March 23, 2023

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

Dear Members of the Colorado Prescription Drug Affordability Board,

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) thanks you for the opportunity to provide input to the Prescription Drug Affordability Board (Board) into the prioritization of selection class criteria for affordability reviews pursuant to Senate Bill 21-175.

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 by driving advances in policy, research, and care. 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 and just last year, strongly supported legislation to establish Colorado's Rare Disease Advisory Council (SB22-186), which was signed into law by Governor Polis on June 8, 2022.

While advancements in research and innovation have brought new and lifesaving treatments to many patients, the high costs of some prescription drugs place a significant and unsustainable strain on patients, their families, and the health care system as a whole. For Americans living with a rare disease, high drug prices can hinder their ability to access needed therapies. However, more than 90% of rare diseases do not have an FDA-approved therapy and rare disease patients also face significant health and financial obstacles when there is no treatment option for their condition. This makes continued research and innovation into new therapies especially important to the rare disease community. Therefore, it is critical that recommendations to address prescription drug costs reflect a careful balance between greater affordability and maintaining appropriate incentives for continued investment in rare disease specific drug development.

As outlined in 3 CCR 702-9, the Board is asked to consider orphan drug status, including the "extent to which the drug addresses an unmet need or treats a rare or serious disease for which limited therapeutic alternatives are available" during an affordability review. This input is critical, but NORD strongly encourages orphan drug status, and the extent to which an orphan product addresses an unmet need or disease with limited available therapeutic alternatives, be considered not only during the affordability review process, but as a heavily weighted factor in the criteria against product selection for an affordability review.

NORD encourages the Board to recognize that the small patient populations and medical complexity associated with rare diseases can create unique challenges to rare disease drug development and these same complicating factors may also make it more difficult to conduct an affordability review or

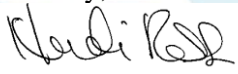
determine an upper payment limit for rare disease products compared to other therapies. Often there are zero, or few, "equivalents" to rare disease therapies. Furthermore, there are often limitations in the available real-world evidence sources for rare versus more common diseases. Ultimately, the impact of a therapy for a rare disease patient for whom no therapy previously existed is not the same as a therapy for a patient with a common disease that has several other currently marketed therapies. Therefore, it is critical that the selection criteria for an affordability review consider the unique value of therapies for patients with rare diseases, as well as the challenges of bringing these products to market.

It is important to note that the FDA's expedited approval programs are of critical importance to the rare disease community. Congress enacted the laws directing FDA to establish these programs out of a recognition of the potential scientific advancements and positive outcomes for patients that come from additional efforts by the FDA to expedite the review and provide greater regulatory guidance for sponsors of these products. These processes still require the FDA to review and, if appropriate, approve the drug by applying the same standard of safety and effectiveness mandated under the Federal Food, Drug and Cosmetic Act as all other therapies that seek FDA approval. The threshold for approval for these drugs is not somehow lesser than products approved through non-expedited pathways and should not be treated as such in terms of selection criteria for affordability reviews.

Finally, as previously referenced, last year Colorado established a Rare Disease Advisory Council (RDAC). 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 RDAC includes a diverse group of rare disease stakeholders including patients, health care providers, and researchers, and NORD encourages the Board to make use of the expertise available to them on the Colorado RDAC whenever conducting affordability reviews or setting upper payment limits for products that have one or more orphan indications.

The last decade has seen a tremendous growth in new, innovative therapies for rare disease patients and many rare disease patients who were once without any possibility of receiving targeted treatments for their condition may soon have new therapeutic options. NORD encourages the Board to establish its selection criteria and affordability reviews in a way that supports patient access to critical therapies and continued investment in development and innovation to address the tremendous unmet therapeutic needs in the rare disease community. For questions regarding NORD or our comments, please contact me at HRoss@rarediseases.org.

Sincerely,



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