PORTAL
Program on Regulation, Therapeutics, And Law

DoPE BWH

Identifying and Selecting Drugs for the Washington PDAB Affordability Review

March 20, 2024

Program On Regulation, Therapeutics, And Law (PORTAL)
Division of Pharmacoepidemiology and Pharmacoeconomics, Department of Medicine
Brigham and Women's Hospital and Harvard Medical School

HARVARD MEDICAL SCHOOL

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Founding Member, Mass General Brigham

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  - Colorado Division of Insurance
  - Oregon Division of Financial Regulation
  - Washington State Health Care Authority

Outline

1. About PORTAL
2. PDAB Process Overview
3. Identifying Eligible Drugs
4. Selecting Drugs for Affordability Review
5. Lessons from Other PDABs

Section 1.

About PORTAL

About PORTAL

The **Program On Regulation, Therapeutics, And Law (PORTAL)** is an interdisciplinary research group based in the **Division of Pharmacoepidemiology & Pharmacoeconomics** at Brigham & Women's Hospital and Harvard Medical School.

We study the intersections between **evidence-based use, regulation, and affordability of prescription medications**, and publish on a variety of topics in these areas.

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The PORTAL PDAB Team

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PORTAL Involvement with PDABs

Colorado PDAB

- Support **methodology development** for the first affordability reviews, including identifying therapeutic alternatives.
- Participated in **educational series** for Board members
- Provide guidance as Colorado begins upper payment limit deliberations.

Oregon PDAB

- Provide **technical guidance** for individual affordability reviews, including strategies for presenting data.
- Support staff in preparing **annual reports** required under statute.
- Deliver **presentations** to educate board members about specific drug classes (e.g., insulins)

National Academy for State Health Policy

- Prepare **white papers and memos** on PDAB processes and the prescription drug supply chain.
- Provide **technical assistance** relevant to the cohort of state PDABs.
- Work with NASHP on PDAB implementation and legislative developments.

Section 2.

PDAB Process Overview

Washington PDAB – Process Overview

Identify eligible drugs	Select drugs for affordability review	Conduct affordability review	Establish upper payment limit
"By June 30, 2023, and annually thereafter…the board must identify prescription drugs" that meet certain statutory criteria.	"The board may choose to conduct an affordability review of up to 24 prescription drugs per year identified pursuant to RCW 70.405.030."	"For prescription drugs chosen for an affordability review, the board must determine whether the prescription drug has led or will lead to excess costs to patients."	"Each year, the board may set an upper payment limit for up to 12 prescription drugs" that were found to have led or will lead to excess costs.
RCW 70.405.030	**RCW 70.405.40**	**RCW 70.405.40**	**RCW 70.405.50**

The Board is tasked with considering various criteria and data elements at each step in this process.

Washington PDAB – Process Overview

"By June 30, 2023, and annually thereafter…the board must identify prescription drugs" that meet certain statutory criteria.

RCW 70.405.030

"The board may choose to conduct an affordability review of up to 24 prescription drugs per year identified pursuant to RCW 70.405.030."

RCW 70.405.40

"For prescription drugs chosen for an affordability review, the board must determine **whether the prescription drug has led or will lead to excess costs to patients**."

RCW 70.405.40

"Each year, the board may set an upper payment limit for up to 12 prescription drugs" that were found to have led or will lead to excess costs.

RCW 70.405.50

What are "Excess Costs"?

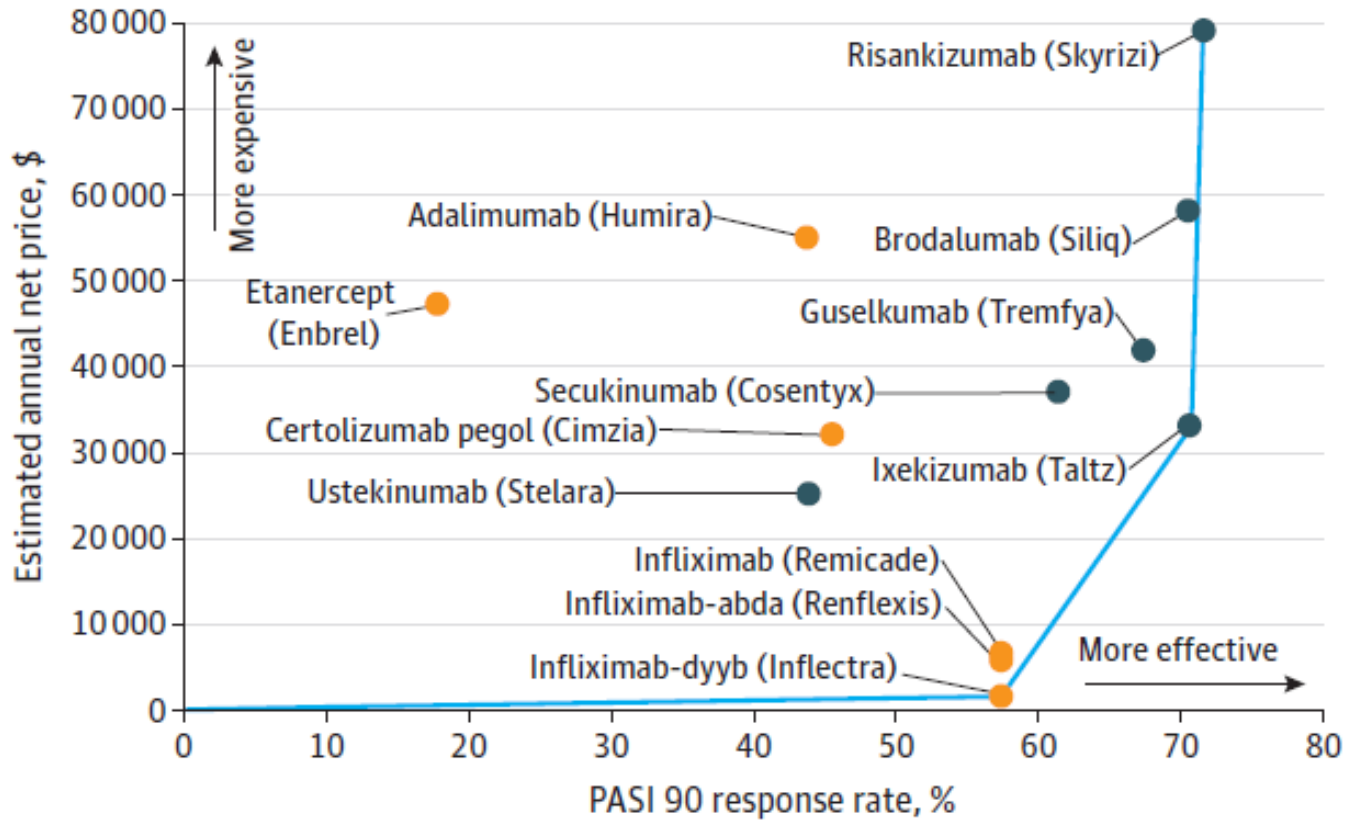
According to the statute (RCW 70.405.010), **"Excess costs"** include costs that:

"…**exceed the therapeutic benefit** relative to other alternative treatments"	"…are **not sustainable to public and private health care systems** over a 10-year time frame."
• Can compare prices directly (**reference pricing**) or use **cost effectiveness analyses.**	• Typically measured using **budget impact analysis**.
• For drugs with multiple indications, may need to assess separately for each indication.	• Depends on the **price** of the drug and the **size of the population** being treated.

RCW 70.405.010

Example: Efficiency Frontier Comparing the Prices and Effectiveness of Psoriasis Biologics

Figure 1. Efficiency Frontier for Psoriasis Biologics in the US



Egilman et al. *JAMA Dermatology.* 2024.

Excess Costs to *Which* Patients?

Patients Using the Specific Drug?

- **Out-of-pocket costs** are borne by patients using the drug.
- High out-of-pocket costs are associated with lower medication adherence and poor clinical outcomes.
- Depends on drug price and **insurance plan design** (copayments, coinsurance, deductibles).

All Patients in the State?

- Spending on prescription drugs is ultimately borne by **all patients**:
  - Health care premiums
  - Lower wages due to premiums paid by employers
  - Taxes for public insurance (state-sponsored, Medicaid, Medicare)

What if drug manufacturers offset out-of-pocket spending using **patient assistance programs and coupons?**

Washington PDAB – Process Overview

"By June 30, 2023, and annually thereafter…the board must identify prescription drugs" that meet certain statutory criteria.

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Section 3.

Identifying Eligible Drugs

Eligibility Criteria

Each year, 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 FDA as a drug solely for the treatment of a rare disease or condition, and
- meet one of the following thresholds:

Branded Drugs & Biologics	Generics	Biosimilars
• **High Price:** WAC $60k+ per year or course of treatment; OR • **Price increase** ≥15% in any 12-month period or ≥50% over three years	• WAC of $100+ for a 30-day supply; AND • Price increase of ≥ 200% in the preceding 12 months	• Initial WAC not at least 15% lower than the reference biologic WAC at the time of biosimilar launch

RCW 70.405.030; WAC 182-52-0035

Drugs that Treat Rare Diseases

[T]he board must identify prescription drugs that…are not designated by the United States Food and Drug Administration under 21 U.S.C. Sec. 360bb ***as a drug solely for the treatment of a rare disease or condition****.*

- **Under the Orphan Drug Act,** drug manufacturers can obtain "orphan" designations for drugs that target "rare diseases or conditions," defined as one that affects **<200,000 people in the US.**

- **Orphan designations come with certain incentives**, including tax credits, fee exemptions, and a guaranteed 7 years of market exclusivity.

- **From 2008-2018, 41% of newly approved drugs had at least one orphan-designated indication.**

RCW 70.405.030; 21 USC Sec. 360bb; Darrow, Avorn, Kesselheim. *JAMA*. 2020

Orphan Designations are for Indications, Not Drugs

Drugs can fall into five general categories based on the orphan status of their approved indications:

Sole Orphan Drugs	Drugs with **one indication**, and **that indication is a rare disease** Example: Trikafta for cystic fibrosis
Multi-Orphan Drugs	Drugs with **multiple indications**, **all of which are rare diseases** Example: Pomalyst for multiple myeloma and Kaposi sarcoma (both rare)
Orphan First Drugs	Drugs with **both rare and non-rare** indications, the **first of which is a rare disease** Example: Keytruda first for unresectable/metastatic melanoma (rare), later for several non-rare cancers
Non-Orphan First Drugs	Drugs with **both rare and non-rare** indications, the **first of which is not a rare disease** Example: Enbrel first for rheumatoid arthritis (non-rare), later for juvenile idiopathic arthritis (rare)
Non-Orphan Drugs	Drugs with one or more indications, **none of which are rare diseases** Example: Farxiga for diabetes, heart failure, and chronic kidney disease (all non-rare)

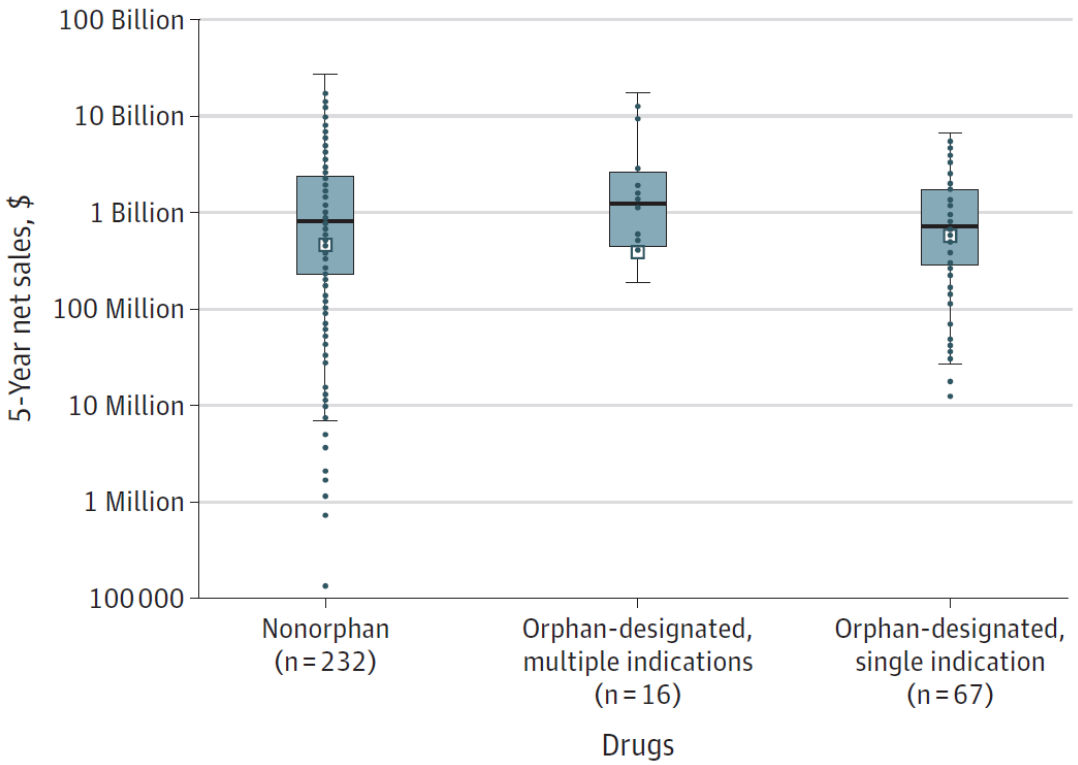
Vogel et al. *JAMA Internal Medicine.* 2023.

Drugs for Rare Diseases Can Be Profitable

Median sales in first 5 years were similar for orphan-designated ($719 million) and non-orphan ($812 million) drugs.

Orphan-designated drugs have launch **prices 7 times higher** than non-orphan drugs.

Figure. Five-Year Net Sales, Stratified by Orphan Drug Designation



Tu SS et al. *JAMA.* 2023.

Eligibility: Branded Drugs & Biologics

High Price: WAC $60k+ per year or course of treatment; OR

Price increase ≥15% in any 12-month period or ≥50% over three years

Brand-name drugs and biologics are likely to represent most drugs eligible for affordability review.

Branded drugs account for **~10% of prescriptions** in the US but **~90% of spending**.

In general, US list prices of brand-name drugs are **4x higher than those in comparable high-income countries.** (3x higher when accounting for rebates/discounts)

RCW 70.405.030; Mulcahy et al. RAND. 2024.; IQVIA, Use of Medicines in the US. 2021.

Eligibility: Branded Drugs & Biologics

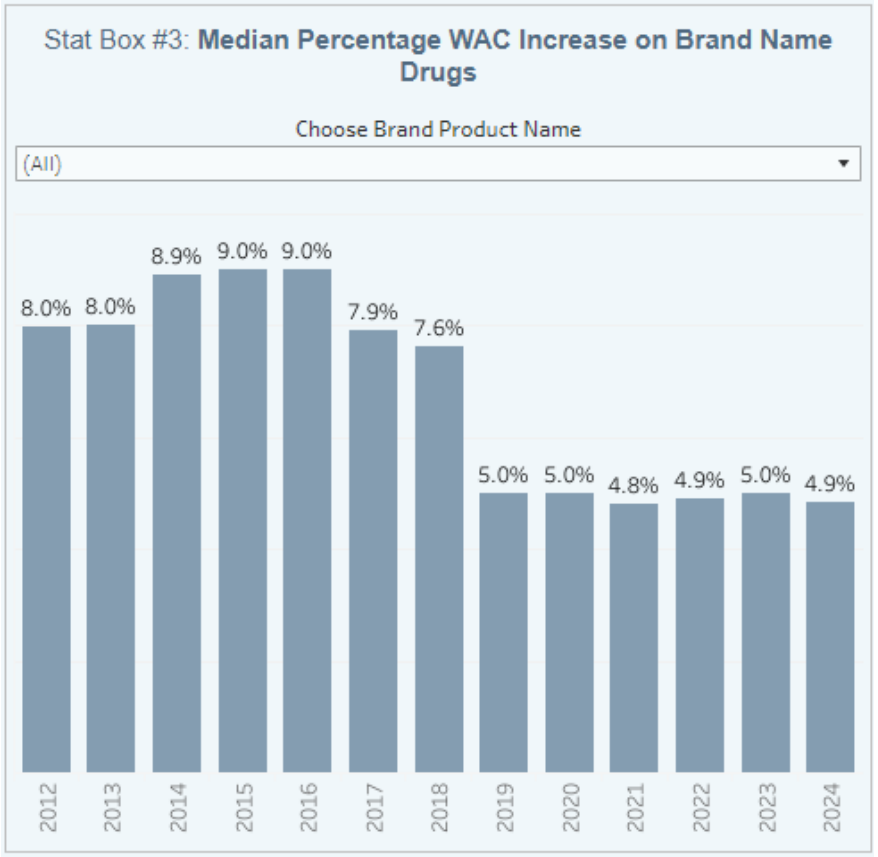
In 2021, the median list price (WAC) for newly marketed drugs was **$180k per year**.

- From 2008-2021, launch prices increased by 20%/year

Annual increases in drug list prices have slowed in recent years.

- Median increase of ~5%/year from 2019-2024

However, **the gap between list and net price continues to widen**. This has implications for patient OOP costs, which are derived from drug list prices, not the post-rebate net price.



46brooklyn. Brand Drug List Price Change Box Score. March 2024; Rome et al. JAMA. 2022

Eligibility: Generic Drugs

WAC of $100+ for a 30-day supply with a price increase of ≥ 200% in the preceding 12 months

Most generic drugs cost <$100 per 30-day supply, and price increases >200% are uncommon (<5%).

Generic price spikes are more common among **sterile injectables and drugs with 3 or fewer manufacturers**.

Generic drugs in **shortage** are twice as likely to experience price increases.

RCW 70.405.030; Patel, Kesselheim, Rome. *Health Affairs.* 2021; Trujillo et al. *Health Economics Review*. 2022; Hernandez et al. *Annals of Internal Medicine.* 2018

Eligibility: Biosimilars

Initial WAC not at least 15% lower than the reference biologic WAC at the time of biosimilar launch

The biosimilar market is just emerging. As of January 2024, 38 biosimilars were approved and marketed in the US for 10 reference biologics.

- 4 additional biologics have FDA approved biosimilars that are not yet marketed.

Concerns about biosimilar pricing strategy. In the case of Humira, some biosimilar makers set prices 5% less than the reference drug, offering rebates to compete for formulary position.

RCW 70.405.030; Samsung Bioepis. Biosimilar Market Report. 2024. Rome, Kesselheim. *Arthritis Rheumatol.* 2023

Example: Eligible Drugs in Colorado

Colorado Eligibility Criteria	No. of Eligible Drugs in 2023
Branded drugs or biologics with WAC **≥$30k per year** or course of treatment*	582
Branded drugs or biologics with a WAC **increase of ≥10%** in the preceding 12 months*	9
Generic drugs with a WAC of ≥$100 and with a WAC increase of 200%+ in the preceding 12 months	0
Biosimilars with an initial WAC not at least 15% lower than the reference biologic	13

*Colorado thresholds for brand-name prices and price increases are different from Washington

Colorado Division of Insurance. CO PDAB 2023 Eligible Drug Dashboard. 2023

Section 4.

Selecting Drugs for Affordability Review

Selection Criteria

RCW 70.405.40 - When deciding whether to conduct a review, the Board shall consider:

Class of the prescription drug and whether any **therapeutically equivalent prescription drugs** are available for sale

Input from relevant advisory groups established pursuant to RCW 70.405.020

The average **patient's out-of-pocket cost** for the drug

RCW 70.405.040

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Drug Class

A drug's class can be assessed from two distinct perspectives, though the terms are often used interchangeably.

Pharmacologic Class	Therapeutic Class
• Drugs that share **similar scientific properties**, including chemical structure, mechanism of action, and/or physiologic effect on the body • Example: tumor necrosis factor (TNF) inhibitors, janus kinase (JAK) inhibitors	• Drugs intended to treat **similar diseases/conditions** or that share clinical indications. Drugs can fall into multiple therapeutic classes if they have multiple indications. • Example**:** antihypertensives, disease-modifying antirheumatic drugs (DMARDs)

FDA. Established Pharmacologic Class. Updated May 2023.

Drug Classification Systems

Several organizations categorize drugs into classes:

- American Society of Health-System Pharmacists (ASHP) AHFS Pharmacologic-Therapeutic Classification
- World Health Organization Anatomical Therapeutic Chemical (WHO-ATC) Classification
- US Pharmacopeia Drug Classification (USP DC)
- Private drug databases (MediSpan, First Databank, Red Book)

Nomenclature varies between these schemes, so it is important to be consistent in the system used to identify drug class for the purposes of selection.

ASHP. AHFS Therapeutic Classification System; WHO. Anatomical Therapeutic Chemical (ATC) Classification; USP. USP Drug Classification (USP DC).

Therapeutic Equivalents

Therapeutic equivalents are defined under PDAB rule as "drug product[s] of the **identical base or salt as the specific drug product prescribed** with essentially the same efficacy and toxicity when administered to an individual in the same dosage regimen."

Therapeutic Equivalent Pairs		Identified via
Branded Small Molecule Drug	Generics	**FDA Orange Book** ("TE" code)
Branded Biologic	Biosimilars*	**FDA Purple Book**

*Biosimilars are not identical/equivalent to their reference biologic, but have been demonstrated to be highly similar with no clinically meaningful difference in safety or efficacy

FDA may grant therapeutic equivalence ratings for generics before they are marketed. Need to confirm marketing dates (e.g., from use in the state), in addition to FDA approval.

WAC 182-52-0010 (effective June 10, 2024); FDA Orange Book; FDA Purple Book

Out-of-Pocket (OOP) Costs

OOP costs typically include the **deductibles, coinsurance, and copayments** patients pay at the point of sale (e.g., pharmacy).

OOP Costs depend on drug cost and benefit design. Because of deductibles and out-of-pocket maximums, these costs may vary throughout a calendar year.

Potential Data Sources

- Claims data (e.g., Washington State All Payer Claims Database)*
- Pharmacy data (e.g., IQVIA, Symphony Health)

**Data from payers typically does not include information about coupons, manufacturer assistance programs, and other forms of patient financial assistance.*

Section 5.

Lessons from Other PDABs

Drug Selection Among Other PDABs

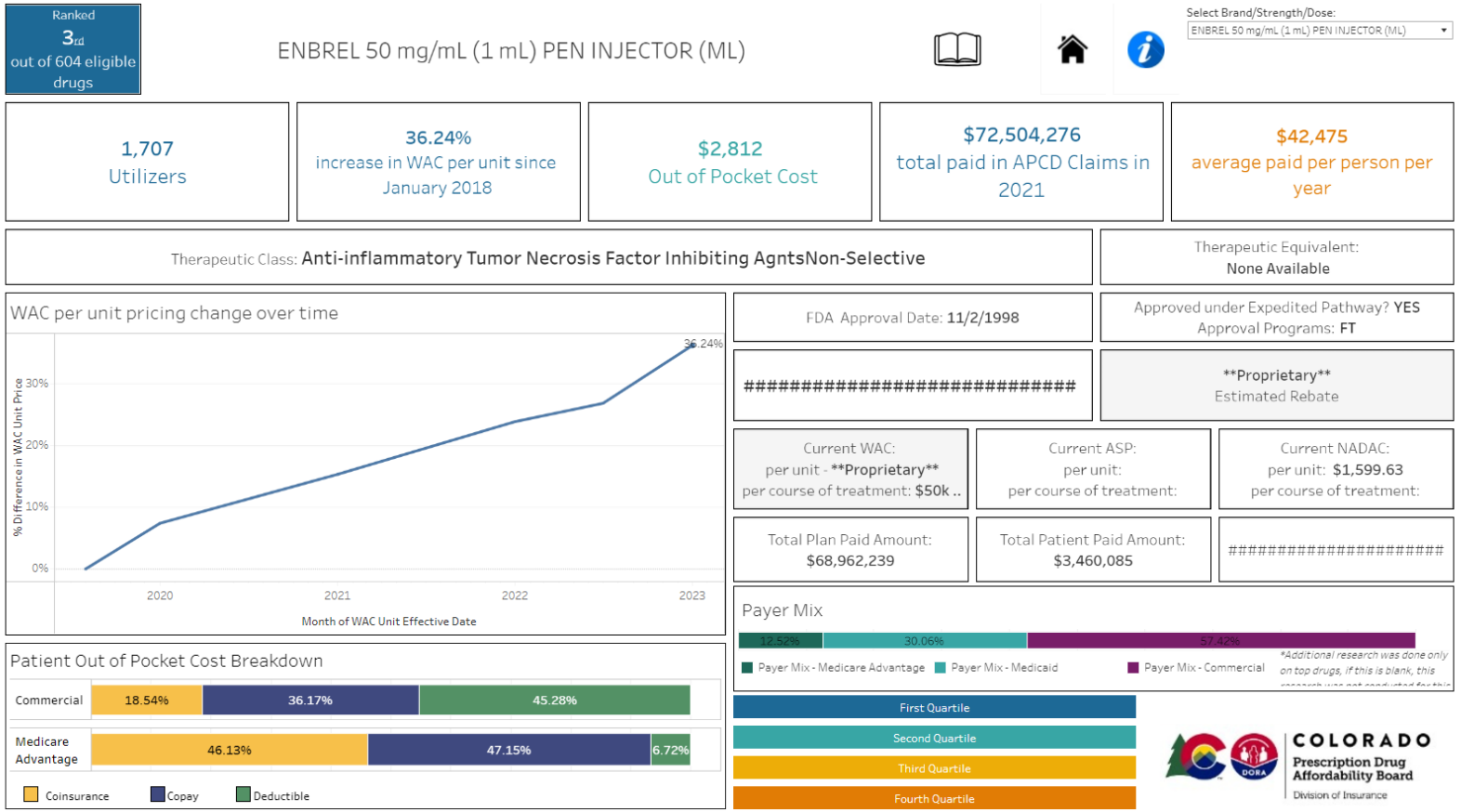
As of March 20, 2024, **Colorado and Oregon have selected drugs** for affordability review. Maryland has announced its initial list of drugs for Board consideration and intends to select drugs in the coming months.

Given that the Colorado and Maryland PDABs are structurally similar to Washington, we will focus on these two states' selection strategies.

Drug Selection in Colorado

The Colorado PDAB considers patient **out-of-pocket costs, therapeutic class,** and **aggregated data on drug spending & utilization** to select drugs.

Data was presented to the Board in the form of a public dashboard, with individual profiles on each drug at the NDC level.

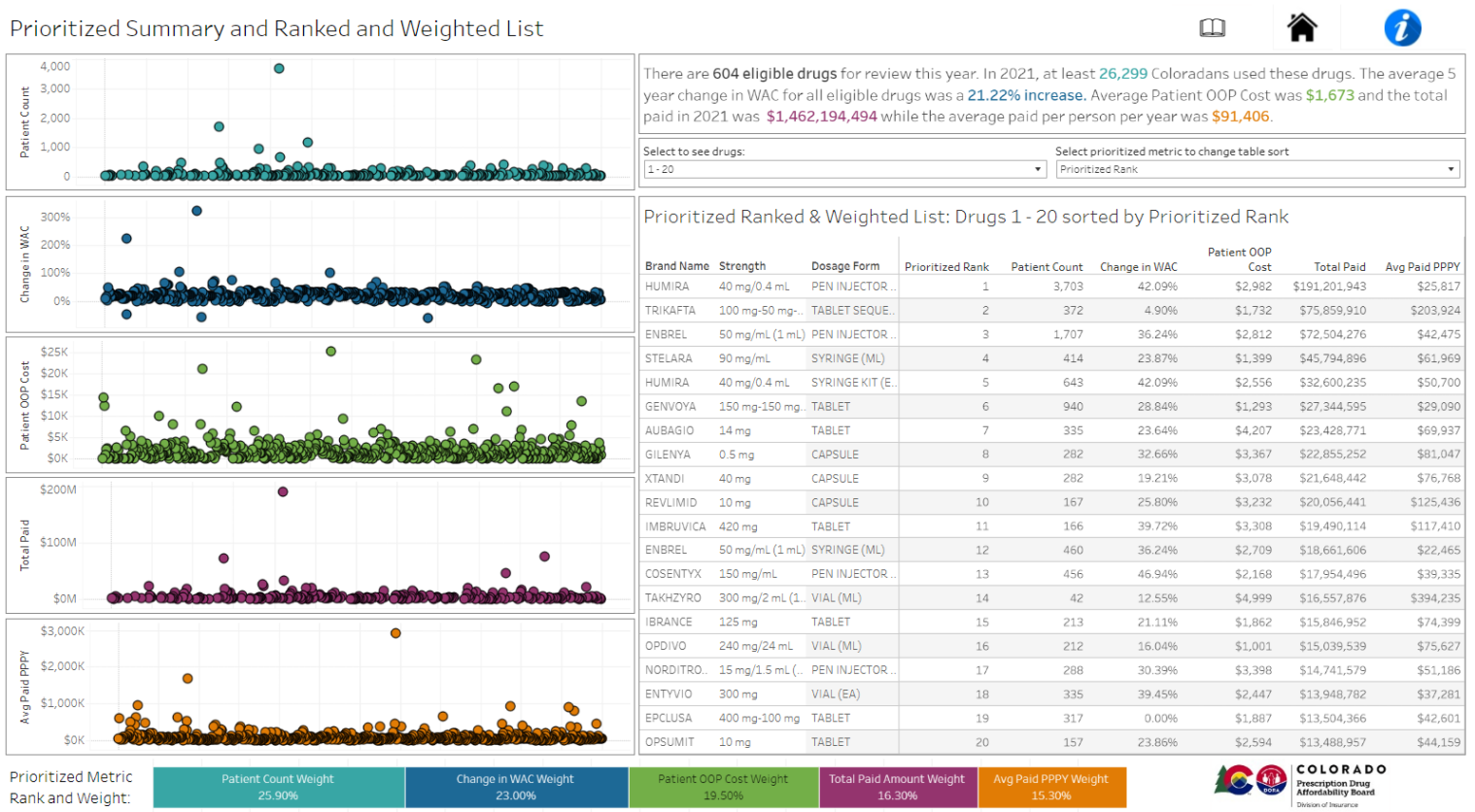


Colorado Division of Insurance. CO PDAB 2023 Eligible Drug Dashboard. 2023

Drug Selection in Colorado

The Colorado PDAB **weighted selection criteria** through a guided prioritization exercise, resulting in a **prioritized drug list** that reflected the Board's priorities.

This prioritized list was used as the foundation of the Board's selection.



Colorado Division of Insurance. CO PDAB 2023 Eligible Drug Dashboard. 2023

Aggregating Drugs Before Selection

For drugs with **multiple strengths and dosage forms**, data may be disaggregated. This can affect how drugs are ranked by total spending, utilization, and other factors.

Example: Colorado PDAB ranking of Gilenya (1 form) vs. Skyrizi (3 forms)

Drug-Strength-Dosage Form	Prioritized Rank	Patient Count	Rank by Utilization	Total Paid in Claims, 2021	Rank by Total Paid
Gilenya 0.5 mg Capsule	**8**	**282**	**16**	**$22,855,252**	**8**
Skyrizi 150 mg/1.66 mL Syringe Kit	31	261	19	$11,339,685	30
Skyrizi 150 mg/mL Pen Injector	67	150	33	$5,200,282	63
Skyrizi 150 mg/mL Syringe	68	153	31	$5,158,946	66
Skyrizi (if aggregated)	**--**	**~564**	**~5**	**$21,698,913**	**9**

Colorado Division of Insurance. CO PDAB 2023 Eligible Drug Dashboard. 2023

Drug Selection in Maryland

The Maryland PDAB uses an **internal dashboard** with data on **each eligible drug's FDA approval and therapeutic class**; **spending and price**; **out-of-pocket costs**; and other factors.

This dashboard was used to create a preliminary list of drugs for Board discussion. Board members can also nominate drugs for consideration. Drugs will then be referred for **stakeholder council and public input**.

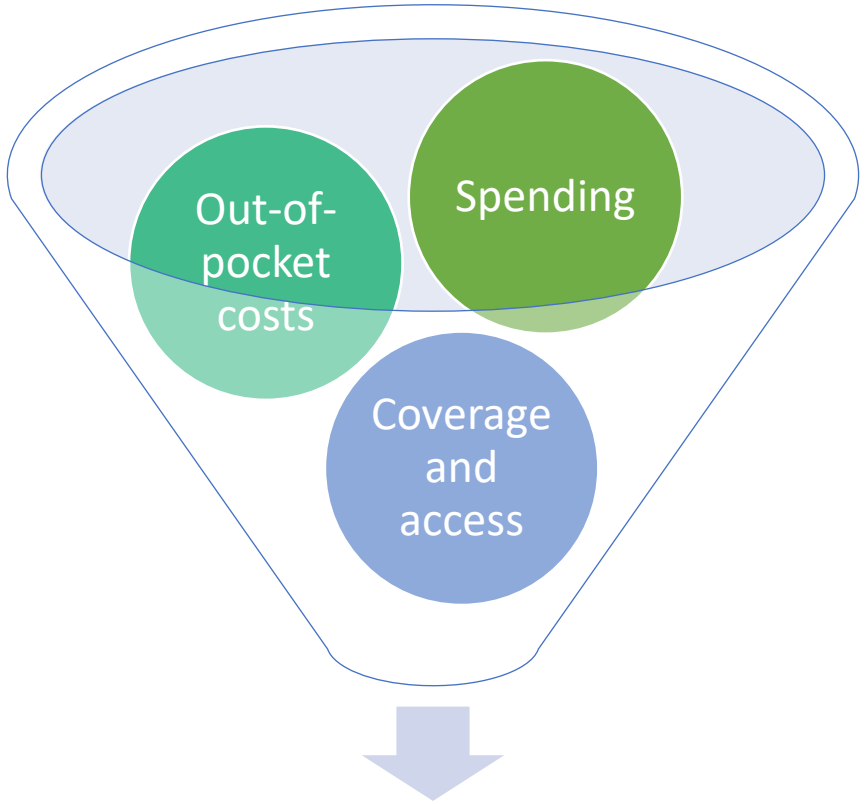
Following this comment period, the Board will weigh selection data and stakeholder comments to select the **final list of drugs to review**.

Attachment A:
March 25, 2024 Preliminary Identification of Potential Drugs for Referral to the Stakeholder Council

Drug	Drug Name	Dose Strength	Dose Strength Unit of Measure
BIKTARVY	Biktarvy	50-200-25	MG
DUPIXENT	Dupixent	300	MG/2ML
	Dupixent	200	MG/1.14ML
FARXIGA	Farxiga	10	MG
	Farxiga	5	MG
JARDIANCE	Jardiance	25	MG
	Jardiance	10	MG
OZEMPIC	Ozempic (0.25 or 0.5 MG/DOSE)	2	MG/1.5ML
	Ozempic (1 MG/DOSE)	2	MG/1.5ML
	Ozempic (1 MG/DOSE)	4	MG/3ML
	Ozempic (2 MG/DOSE)	8	MG/3ML
SKYRIZI	Skyrizi	150	MG/ML
	Skyrizi (150 MG Dose)	75	MG/0.83ML
	Skyrizi Pen	150	MG/ML
TRULICITY	Trulicity	0.75	MG/0.5ML
	Trulicity	1.5	MG/0.5ML
	Trulicity	3	MG/0.5ML
	Trulicity	4.5	MG/0.5ML
VYVANSE	Vyvanse	70	MG
	Vyvanse	60	MG
	Vyvanse	50	MG
	Vyvanse	40	MG
	Vyvanse	30	MG
	Vyvanse	20	MG

Maryland PDAB. March 25, 2024 Meeting Agenda; Maryland PDAB. Cost Review Process Overview. 2024.

The Central PDAB Challenge



Does the drug create excess costs to patients?

Questions?