



Alone we are rare. Together we are strong.®

April 26, 2024

The Honorable Lindsey Daugherty, Chair
The Honorable Mary Young, Vice-Chair
House Committee on Health & Human Services
Colorado General Assembly
200 E. Colfax Avenue
Denver, CO 80203

Dear Chair Daugherty, Vice-Chair Young, and Members of the Committee on Health & Human Services,

On behalf of the more than 30 million Americans living with one of the over 10,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks you for your commitment to ensuring that all Coloradans have access to affordable medications, and that the perspectives of rare disease patients and the broader rare disease community are included in the prescription drug cost evaluation process.

NORD is a unique federation of non-profits and health organizations dedicated to improving the health and well-being of people living with rare diseases. NORD was founded over 40 years ago, after the passage of the Orphan Drug Act, to formalize the coalition of patient advocacy groups that were instrumental in passing that landmark law. Our mission has always been, and continues to be, to improve the health and well-being of people with rare diseases by driving advances in care, research, and policy.

NORD is supportive of the intent of SB24-203, sponsored by Sens. Ginal and Kirkmeyer and Reps. Hartsook and Ortiz, to increase collaboration between the Rare Disease Advisory Council (RDAC) and the Prescription Drug Affordability Board (PDAB) in the prescription drug cost evaluation process for therapies that treat a rare disease. In a July 28, 2023, letter to the Colorado Prescription Drug Affordability Advisory Council, NORD outlined several recommendations to ensure perspectives from the rare disease community were captured on pertinent drugs, including formalizing involvement with the RDAC.¹ We believe that the RDAC is an invaluable resource to the prescription drug cost assessment process. It is our understanding that Colorado's RDAC and PDAB have already had a number of discussions during the first round of the PDAB's cost assessment process, and we believe formalizing the collaboration in statute between the two entities will be beneficial to ensuring the unique and complex considerations for orphan therapies are taken into account in the future.

To support the development of legislation that best meets the needs of the rare disease community, NORD wishes to highlight the differences between orphan designations and FDA approved orphan indications and offer two minor modifications to the bill to provide additional clarity for effective PBAB and RDAC collaboration moving forward.

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Distinguishing between orphan designations and FDA approved orphan indications

Orphan drug designations (i.e., designating a drug to treat a rare disease, defined as any disease affecting fewer than 200,000 individuals in the United States) are distinct from FDA approved orphan indications.

An orphan designation is usually granted early in the drug development process, when a drug sponsor submits preliminary (i.e., pre-clinical or early clinical) information to the FDA suggesting that the drug could potentially treat a targeted rare disease. If the FDA agrees, the drug is granted an orphan designation. However, this orphan designation does not allow the manufacturer to market the drug. Rather, an orphan designation qualifies the manufacturer to receive specific research and development incentives, including tax credits for qualified clinical trials, exclusion from FDA user fees, and potentially seven years of market exclusivity if the product is approved.

In contrast, orphan indications are FDA approved uses for orphan drugs, granted at the end of extensive clinical drug development cycle, and after the FDA has deemed the product to be safe and effective for a specific disease and patient population. FDA approval allows the product to be marketed for those specific indications. As designations are generally a preliminary measure and many early research programs do not ultimately achieve clinical success, many orphan designations do not result in a corresponding FDA approved orphan indication. In fact, since the passage of the Orphan Drug Act (ODA) in 1983, more than 6,850 designations have been granted, but only 1,226 orphan indications have been approved to date.

Furthermore, recognizing that a product can have multiple approved indications, of which some may be tied to orphan designations and others may not, we recommend clarifying that the RDAC input should be limited to any FDA approved indications tied to orphan designations and is not necessary for non-orphan indications of a product.

Recommendation 1: Ensure all products with an approved orphan indication, or orphan indications, are appropriately considered by the RDAC for their orphan uses

To further utilize the RDAC's unique perspective, NORD encourages you to expand the drugs where RDAC input is required to include any product selected for review that has an orphan indication (or indications). While some products may be FDA approved to only treat rare diseases (orphan-only products), many drugs have been approved to treat both non-rare diseases and rare diseases (partial-orphan products). Indeed, a product which has been deemed unaffordable by the Colorado PDAB and is set to undergo the upper payment limit rulemaking process this year is an example of a partial-orphan product with both an orphan indication tied to an orphan designation, as well as FDA approved indications for the treatment of non-rare diseases.

Rare disease patients and health care providers frequently have a unique perspective on the value a therapy provides. Furthermore, while there are often therapeutic alternatives for non-rare diseases, rare disease patients are usually much more limited in their treatment options for their specific rare

condition. Involving the RDAC in deliberations about a partial-orphan product's orphan indication(s) will ensure that the voice of the rare disease community is represented in an appropriate and nuanced way.

Recommendation 2: Clarify that in order for a product to be referred to the RDAC for input, the product must have received at least one FDA approved orphan indication for at least one of its orphan designated uses

NORD strongly believes the RDAC's input is valuable on both partial-orphan products and orphan-only products, but does not believe it is necessary for an RDAC to provide input on products that do not have an FDA approved orphan indication. For example, a product selected by the PDAB for an affordability review in the initial round is currently approved for four non-orphan indications. The product has also been granted six orphan designations, none of which are tied to currently approved indications. Of the six orphan designations granted, four have been withdrawn or revoked, while two remain active. Therefore, we do not recommend the RDAC weigh in on this product since it has no FDA approved orphan indications.

Consistent with the two recommendations outlined above, NORD proposes the following line edits to SB24-203 for your consideration: In Colorado Revised Statutes, 10-16-1406, section 1(e):

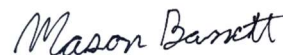
1. Lines 14-15, strike "and no other indications" and replace with "one or more FDA approved indication or indications tied to the orphan designations"
2. Line 17, after "25-1-1503" add "on these indications tied to orphan designations."

Thank you again for your support of the rare disease community. If you have any questions, please do not hesitate to contact Lindsey Viscarra at lviscarra@rarediseases.org or Mason Barrett at mbarrett@rarediseases.org.

Sincerely,



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