Oregon Prescription Drug Affordability Board

Policy Memorandum

To: Oregon Prescription Drug Affordability Board
From: Cortnee Whitlock, program and senior policy analyst
Re: Orphan drug review refinements
Date: Sept. 8, 2026

Summary

This memorandum presents policy options for the Oregon Prescription Drug Affordability Board to address a need to update the board's statutory language related to drugs with an FDA orphan designation. ORS 646A.694 establishes the framework for identifying and reviewing prescription drugs that may create affordability challenges. ORS 646A.694(2) does not explicitly address whether the exemption for a drug designated by the U.S. Food and Drug Administration for a rare disease or condition applies to the entire drug or only to the orphan indication associated with the designation. However, OAR 925-200-0020 interprets the exemption as not applying to a drug's other FDA-approved indications for treatment of more common conditions.

A related implementation challenge is the limited ability to distinguish claims associated with orphan indications from claims associated with more common conditions. Pharmacy and medical claims do not always include sufficient diagnostic information to reliably identify the condition for which a drug was prescribed or administered.

Updating the statutory language would allow the board to distinguish between drugs approved exclusively for orphan indications and drugs approved for both orphan and non-orphan indications. Drugs approved exclusively for orphan indications could remain exempt from review, while drugs with broader FDA-approved uses could remain eligible for review based on their potential affordability effects on Oregon patients and the health care system. This refinement would preserve protections for treatments approved exclusively for rare diseases while preventing an orphan designation for one indication from excluding review of a drug that is also used for non-rare conditions. Refining ORS 646A.694(2) would improve the consistency and usefulness of the review process without creating a difficult claims data separation requirement for drugs with orphan indications.

Oregon's current framework

ORS 646A.694(1) requires the board to identify drugs that may create affordability challenges and evaluate specified factors. Subsection (2) provides that a drug designated by FDA under 21 U.S.C. 360bb, as a drug for a rare disease or condition is not subject to review.[1] The statute does not expressly state whether the exemption applies only to the use associated with the orphan designation or to the drug as a whole. ORS 646A.694(2)(n) interprets the exemption as indication-specific by providing that a drug is not exempt from review for its other indications.

OAR 925-200-0020(2)(n) clarifies the statutory language, indicating a drug is not exempt from an affordability review for those other indications.[2]

The current language creates three related problems:

- **Legal ambiguity.** The statute refers to a "drug" with a designation, while the rule treats the exemption as indication-specific. Pharmaceutical manufacturer representatives have argued that the statutory exemption applies to the entire product once the drug receives an orphan designation.[3]
- **Federal terminology mismatch.** FDA grants orphan designation for a specified rare disease or condition, and designation is separate from marketing approval. An already approved drug may later obtain orphan designation for an unapproved use and approved uses may change over time.
- **Data mismatch.** Drug spending is commonly recorded on the claim at the NDC level, while the diagnosis may be absent, incomplete, located on another claim, or insufficient to identify the reason for treatment. Requiring consistent attribution can make a mixed-indication review impossible even when the product has substantial non-orphan use.
- **Unclear object of the finding.** With a mixed-indication review, the board needs to state whether it is assessing the affordability of the entire product in Oregon, the affordability only for non-orphan uses, or both. Without that explanation, stakeholders can read and interpret the same evidence differently.

[1] Oregon Revised Statutes 646A.694(1). https://www.oregonlegislature.gov/bills_laws/ors/ors646a.html.
[2] Oregon Secretary of State Administrative Rules 925-200-0020. https://secure.sos.state.or.us/oard/displayDivisionRules.action?selectedDivision=7789.
[3] Pharmaceutical Research and Manufacturers of America, Letter to the Oregon Prescription Drug Affordability Board, February 9, 2026, https://dfr.oregon.gov/pdab/Documents/Letters/PhRMA-20260209.pdf.

Federal orphan designation

Federal law and regulation support treating orphan status as tied to a use or indication rather than as an immutable characteristic of the molecule. FDA explains that designation is granted to a drug or biologic to prevent, diagnose, or treat a rare disease or condition and is a separate process from marketing approval or licensing.[4] Requests for designation of a drug for a rare disease are made before the sponsor applies for marketing approval or licensing and must indicate that the drug is being or will be investigated for a rare disease or condition.[5]

In addition to new molecules, FDA regulations allow a sponsor to request orphan designation for a new use of an already marketed drug when the new use is being investigated and before seeking marketing approval for the new use.[6] They also provide that orphan drug exclusivity protects only the approved rare disease indication or use. FDA may approve the same drug for additional indications outside that protected use.[7]

That federal structure does not decide the meaning of Oregon's exemption; Oregon may define a broader molecule-wide exclusion. It does, however, give the Legislature a precise basis for clarifying whether it intends to exempt orphan-only products or every product that has ever received any orphan designation.

What other states are doing

State PDAB laws do not use one uniform approach. The most relevant distinction is whether orphan status bars review, triggers an additional process, or is simply one factor of the review. None of the statutes in Table 1 make diagnosis-level claim separation a condition of a board's authority.

[4] Designating an Orphan Product: Drugs and Biological Products. U.S. Food & Drug Administration, Aug. 12, 2024. https://www.fda.gov/industry/medical-products-rare-diseases-and-conditions/designating-orphan-product-drugs-and-biological-products.
[5] 21 USC 360bb: Designation of drugs for rare diseases or conditions. https://uscode.house.gov/view.xhtml?req=(title:21%20section:360bb
[6] 21 CFR § 316.23 Timing of requests for orphan-drug designation; designation of already approved drugs. https://www.ecfr.gov/current/title-21/chapter-I/subchapter-D/part-316/subpart-C/section-316.23.
[7] 21 CFR § 316.31 Scope of orphan-drug exclusive approval. https://www.ecfr.gov/current/title-21/chapter-I/subchapter-D/part-316/subpart-D/section-316.31

Table 1 How other state PDABs treat orphan designations

State	Treatment of orphan status	Considerations for Oregon
Colorado[8]	The board must consider whether a drug has orphan designation for one or more rare diseases and is approved for no other indications. If it is orphan-only, the board considers input from consumers and the Rare Disease Advisory Council. The council also provides input during affordability review.	Expressly identifies the orphan-only category and adds rare-disease stakeholder participation instead of creating a mixed-indication claims test. Extra review steps apply to drugs that are approved solely to treat rare diseases.
Washington[9]	Orphan drug status is one mandatory factor in an affordability review, alongside patient access, cost sharing, assistance programs, alternatives, and stakeholder input.	Preserves review authority while making orphan status part of context and deliberation.
Maryland[10]	The statute directs the board to review prescription drug products and determine whether use consistent with FDA labeling or standard medical practice creates affordability challenges. It contains no categorical orphan-drug exclusion.	Demonstrates a product-level affordability review model that does not categorically exclude drugs with orphan designations or make diagnosis-level claims attribution a condition of review.
Minnesota[11,12]	The statute directs the board to identify and review prescription drug products under price and affordability criteria. It contains no categorical orphan-drug exclusion.	Demonstrates a product-level affordability review model that does not categorically exclude drugs with orphan designations or make diagnosis-level claims attribution a condition of review.

[8] Colorado Legislature Senate Bill 24-203. https://leg.colorado.gov/bill_files/47099/download.
[9] Washington State Legislature Chapter 70.405 Prescription Drug Affordability Board. https://app.leg.wa.gov/RCW/default.aspx?cite=70.405&full=true
[10] Maryland General Assembly §21–2C–08 and §21–2C–09. https://mgaleg.maryland.gov/mgawebsite/Laws/StatuteText?article=ghg&enactments=false&section=21-2C-09
[11] 2025 Minnesota Statutes. 62J.90 Prescription drug price information; decision to conduct cost review. https://www.revisor.mn.gov/statutes/cite/62J.90.
[12] 2025 Minnesota Statutes. 62J.91 Prescription drug product reviews. https://www.revisor.mn.gov/statutes/cite/62J.91

Recommended Oregon drug review framework

1. Classify the product before selection

Oregon can address the statutory ambiguity without creating a separate claims-attribution requirement by establishing three review categories. The categories would be based on the drug's FDA-approved indications and any corresponding orphan designations at the time the board selects drugs for review.

Table 2 Oregon PDAB proposed orphan drug review categories

Category	Definition	Treatment
Orphan-only	Every FDA-approved indication for the product is within the scope of an FDA orphan designation at the time of the board's selection for review.	Not eligible for affordability review. Document the basis for exclusion.
Mixed indication	At least one FDA-approved indication is within an orphan designation and at least one FDA-approved indication is not at the time of selection for review.	Eligible for affordability review.
Non-orphan	No FDA-approved indication is within an orphan designation at the time of selection for review.	Eligible for affordability review.

2. Proposed statutory clarification

The following proposed policy language could be used to implement this change

Current text of ORS 646A.694(2):

> (2) 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.

Proposed revision to ORS 646A.694(2):

> (2)(a) A prescription drug is not subject to review under subsection (1) of this section if, at the time the board selects drugs for review, every indication for which the drug is approved by the United States Food and Drug Administration is within the scope of a designation granted under 21 U.S.C. 360bb for a rare disease or condition;
>
> (b) A prescription drug is subject to review if the drug is approved by the United States Food and Drug Administration for at least one indication that is not within the scope of a designation described in paragraph (a) of this subsection; and

(c) In reviewing a prescription drug described in paragraph (b), the board shall consider any orphan designations and the effect of the drug's price on access for patients with rare diseases. The board may use indication-specific utilization or claim information when reliable and available, but the absence of such information does not preclude review of the prescription drug.

Conclusion

The current language in ORS 646A.694(2) does not clearly distinguish between a drug approved exclusively to treat rare diseases and a drug that has an orphan designation for one indication and is also approved to treat one or more non-rare conditions. This lack of distinction has resulted in differing interpretations of the board's authority and has complicated the review of mixed-indication drugs when available claims data cannot reliably identify the condition associated with every prescription or administration.

Clarifying ORS 646A.694(2) as proposed would preserve the exemption for drugs whose FDA-approved indications are exclusively associated with treatment of rare diseases or conditions while allowing PDAB to review drugs that also have FDA-approved non-orphan indications. The updated approach would recognize the indication-specific nature of the federal orphan-drug framework and include drugs that have approved non-orphan indications consistent with approaches used by other state prescription drug affordability boards.

The proposed clarification would strengthen the consistency, transparency, and usefulness of Oregon's affordability review process. It would restrict review of treatments developed exclusively for rare diseases while ensuring that an orphan designation for one indication does not prevent the board from examining the broader affordability effects of a drug also used to treat non-rare conditions. Analysis conducted by the board in 2026 demonstrates that drugs with multiple indications may have high levels of use for non-rare conditions. Because the board does not currently establish upper payment limits or otherwise regulate prescription drug prices, the review would provide information for the board's findings and legislative recommendations without directly changing the drug's price, reimbursement, coverage, or availability.