



July 28, 2023

Prescription Drug Affordability Advisory Council
Colorado Division of Insurance
1560 Broadway, Suite 850
Denver, CO 80202

Dear Members of the Colorado Prescription Drug Affordability Advisory Council,

On behalf of the more than 25 million Americans living with one of the over 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) would like to thank you for your commitment to ensuring that all Coloradans have access to affordable medications. On March 23, 2023, NORD submitted comments regarding the prioritization of selection class criteria for affordability reviews. We are writing today to provide more detailed feedback as the prioritization and selection of therapies for the affordability review process continues.

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 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.

Recommendation 1: To ensure the effectiveness of the affordability review, the prioritization and selection of therapies should consider the key factors besides costs that ultimately determine the outcomes of the review

Advancements in research and innovation have created revolutionary and lifesaving treatments for some in the rare disease community, but the high cost of some therapies can create significant and unsustainable financial barriers for patients, their families, and the health care system. However, to be successful, any affordability review and possible upper payment limit determination cannot only consider the savings that accrue in the near term on prescription drug costs, but also must be evaluated based on the impacts on patients, communities, and the broader health care system. While NORD supports the factors outlined in Senate Bill 21-175 for consideration in the affordability review itself, it is crucial that some of the same key factors are first considered in prioritizing and selecting drugs from the list of eligible drugs. This will ensure that the affordability reviews can focus on the highest priority drugs to have the maximum impact.

As stated in our March 23rd letter¹, NORD strongly encourages orphan drug status, which is one of the factors outlined in Senate Bill 21-175 to be considered when the Prescription Drug Affordability Board (PDAB) is conducting affordability review, also be a factor when then PDAAC is prioritizing eligible products in the first place. Additionally, considerations of out-of-pocket costs and other patient cost

¹ https://rarediseases.org/wp-content/uploads/2023/03/NORD-Comments_Colorado-Prescription-Affordability-Board.pdf

sharing, available therapeutic alternatives, and expected impacts on safety net providers are vital considerations to ensure the appropriate prioritization among prescription drugs eligible for review. Considering these key factors not just in the affordability review but already in the prioritization of drugs for review will ensure any recommendations to address prescription drug costs reflect a careful balance between greater affordability and patient value, ensuring greater patient access while maintaining valuable incentives for continued rare disease drug development.

Recommendation 2: Clarify and further define how exactly orphan status will be considered in the prioritization and affordability review process

As outlined above, NORD appreciates that orphan drug status is one of the key factors listed in Senate Bill 21-175 for affordability reviews and we urge the PDAAC to also consider it as one of the key factors in prioritizing drugs for review. However, the statute is silent as to exactly how “orphan drug status” should be considered, and therefore, NORD respectfully urges the PDAAC and PDAB to further clarify how exactly orphan drug status will be considered. This will ensure greater transparency and consistency in the prioritization and affordability review. Specifically, we urge you to carefully consider the following specific aspects of the rare disease patient experience and therapy development and ensure they are appropriately weighted when prioritizing drugs for and conducting an affordability review.

Research and development into therapies to treat rare diseases is particularly challenging. By definition, rare diseases impact small patient populations² that are often geographically dispersed. Limited natural history data and knowledge of disease progression combined with heterogeneous disease presentation mean that clinical trials to determine the safety and effectiveness of potential treatments can be very difficult and expensive to conduct. Congress recognized this challenge four decades ago when it passed the Orphan Drug Act, which established a set of incentives to encourage rare disease drug development.³ According to the FDA’s orphan drug product database, since the passage of the Orphan Drug Act, manufacturers have obtained 6530 orphan designations,⁴ a process that is usually done early in the drug development process and unlocks access to ODA incentives such as funding and tax credits for clinical research to help de-risk this phase of drug development. However, an orphan drug designation does not allow the company to market the drug; it is only the first in many steps towards product approval and to date, only 1150 orphan indications⁵ have been determined to be safe and effective and therefore, FDA approved. Even with the ODA’s incentives in place, more than 95% of rare diseases do not have an FDA-approved therapy⁶ and often there are no therapeutic alternatives available when there is an existing rare disease therapy.

The tremendous unmet needs that exist in the rare disease community have significant economic impacts on patients, their families, and the broader health care system. As previously mentioned, it is estimated that more than 25 million Americans are living with a rare disease, but only 5% of rare diseases have an FDA approved therapy, leaving millions of Americans with significant unmet medical needs. According to a study published in *Orphanet* in April 2022, a review of just 379 of the more than 7,000 known rare

² <https://www.fda.gov/industry/designating-orphan-product-drugs-and-biological-products/orphan-drug-act-relevant-excerpts>

³ Ibid.

⁴ <https://www.accessdata.fda.gov/scripts/opdlisting/oopd/listResult.cfm>

⁵ Ibid.

⁶ <https://rdcu.be/dh0XV>

diseases showed a total economic burden of \$997 billion in 2019, with \$449 billion in direct medical costs and \$439 billion in non-medical costs.⁷ Patients living with a rare condition that does not have a safe and effective therapy need access to that therapy in order to achieve their optimal health, which can benefit the individual and their family financially through an improved ability to go to school or work and reduced caregiver burdens. Without access to these critical therapies, patients face significant disease progression, increased out of pocket health care costs, and even die without access to treatment. Timely patient access to safe and effective therapies is critical to the rare disease community, but it also benefits broader society.

Complexities associated with rare disease therapies and the available data to determine their cost-effectiveness create unique challenges for determining fair prices for these products. It is a recognized challenge that for many rare diseases, data relevant to clinical benefit, therapeutic alternatives, or unmet medical need often does not currently exist in peer-reviewed journals or consensus treatment guidelines. Additionally, the lack of disease-specific International Classification of Disease (ICD-10) codes for most rare diseases makes strategies relying on existing real-world data (RWD) from sources such as electronic health records (EHRs) or medical claims data largely infeasible for many rare diseases.

Continued investment in rare disease research and innovation critical to the rare disease community. Serial innovation and the investigation and development of new and multiple rare disease indications of use is an increasingly important dimension of orphan drug development. Drug sponsors are making decisions today that will impact their investments and drug development pipeline for decades to come. Uncertainty about how an orphan drug will become eligible for affordability reviews and upper payment limit setting creates business risks that work as strong disincentives to develop drugs for the limited populations impacted by rare diseases. These limitations on innovation directly contribute to the ongoing unmet need in drug therapies for rare diseases.

Recommendation 3: The Colorado Rare Disease Advisory Council can be a resource for the prioritization and affordability review of orphan products

Last year, Colorado established a Rare Disease Advisory Council (RDAC).⁸ Colorado's RDAC aims to inform state agencies, the public, and the legislature about rare diseases and make recommendations concerning the needs of Coloradans living with rare diseases. The Colorado RDAC is comprised of various stakeholders with experience in the rare disease community, including a wide range of lived experience from rare disease patients and caregivers and medical professionals, and members of the biotechnology and pharmaceutical industries. Also represented are rare disease patient organizations and those with an understanding of the rare disease research landscape. We urge the PDAAC and PDAB to utilize the RDAC as a resource should a product be selected for an affordability review that has any orphan indications.

In conclusion, it is critical that recommendations to address prescription drug costs reflect a careful balance between greater affordability and maintaining appropriate incentives for continued investment, which is especially critical in rare disease drug development given the tremendous unmet needs that still exist. As the PDAAC works to determine which medications to prioritize and recommend for an affordability review and the PDAB possibly establishes an upper payment limit for, it is of utmost

⁷ <https://pubmed.ncbi.nlm.nih.gov/35414039/>

⁸ https://leg.colorado.gov/sites/default/files/2022a_186_signed.pdf

importance that patient access be maintained to for those in the rare disease community fortunate to have an FDA approved therapy. NORD also remains a resource as the PDAAC works to determine the list of products for affordability review. For questions regarding NORD or our comments, please contact Heidi Ross, Vice President at HRoss@rarediseases.org or Lindsey Viscarra at lviscarra@rarediseases.org.

Sincerely,



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CC: Members of the Colorado Prescription Drug Affordability Board

