Oregon Prescription Drug Affordability Board

Oregon PDAB orphan drug review refinement

Recommendation

The Oregon Prescription Drug Affordability Board (PDAB) recommends amending ORS 646A.694(2) to clarify when a prescription drug with an FDA orphan designation is eligible for an affordability review. The amendment would align Oregon law with the indication-specific nature of federal orphan drug designation, eliminate the need to assign every claim to an orphan or non-orphan indication, and require the board to consider the distinctive clinical, access, and affordability circumstances affecting patients with rare diseases.[1] The board has identified two potential statutory approaches. Both would clarify the review process and require consideration of these rare disease circumstances.

Problem to solve

Current ORS 646A.694(2) provides that a drug designated by FDA under 21 U.S.C. 360bb for a rare disease or condition is not subject to review.[2] The statute does not expressly state whether the exemption from review applies to the entire drug once any orphan designation is granted or only to the indication covered by the orphan designation.

OAR 925-200-0020(2)(n) treats the exemption as indication-specific and allows review of other indications.[3] In practice, however, pharmacy claims often lack diagnosis information and medical claims may have only limited diagnosis information. Requiring PDAB to separate utilization and spending for every orphan and non-orphan indication may prevent a meaningful affordability review of a drug with substantial use for common conditions.

Drugs approved to treat common conditions may also have FDA orphan designations for rare diseases or conditions. Of the 137 drugs on PDAB's preliminary list for 2026 affordability reviews, 59 (43 percent) had one or more FDA orphan drug designations. Among those preliminary list drugs 20 drugs had net annual spending exceeding $10 million in 2024 Drug Price Transparency (DPT) program data, 12 (60 percent) had at least one FDA orphan designation.[4] These findings illustrate the relevance of orphan designation to PDAB's review process and support clarifying how it affects review eligibility.

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[1] FDA, Designating an Orphan Product: https://www.fda.gov/industry/medical-products-rare-diseases-and-conditions/designating-orphan-product-drugs-and-biological-products

[2] ORS 646A.694: https://www.oregonlegislature.gov/bills_laws/ors/ors646a.html; 21 U.S.C. § 360bb: https://uscode.house.gov/view.xhtml?req=21+U.S.C.+%EF%BF%BD+360bb&f=treesort&fq=true&num=5&hl=true&edition=prelim&granuleId=USC-prelim-title21-section360bb

[3] OAR 925-200-0020: https://secure.sos.state.or.us/oard/displayDivisionRules.action?selectedDivision=7789

[4] 2026 Drug Review Preliminary List, v05: https://dfr.oregon.gov/pdab/Pages/data.aspx

Policy framework

Regardless of which exemption approach is selected, the statutory amendment would:

- Define eligibility using the drug's FDA-approved indications and corresponding orphan designations as of the date PDAB selects the drug for review.
- Confirm that an FDA orphan designation applies to a disease, condition, or indication and does not necessarily define the entire product.
- Allow PDAB to use indication-specific utilization or claims information when reliable and available, without making complete diagnosis-level attribution a condition of review.
- Require considerations of orphan status, disease rarity, patient access, therapeutic alternatives, cost sharing, assistance programs, stakeholder input, and relevant evidence limitations.
- Allow PDAB to distinguish quantitative evidence, qualitative evidence, and unresolved questions.
- Clarify that an affordability review is advisory and does not determine coverage, formulary placement, prior authorization, reimbursement, market availability, or price-setting decisions.

Retain an exemption for orphan-only drugs

Policy intent

A drug would be excluded from PDAB affordability review only when every FDA-approved indication is within the scope of an FDA orphan designation. A drug with at least one approved non-orphan indication would remain eligible for review, without requiring PDAB to separate claims by indication. A drug with an FDA orphan designation that is not yet approved to treat the orphan designated indication would also remain eligible for review.

Argument for alternative

- Excludes treatments developed and approved exclusively for rare diseases from PDAB affordability review.
- Prevents one orphan designation from excluding a drug from PDAB affordability review that is also approved and widely used for common conditions.
- Offers a focused legislative clarification that will substantially reduce the current data analysis burden.

Argument against alternative

Affordability challenges associated with orphan-only drugs would remain outside PDAB's individual-drug review authority, including high-cost therapies with substantial effects on patients or the health care system. Alternative 1 would provide greater scrutiny of affordability and access challenges that affect patients with rare diseases.

Draft statutory concept

The current ORS 646A.694(2) would be amended to read substantially as follows:

(2)(a) A prescription drug is not subject to review under subsection (1) of this section if, as of the date the board selects the drug 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 not excluded from review under this subsection 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 granted under 21 U.S.C. 360bb for a rare disease or condition.

(c) In reviewing a prescription drug described in paragraph (b) of this subsection, the board shall consider the orphan designation or designations and any issues related to affordability of and access to the drug for patients with rare diseases or conditions.

(d) The board may review a prescription drug without attributing utilization or spending to each orphan and non-orphan indication. In its review, the board shall identify limitations in the data and resulting findings when utilization or spending cannot be attributed to a specific indication.

(e) An orphan designation for an indication that has not been approved by the United States Food and Drug Administration does not, by itself, exclude a prescription drug from review under this subsection.

Requested legislative action

Before finalizing the legislative recommendation for inclusion in the 2026 annual report, the board will select one of the two statutory approaches presented in this draft.

Amend ORS 646A.694(2) to clarify PDAB's authority to:

- Review prescription drugs with FDA orphan designations;
- Eliminate indication-level claims attribution as a prerequisite to review; and
- Require meaningful consideration of rare-disease affordability and access.