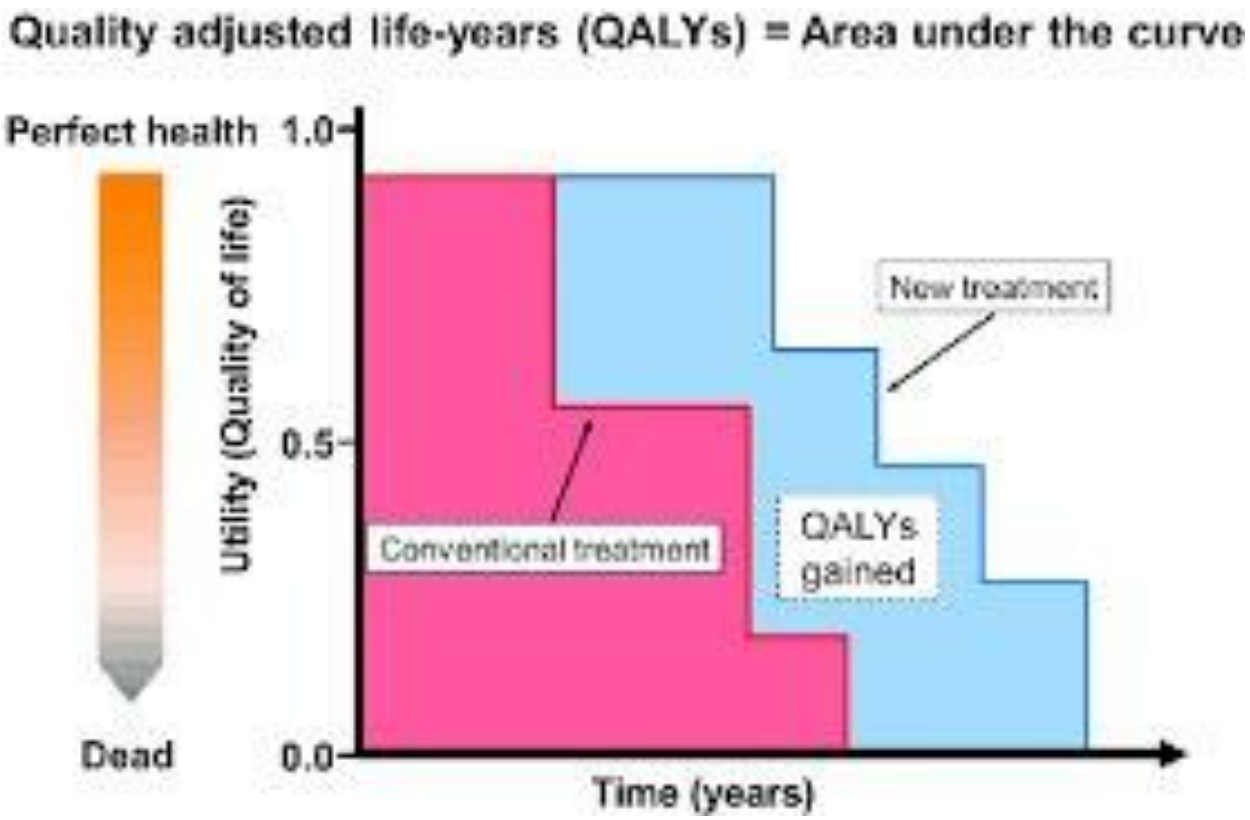
What is a QALY?

Quality Adjusted Life Year

The Quality Adjusted Life Year is a metric used to assess the" value" of a treatment. It measures the length of life versus the perceived quality of the years lived.

Does Not Include

- Fewer Hospitalizations
- Ability to Work
- Improvement in Condition



www.researchgate.net/figure/A-schematic-representing-a-QALY-calculation-for-a-comparison-of-two-treatments-The-QALYs_fig1_370652527

Goals

We are working to be sure the affordability review process stays patient centered, maintains data integrity, is transparent, and consistent.

Ban Discriminatory Data

Protect Rare Disease Patients

Help with the adoption of a more robust and inclusive survey.

Keep the focus on patients, not insurance companies.

CF United

Thank You!

Contact Us

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ADVOCATING AGAINST PDABs

Laura Bonnell

The Bonnell Foundation:
Living with Cystic Fibrosis

This presentation is provided by Laura Bonnell and the views expressed are solely those of the hers and The Bonnell Foundation.

The Bonnell Foundation:
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STATE

ADVOCACY

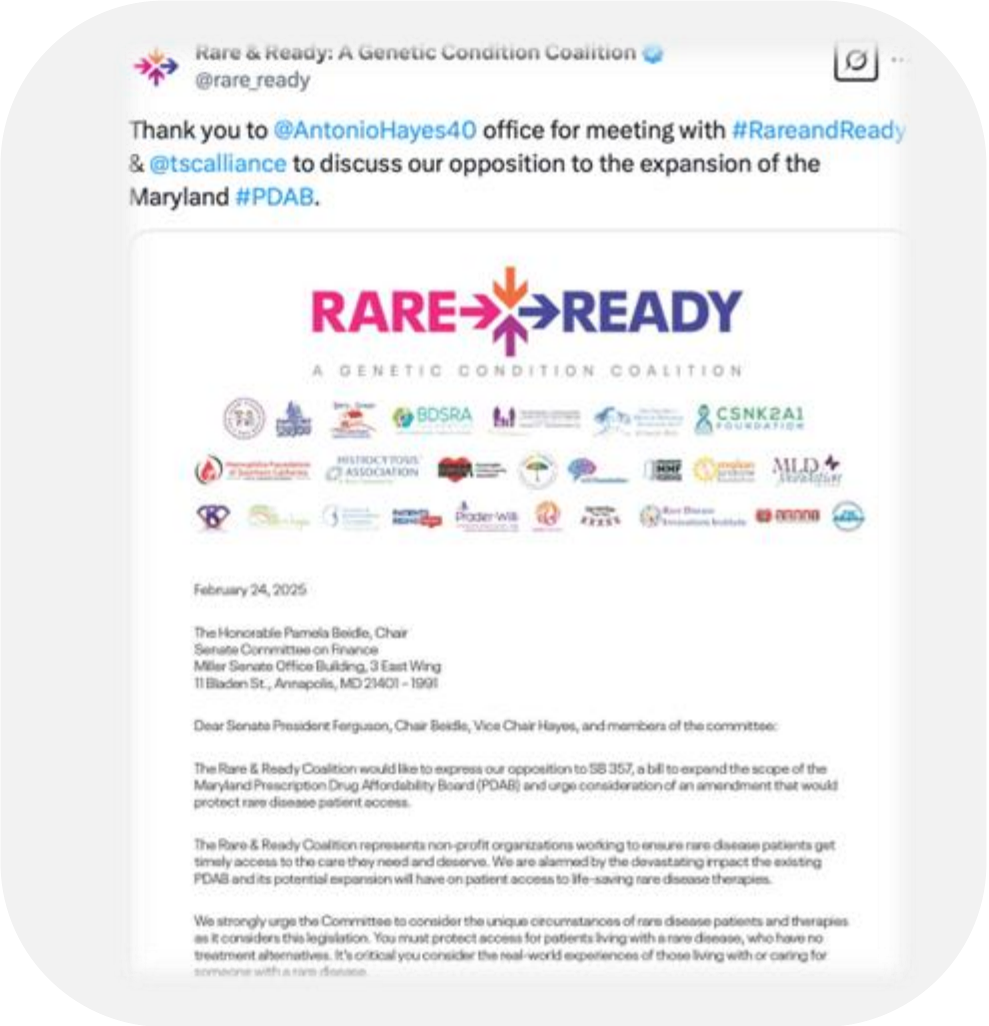
Kari Lato

Sr. Director, Policy & Advocacy

Rx4good

Effective Advocacy When PDAB Legislation is Introduced

- Once legislation is introduced, it is referred to committee for a hearing.
- Testify in person or submit written comments opposing the formation of a PDAB.
- Meet with decision-makers to share why PDABs are harmful to the rare disease community.
- Lend your logo to group letters.
- Join the coalition – kari.lato@rx4good.com.
- Use social media to educate and bring awareness to the issue – make sure to tag legislators and include relevant hashtags.



Rare & Ready: A Genetic Condition Coalition
@rare_ready

Thank you to @AntonioHayes40 office for meeting with #RareandReady & @tscalliance to discuss our opposition to the expansion of the Maryland #PDAB.

February 24, 2025

The Honorable Pamela Beidle, Chair
Senate Committee on Finance
Miller Senate Office Building, 3 East Wing
11 Bladen St., Annapolis, MD 21401 - 1991

Dear Senate President Ferguson, Chair Beidle, Vice Chair Hayes, and members of the committee:

The Rare & Ready Coalition would like to express our opposition to SB 357, a bill to expand the scope of the Maryland Prescription Drug Affordability Board (PDAB) and urge consideration of an amendment that would protect rare disease patient access.

The Rare & Ready Coalition represents non-profit organizations working to ensure rare disease patients get timely access to the care they need and deserve. We are alarmed by the devastating impact the existing PDAB and its potential expansion will have on patient access to life-saving rare disease therapies.

We strongly urge the Committee to consider the unique circumstances of rare disease patients and therapies as it considers this legislation. You must protect access for patients living with a rare disease, who have no treatment alternatives. It's critical you consider the real-world experiences of those living with or caring for someone with a rare disease.

Polling Question

- After attending this webinar, how would you rate your understanding of Prescription Drug Affordability Boards compared to before?
  - **Significantly improved** – I feel much more informed and confident in my understanding.
  - **Somewhat improved** – I have a better grasp but still have some questions.
  - **About the same** – My understanding hasn’t changed much.
  - **Still unclear** – I need more information to fully understand PDABs.

THANK YOU

WWW.RAREANDREADY.ORG

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RARE READY COALITION