July 14, 2025

Oregon Prescription Drug Affordability Review Board
Labor & Industry Building
350 Winter Street, NE
Salem, OR 97309-0405

RE: Selection of Sacubitril-Valsartan for Oregon PDAB

Dear Members of the Board,

The Partnership to Advance Cardiovascular Health (PACH) is a nonprofit cardiovascular stakeholder coalition of patient, provider, and advocacy organizations dedicated to advancing public policies and practices that accelerate innovation and improve cardiovascular health for heart patients. As a platform for the 20 member organizations that collaborate with us, PACH advocates at the federal, state, and health plan levels for reforms that increase access to care for patients with cardiovascular and related conditions.

As we are keenly aware that high medication costs complicate access for many patients, we agree with the Oregon Prescription Drug Review Board's goal of making medications affordable for Oregonians. It is our organizational goal to promote both access and innovation in cardiovascular science and medicine so that we can both save and improve lives. We are writing today to provide context for one of the medications being reviewed by the Oregon PDAB.

The Cardiovascular Disease Burden:

Cardiovascular disease remains the second leading cause of death in Oregon, and the number one cause of death nationally. America's progress in decreasing the death rate due to heart disease and stroke has stalled. The death rate for cardiovascular disease, including heart disease and strokes, has fallen just 4% since 2011 after dropping more than 70% over the prior six decades. Particularly alarming, certain age and demographic groups are seeing increases in the rate of cardiovascular-related death. These trends are worse for minority communities, rural communities and those with lower socioeconomic status. Ensuring that patients have access to cardiovascular primary and secondary preventative treatment, and promoting new innovation and modalities for treatment, are of the utmost importance to PACH and our partners.

Innovation in Cardiovascular Disease Management

The cardiovascular medications being considered by the Oregon PDAB represent some of the best relatively recent pharmaceutical interventions cardiovascular medicine has to offer. Every cardiovascular medication on the PDAB subset list provides immense value not only for patients but for the healthcare system as a whole. Sacubitril-Vasartan provides an excellent example.

Sacubitril-Valsartan is a treatment for chronic heart failure and is indeed the only angiotensin receptor-neprilysin inhibitor (ARNi) approved by the FDA for the treatment of heart failure – there are currently no other alternatives. For the 6.2 million Americans impacted by heart failure, many of those who take sacubitril-valsartan see a meaningful risk reduction for death and hospitalization – this is a remarkable feat in cardiology. An important study looking at the cost effectiveness of sacubitril-valsartan showed that inpatient treatment was cost saving to the healthcare system.[3] Not only is this medication effective, but it limits costs associated with heart failure on the entire healthcare system.

Comprehensive Approach to Affordability and Access:

All of the cardiovascular treatments being reconsidered by the Oregon PDAB have already been subject to the Centers for Medicare and Medicaid Services "Maximum Fair Price" drug negotiations that were authorized by the Inflation Reduction Act. During the comment period of those negotiations, PACH, our clinician partners and patient advocacy organizations all supported the broad aim of making medications more affordable *for Medicare recipients.* We expressed concern, however, that patients would not actually realize the lower prices as set by the government, and that, without a comprehensive assessment of the medication pipeline, affordability would not be achieved.

We also expressed concern that utilization management of these negotiated medications would increase, which can have devastating consequences for patients – particularly patients on anticoagulant therapy. Cardiovascular medicine has seen remarkable increases in prior authorization and step therapy protocols in recent years, far outpacing other disease states. Clinicians and patients bear the majority of the burden of these oftentimes unnecessary administrative hurdles.

We believe that these same concerns translate to the state level and that Oregon's PDAB could frustrate both access and affordability for patients.

Actions to Protect Patients and Increase Affordability and Access

A more holistic approach to address affordability should include reviewing health insurer and pharmacy benefit manager practices like step-therapy and prior authorization protocols, prohibiting spread pricing, prohibiting co-pay accumulator or "maximizer" programs so that any dollars spent toward a patient's deductible count toward their out-of-pocket limit, and requiring pass-through savings directly to patients. Until more transparency is brought to bear on the medication pipeline, we believe efforts such as the Oregon PDAB's will not achieve their stated goal.

PACH appreciates the Board's work in addressing prescription drug affordability. At this time, we ask that the board remove the cardiovascular medications from the prescription drug subset list as these medications have all been subject to "Maximum Fair Price" negotiations in the Inflation Reduction Act (IRA) and have proven to reduce the economic impact on the healthcare system. Evidence suggests that further "review" will not achieve the PDAB's stated goal. We submit that the above-mentioned actions would do much more to create transparency in the medication delivery pipeline and more effectively support patient affordability.

Respectfully Submitted,

Sarah Hoffman
Senior Director
Partnership to Advance Cardiovascular Health

1. Graham DJ, Baro E, Zhang R, Liao J, Wernecke M, Reichman ME, Hu M, Illoh O, Wei Y, Goulding MR, Chillarige Y, Southworth MR, MaCurdy TE, Kelman JA. Comparative Stroke, Bleeding, and Mortality Risks in Older Medicare Patients Treated with Oral Anticoagulants for Nonvalvular Atrial Fibrillation. Am J Med. 2019 May;132(5):596-604.e11. doi: 10.1016/j.amjmed.2018.12.023. Epub 2019 Jan 9. PMID: 30639551.
2. Duvalyan, A., Pandey, A., Vaduganathan, M., Essien, U. R., Halm, E. A., Fonarow, G. C., & Sumarsono, A. (2021). Trends in anticoagulation prescription spending among Medicare Part D and Medicaid beneficiaries between 2014 and 2019. *Journal of the American Heart Association*, *10*(24). https://doi.org/10.1161/jaha.121.022644
3. Virani, S. S., Alonso, Á., Benjamin, E. J., Bittencourt, M. S., Callaway, C. W., Carson, A. P., Chamberlain, A. M., Chang, A. R., Cheng, S., Delling, F. N., Djoussé, L., Elkind, M. S., Ferguson, J. F., Fornage, M., Khan, S. S., Kissela, B. M., Knutson, K. L., Kwan, T., Lackland, D. T., . . . Tsao, C. W. (2020). Heart disease and stroke statistics—2020 Update: A report from the American Heart Association. *Circulation*, *141*(9). https://doi.org/10.1161/cir.0000000000000757

July 14, 2025

Oregon Prescription Drug Affordability Review Board
Labor & Industry Building
350 Winter Street, NE
Salem, OR 97309-0405

RE: Selection of Cardiovascular Medications for the Oregon Prescription Drug Affordability Review

Dear Members of the Board,

On behalf of the Preventive Cardiovascular Nurses Association, thank you for the opportunity to comment on the July 16th Oregon Prescription Drug Affordability Board 2025 meeting. PCNA writes to reiterate the importance of ensuring patient access to the three crucial cardiovascular medications from the 2025 prescription medication list for the Oregon Prescription Drug Affordability Review Board (PDAB) review.

The Preventive Cardiovascular Nurses Association (PCNA) was founded in 1992 by a group of nurses working in cardiovascular disease management and has since expanded into 41 chapters across the United States, including the Pacific Northwest Chapter, which serves Portland, Oregon and surrounding counties. As PCNA has expanded, we are the leading nursing organization dedicated to preventing and managing cardiovascular disease. Our mission is to support the utilization and dissemination of research and support evidence-based practice in cardiovascular risk reduction and disease management as a means to ensure patient access to treatment and empower nurses as leaders in cardiovascular care.

PCNA acknowledges the barriers to access for many patients that arise from high medication costs, and we align with the Oregon Prescription Drug Affordability Board's intention of ensuring affordable medications for Oregonians. As the board undertakes this effort, PCNA urges a focus not only on top-line cost, but also on patient benefit and healthcare system cost savings from access to these therapies.

According to the Centers for Disease Control and Prevention (CDC), cardiovascular disease remains the second leading cause of death in Oregon[1], and the number one cause of death in the United States.[2] Notably, the cardiovascular disease (CVD) mortality rate in the United States declined by over 20% from 1980 to 2010. However, in the following years, the rate of CVD-related deaths has increased, particularly for adults aged 35 to 64.[3] PCNA believes that ensuring patients have access to preventive cardiovascular care and maintaining healthcare providers' ability to treat their patients with the best available treatment modalities are of utmost importance.

The Oregon PDAB has selected three cardiovascular medications for affordability reviews in 2025. The medications selected represent some of the most effective recent pharmaceutical interventions in cardiovascular medicine, providing immense value not only for patients but for the healthcare system as a whole.

As the Board considers its reviews, PCNA would like to provide comments on the following cardiovascular medications selected for affordability review: Sacubitril-Valsartan (Review Group 1); Apixaban (Review Group 2); and Rivaroxaban (Review Group 2).

Sacubitril-Valsartan:
Sacubitril-Valsartan is a treatment for chronic heart failure and is indeed the only angiotensin receptor-neprilysin inhibitor (ARNi) approved by the FDA for the treatment of heart failure. When compared to angiotensin-converting-enzyme inhibitor (ACEI), also used to treat heart failure, sacubitril-valsartan demonstrated significant reductions in cardiovascular death and heart failure hospitalizations (21%), further emphasizing the value of sacubitril-valsartan for patients living with heart failure.[4] Moreover, a study using the Kansas City Cardiomyopathy Questionnaire (KCCQ), the most well-validated resource in heart failure, to evaluate quality of life revealed that patients being treated with sacubitril-valsartan experienced improvements in all domains, including physical limitations, quality of life, self-efficacy, social limitations, and symptom stability.[5] In a broader context, the efficacy of sacubitril-valsartan in heart failure management helps reduce healthcare costs associated with hospitalizations and patient interactions with the healthcare system.

In summary, recent data shows that sacubitril-valsartan significantly reduces patients' risk of hospitalization, improves overall quality of life, and therefore reduces the burden placed on the healthcare system.

Apixaban:
Apixaban is a factor Xa inhibitor, direct oral anticoagulant (DOAC) used for the treatment of patients with nonvalvular atrial fibrillation (AF), deep vein thrombosis (DVT) and pulmonary embolism (PE).[6] Initially approved by the U.S. Food and Drug Administration (FDA) in 2012, apixaban helps reduce the risk of stroke and blood clots.[7] The drug is also approved for reducing the risk of recurrent DVT and PE after initial therapy.

Efficacy studies on DOACs like apixaban have demonstrated a significant decrease in patients' risk of developing a stroke or systemic embolism, as well as a 15% decreased risk of experiencing a major bleeding episode.[8] Apixaban and other DOACs hold value in minimizing healthcare cost burdens for both patients and the broader healthcare system.

Rivaroxaban:
Rivaroxaban is a factor Xa inhibitor, DOAC used to treat and manage DVT, as well as to prevent blood clots and stroke in patients who have undergone elective orthopedic surgery.[9] In addition to the previously mentioned indications, rivaroxaban has received FDA approval for secondary prevention of acute coronary syndrome (ACS) or peripheral artery disease (PAD).[10] In addition to other DOACs, rivaroxaban has been proven to reduce patients' risk of thromboembolic stroke by 20-29% and decrease risk of death by 19-34% when compared to current generic medications.[11]

By decreasing patients' risk of experiencing adverse events such as strokes, PE, and DVT, rivaroxaban and other DOACs protect patients and help reduce healthcare costs.

Each of the three medications are essential tools for cardiovascular care. PCNA encourages the Board to consider the perspectives of both patients and providers when conducting affordability reviews of the cardiovascular drugs selected.

The cardiovascular treatments being considered by the Board already face access limitations in the form of utilization management[12], which can have devastating consequences for patients – especially for treatments with no other alternative, such as sacubitril-valsartan. Use of utilization management for single-source cardiovascular brand drugs in the commercial market showed increases of over 450% from 2014 to 2020.[13] Excessive use of utilization management tools harms patient access and limits the benefits that medicines have for patients and in controlling overall healthcare costs. A determination of unaffordability could lead to more restricted access while not guaranteeing any patient affordability savings.

PCNA appreciates the Board's work in addressing prescription drug affordability. At this time, we ask that the Board keep in mind the importance of maintaining access to the cardiovascular treatments selected for patient outcomes, as these medications have been proven to help patients and reduce the economic impact on the healthcare system.

Sincerely,

Sue Koob
CEO
Preventive Cardiovascular Nurses Association

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[1] Centers for Disease Control and Prevention. (2024, October 3). *Oregon*. Centers for Disease Control and Prevention. https://www.cdc.gov/nchs/pressroom/states/oregon/or.htm.

[2] Centers for Disease Control and Prevention. (2025, June 5). *FASTSTATS - leading causes of death*. Centers for Disease Control and Prevention. https://www.cdc.gov/nchs/fastats/leading-causes-of-death.htm.

[3] Virani, S. S., Alonso, A., Benjamin, E. J., Bittencourt, M. S., Callaway, C. W., Carson, A. P., Chamberlain, A. M., Chang, A. R., Cheng, S., Delling, F. N., Djousse, L., Elkind, M. S. V., Ferguson, J. F., Fornage, M., Khan, S. S., Kissela, B. M., Knutson, K. L., Kwan, T. W., Lackland, D. T., … Tsao, C. W. (2020a). Heart disease and stroke statistics—2020 update: A report from the American Heart Association. *Circulation*, *141*(9). https://doi.org/10.1161/cir.0000000000000757.

[4] McMurray JJ, Packer M, Desai AS, Gong J, Lefkowitz MP, Rizkala AR, Rouleau JL, Shi VC, Solomon SD, Swedberg K, Zile MR; PARADIGM-HF Investigators and Committees. Angiotensin-neprilysin inhibition versus enalapril in heart failure. *N Engl J Med*. 2014;*371*:993–1004. doi: 10.1056/NEJMoa1409077.

[5] Lewis, E. F., Claggett, B. L., McMurray, J. J., Packer, M., Lefkowitz, M. P., Rouleau, J. L., Liu, J., Shi, V. C., Zile, M. R., Desai, A. S., Solomon, S. D., & Swedberg, K. (2017). Health-related quality of life outcomes in paradigm-HF. *Circulation: Heart Failure*, *10*(8). https://doi.org/10.1161/circheartfailure.116.003430.

[6] Lewis, E. F., Claggett, B. L., McMurray, J. J., Packer, M., Lefkowitz, M. P., Rouleau, J. L., Liu, J., Shi, V. C., Zile, M. R., Desai, A. S., Solomon, S. D., & Swedberg, K. (2017). Health-related quality of life outcomes in paradigm-HF. *Circulation: Heart Failure*, *10*(8). https://doi.org/10.1161/circheartfailure.116.003430.

[7] *Id*.

[8] Makam, R. C., Hoaglin, D. C., McManus, D. D., Wang, V., Gore, J. M., Spencer, F. A., Pradhan, R., Tran, H., Yu, H., & Goldberg, R. J. (2018). Efficacy and safety of direct oral anticoagulants approved for cardiovascular indications: Systematic Review and meta-analysis. *PLOS ONE*, *13*(5). https://doi.org/10.1371/journal.pone.0197583.

[9] Singh, R. (2023, April 17). *Rivaroxaban*. StatPearls [Internet]. https://www.ncbi.nlm.nih.gov/books/NBK557502/.

[10] *Id*.

[11] David J. Graham MD, Hernandez, I., Lip, G., Pisters, R., Connolly, S., Patel, M., Granger, C., Giugliano, R., Lip, G., Noseworthy, P., Deitelzweig, S., Adeboyeje, G., … Ray, W. (2019, January 9). *Comparative stroke, bleeding, and mortality risks in older Medicare patients treated with oral anticoagulants for nonvalvular atrial fibrillation*. The American Journal of Medicine. https://www.sciencedirect.com/science/article/abs/pii/S0002934319300518.

[12] Juliette Cubanski, A. D. (2023, September 26). *How Medicare's New Drug Price Negotiation Program could expand access to selected drugs*. KFF. https://www.kff.org/medicare/issue-brief/how-medicares-new-drug-price-negotiation-program-could-expand-access-to-selected-drugs/#:~:text=Six%20of%20the%2010%20selected,out%2Dof%2Dpocket%20costs.

[13] Meyer, T., Yip, R., Hamilton, R., Mengesha, Y., & Santiesteban, D. (2021, November 24). *Utilization management trends in the commercial market, …* Avalere Health. https://advisory.avalerehealth.com/wp-content/uploads/2021/11/UM-Trends-in-the-Commercial-Market.pdf.

July 14, 2025

Oregon Prescription Drug Affordability Board
c/o Department of Consumer and Business Services
350 Winter Street NE
Salem, OR 97309-0405

TO: Members of Oregon Prescription Drug Affordability Board

As a physician with decades of experience caring for patients whose families often struggle to access and afford necessary medications, I am deeply concerned that the Board's process for selecting medications and conducting affordability reviews will leave Oregon patients without access to necessary medications.

I am a board-certified pediatrician and pediatric rheumatologist who has spent my career caring for young people with chronic or disabling conditions. Many of my patients, including those with juvenile idiopathic arthritis and lupus, rely on specialized, innovative, expensive therapies. My primary focus is always ensuring the well-being of my patients, but I fear that the Board's decisions do not reflect this same mandate.

The Board's search for "therapeutic alternatives" is fundamentally misguided and dangerous for patients for whom substitution is not clinically appropriate due to unique medical conditions or treatment needs. The criteria for selecting these so-called "alternatives" often fail to account for the complexities of individual patient care. Unilaterally designating certain medications as "therapeutic alternatives" fundamentally disrupts the physician's ability to exercise their medical expertise in concert with their patient.

Healthcare providers like myself consider therapeutic equivalents when considering medication substitutions as a matter of standard practice, but therapeutic alternatives do not constitute therapeutic equivalents. Even if medications are in the same "therapeutic category", their different modes of action are considered when making individualized therapeutic decisions. It is not unexpected, therefore, that "therapeutic alternatives" within drug categories are not equivalent and result in patients only responding to specific medications and not others within a category. Patients who suffer from complex chronic conditions, such as rheumatoid arthritis and other rheumatologic diseases, require continuity of care to successfully manage their conditions. Policymakers have no business overriding their doctor's prescribing recommendations.

Further, I find the lack of consideration of the real-world consequences of a UPL problematic. We have seen that the creation of the Maximum Fair Price (MFP) within the Inflation Reduction Act has resulted in a 32% increase in out-of-pocket costs to patients.[1] Since a UPL creates a similar situation to the MFP, there is no reason not to expect a similar consequence within Oregon. Similarly, NCPA has reported that many of its member pharmacies will not be carrying the medications with a MFP because they cannot afford to do so. This too is likely to occur in Oregon[1]. We also know that insurers and PBMs will likely adjust their formularies if the UPL reduces their profits by shifting such a medication to a higher tier or excluding it from the formulary; what will be the Board's response to such actions?

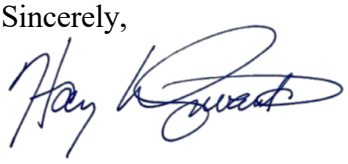
The opaque evaluation process of collected data further undermines confidence in the affordability review process. Without detailed definitions, methodologies or standards for assessing therapeutic alternatives and other critical factors, the Board risks decisions that do not adequately reflect real-world patient experiences or clinical realities. Establishing clear, consistent processes and ensuring transparency in decision-making are essential steps toward improving access to affordable medications for those who depend on them. While you are doing your best to be transparent, more time and extensive public contributions and deliberations will only improve your process.

The proposed list of potential therapies for affordability review is extensive and could significantly impact Oregon patients across a wide range of disease states. I am deeply concerned about the potential predictable and unintended consequences of such evaluations, especially when conducted under short timelines and without sufficient public input.

I share your goal to lower prescription drug costs, but the current process risks limiting access to essential medications. Physicians and patients are eager to collaborate with the Board to ensure affordability decisions reflect real-world patient needs on a more thoughtful, patient-centered approach. As it stands now, the Board's actions could inadvertently restrict access to medications for those who need them most in Oregon.

Thank you for your attention to this critical issue.

Sincerely,



Harry L. Gewanter, MD, FAAP, MACR
Board Member, Let My Doctors Decide Action Network
President, Virginia Society of Rheumatology

[1] https://pioneerinstitute.org/the-inflation-reduction-act-ira-overview/

The Headache & Migraine Policy Forum

July 15, 2025

Shelley Bailey, MBA, Chair
Oregon Prescription Drug Affordability Board
Department of Consumer and Business Services
350 Winter Street NE
Salem, OR 97309-0405

RE: Comments on Patient Access and Migraine Treatments Under Consideration for Affordability Review

Dear Chair Bailey and Members of the Oregon Prescription Drug Affordability Board:

The Headache and Migraine Policy Forum (HMPF) appreciates the opportunity to provide comments regarding the Oregon Prescription Drug Affordability Board's list of drugs selected for affordability review. We understand that four migraine therapies are currently under consideration for review and believe that including these treatments is misguided and ill-advised for several reasons, given the critical role they play in managing a disabling neurological disease.

HMPF is a group of diverse stakeholders seeking to advance public policies and practices that promote access to care for persons living with headache disorders and migraine disease. Migraine is not only one of the most common neurological conditions affecting more than 40 million Americans, with an estimated 640,000 of which are Oregonians. It is also the second leading cause of disability globally. Despite its significant burden, migraine is frequently underdiagnosed, undertreated, and often mischaracterized as a lifestyle issue instead of a serious neurological disease.

Thankfully, after more than 25 years without therapies specifically developed for migraine disease, innovative treatments have finally transformed care for many patients. However, these therapies are only effective if patients can access and can bear these costs. Like other stakeholders in the chronic disease community, we are concerned that affordability policies, if not carefully designed and implemented, could inadvertently limit patient access through restrictive coverage policies or utilization management, such as step therapy, prior authorization, or non-medical switching.

As a community, we share concern that affordability reviews can lead to direct reductions in patient out-of-pocket costs, that such policies may result in narrowed access or the steering of patients to less effective alternatives, and that patients with a heterogenous and complex disease such as migraine may not be afforded sufficient engagement throughout such a process.

Moreover, migraine imposes an enormous economic and social burden. Inadequate access to effective treatment can lead to frequent ER visits, missed work, and long-term disability. Migraine imposes more than $78 billion in annual economic burden in the United States, exceeding those of epilepsy, asthma, and ovarian cancer combined. In fact, individuals with chronic migraine can lose tens of thousands of dollars annually in lost productivity and face up to $300,000 in lost lifetime earnings. Migraine patients are the fourth highest users of the emergency department.

In addition, research shows that coverage barriers — not drug efficacy — are often the limiting factor in treatment access. Formulary exclusions, prior authorization, and step therapy delays prevent patients from obtaining the therapy selected by their provider, increasing the risk of poor outcomes and avoidable costs elsewhere in the healthcare system.

We are also concerned that the current review process lacks a mechanism to protect access for patients who rely on therapies deemed "unaffordable." Without protections in place, affordability reviews could lead to increased utilization management or the removal of therapies from preferred formularies, leaving patients with fewer, often less effective, options.

Migraine treatment is not one-size-fits-all. Patients respond differently to medications depending on their symptom patterns, comorbidities, and past treatment experience. As such, decisions that prioritize cost above clinical appropriateness risk undermining personalized care — especially in an area where therapeutic innovation is still emerging.

We strongly urge the PDAB to incorporate these considerations into your review process and to ensure that Oregon patients with migraine and other disabling conditions are not left behind in efforts to reduce costs.

While we appreciate the Board's work, we do not believe it is appropriate or timely for migraine therapies to be subject to an affordability review. Thank you for your attention to this request.

Sincerely,

Lindsay Videnieks

Executive Director
The Headache & Migraine Policy Forum
lindsay@headachemigraineforum.org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AT SCHOOL


MIGRAINE
AT WORK


Migraine
Meanderings


MIGRAINE WORLD SUMMIT


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HEADACHE
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U.S. PAIN
FOUNDATION


WORLD HEALTH EDUCATION
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VIA Electronic Delivery

July 14, 2025

Oregon Prescription Drug Affordability Board
Department of Consumer and Business Services
350 Winter Street NE
Salem, OR 97309-0405

Re: July 2025 Oregon Prescription Drug Affordability Board Meeting

Dear Prescription Drug Affordability Board Members and Staff:

The Biotechnology Innovation Organization (BIO) appreciates the opportunity to provide comments for the Oregon Prescription Drug Affordability Board (PDAB)'s July 2025 meeting.

BIO is the premier biotechnology advocacy organization representing biotech companies, industry leaders, and state biotech associations in the United States and more than 35 countries around the globe. BIO members range from biotech start-ups to some of the world's largest biopharmaceutical companies – all united by the same goal: to develop medical and scientific breakthroughs that prevent and fight disease, restore health, and improve patients' lives. BIO also organizes the BIO International Convention and a series of annual conferences that drive partnerships, investment, and progress within the sector. Learn more at bio.org.

At the June 2025 Oregon PDAB meeting, a board member indicated a desire to recommend to the legislature that the orphan drug exemption be removed from Oregon PDAB's review affordability process. These comments were extremely concerning for the entire patient community and represents a key misunderstanding of the critical role that the orphan drug exemption plays in ensuring patient access to life-saving treatments for individuals with rare diseases. **BIO strongly opposes any recommendation to alter or remove the orphan drug exemption, which would threaten the delicate ecosystem of innovation that makes these therapies possible.**

The EveryLife Foundation's "National Economic Burden of Rare Disease Study" found that rare diseases affect over 30 million Americans and imposed nearly $1 trillion in costs in 2019 alone[1]—highlighting both the scale of the burden and the urgent need for treatment innovation. Many rare disease patients have few or no previously approved medicines for

[1] Yang G., Cintina I., Pariser A., Oehrlein E., Sulliva n J., Kennedy A. (2022). The national economic burden of rare disease in the United States in 2019. Orphanet Journal of Rare Diseases, 17:163.

Biotechnology Innovation Organization
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their conditions. With 95% of the 7,000+ known rare diseases lacking an FDA-approved therapy, continued investment in rare disease research and development is vital. Further, continued investment in post-marketing R&D helps uncover new uses or populations that benefit from existing drugs, expanding their value toward patients.

Applying traditional affordability assessments—designed for broader populations—to rare disease therapies, without accounting for their distinct challenges, can significantly restrict access. These therapies often serve small, medically complex populations and involve high development costs, yet they can deliver life-altering or even curative outcomes. Without a tailored review approach that reflects the realities of rare disease—including disease heterogeneity, long diagnostic journeys, and profound unmet need—Oregon risks discouraging innovation and denying patients access to critical treatments.

We respectfully urge the PDAB and state policymakers maintain the rare disease exemption from affordability review, which provides a critical lifeline for rare disease patients to access these important treatments.

In addition, we urge the OR PDAB to communicate a schedule for their reviews and to promptly provide details on what the process for stakeholder input will be moving forward. In the June 2025 meeting, Board members made comments about wanting to standardize their review criteria and identified a need to establish critical definitions for key terms, like affordability. These concerns are carried over from the OR PDAB's aborted 2024 process, and it seems prudent for the Board to address them in earnest before commencing with reviews in 2025.

Sincerely,

Primo J. Castro

Primo J. Castro
Director, State Government Affairs – Western Region

Chronic Disease Coalition

Oregon Prescription Drug Affordability Board (PDAB)
Department of Consumer and Business Services
350 Winter St. NE Room 410
Salem, OR 97309

July 21, 2025

Chair Bailey and members of the board,

On behalf of the Chronic Disease Coalition, thank you for the opportunity to provide our thoughts and feedback as the PDAB considers supporting legislation that would remove the rare disease exemption from your review process.

Founded in Oregon, the Chronic Disease Coalition is a national nonprofit organization dedicated to raising the patient voice and perspective in healthcare policymaking. Our patient volunteers represent both common diseases like diabetes and kidney disease and rare diseases such as Guillain-Barré, von Willebrand's Disease Type 2N, Partial Trisomy 8q and hypoparathyroidism.

We are especially attuned to the needs of our rare disease patients because they have a unique experience of the healthcare system. It is not an exaggeration to say that just getting to diagnosis is a tortured path. Symptoms are dismissed or misunderstood, and patients are often left to research and diagnose themselves. Then they have to find and connect with national expert providers and build informed local care teams. There is not always a treatment for a rare condition, of course, so many willingly participate in clinical trials, desperate for anything that helps.

They are willing to risk so much for access to care. The reality is that chronic disease patients need more access to better treatments, and any action to address pricing must consider its potential impact on current availability and future development. This Colorado patient's experience and concerns are similar to that of thousands of other families (read here).

To improve affordability for rare disease patients, it's especially important to protect, support and expand discounts at the patient level. By prioritizing reforms that offer immediate and tangible benefits to patients, we can advance the cause of more accessible and effective care.

Sincerely,

Mary Kay Clunies-Ross
Executive Director
marykay@chronicdiseasecoalition.org
(206) 817-4845

From: MacKay Jimeson, Executive Director, Patients Rising
Sent: Tuesday, July 22, 2025
To: PDAB
Subject: Protecting the Rare Disease Patients in Oregon's PDAB Law

Chairwoman Bailey, Senator Patterson, and Representative Nosse,

On behalf of Patients Rising, I urge the Board to preserve the rare disease exemption in Oregon's Prescription Drug Affordability Board (PDAB) statute.

Removing this exemption would endanger access to life-saving therapies for individuals facing some of the most devastating medical challenges. For rare disease patients—many of them children—these treatments are often the only options available, and they arrive only after years of advocacy and scientific perseverance.

At the core of this issue is how we define "value." Health Technology Assessments (HTAs)—like those often used by affordability boards—are intended to guide payers and policymakers by projecting the costs and benefits of new therapies. Unfortunately, these assessments often rely on generic, population-level quality-of-life models that do not reflect the lived experience of patients with rare conditions.

These models fail in three fundamental ways:

1. **They ignore indirect and non-medical costs** such as caregiving burdens, home adaptations, and the economic impact on families who must forgo work or education to care for a loved one.
2. **They overlook societal benefits**, including productivity regained, educational opportunity restored, and caregiver reentry into the workforce—real-world impacts that matter deeply to families and communities.
3. **They are not designed to evaluate rare diseases**, where traditional randomized clinical trials may be impractical or unethical. Small, heterogeneous patient populations, unpredictable disease progression, and rapid degeneration often make placebo-controlled trials impossible.

More than 90 percent of rare diseases currently have no FDA-approved treatment. For the small number that do, the evidence base may come from alternative clinical designs, such as natural history studies, real-world data, or single-arm trials—because it's not feasible or humane to randomize patients to certain decline or death. Yet HTA frameworks often penalize such methods, undervaluing the therapy's true benefit and placing it at risk of being deemed "unaffordable."

The rare disease exemption in Oregon's PDAB law is not a loophole. It is a necessary safeguard that recognizes the limitations of traditional cost-effectiveness frameworks when applied to rare conditions. Eliminating this exemption would allow incomplete, generalized, and sometimes discriminatory valuation models to dictate access decisions for patients with no therapeutic alternatives.

This is not just a technical issue—it is a moral one. Oregon must not become the first state to institutionalize health inequity for people with rare conditions by stripping away the one protection that ensures their needs are judged on their own terms.

On behalf of our community, we respectfully ask that the Board:

1. **Retain the rare disease exemption** in full;
2. **Formally recognize the limitations of current HTA methodologies**, especially for small-population, high-need conditions;
3. **Engage patients and caregivers directly** in any future evaluations or working groups; and
4. **Adopt patient-centered best practices** that account for indirect costs, real-world outcomes, and non-traditional evidence when reviewing access and affordability.

Thank you for your attention and your commitment to making healthcare more equitable and responsive. Patients Rising stands ready to support the Board in its work while defending the rights of those living with rare and life-threatening conditions.

Sincerely,

MacKay Jimeson