Rare Disease Company Coalition

BY ELECTRONIC DELIVERY

August 24, 2022

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

RE: Department of Regulatory Agencies Prescription Drug Affordability Board Draft Proposed Rule – Part 3, sections 10-16-1403(5) and 10-16-1406, C.R.S.

Dear Colorado Prescription Drug Affordability Board Members:

The Rare Disease Company Coalition (RDCC) thanks you for the opportunity to provide comments on the Department of Regulatory Agencies Prescription Drug Affordability Board (PDAB or "the Board") draft proposed rule on Affordability Review of Prescription Drugs. The RDCC shares Colorado's goal of ensuring patients have access to innovative and life-saving therapies; however, as written, the rule could seriously impede research and development for new and innovative therapies, which will in the long run harm more patients than it helps.

The RDCC is a coalition of 21 life science companies that are committed to changing the paradigm in rare disease treatment by discovering, developing, and delivering life-changing therapies to rare disease patients around the globe. Collectively, RDCC members invested over $12.4 billion in R&D in 2021; have brought 31 treatments to market to date, the majority of which are the first FDA approved treatments available for a given disease; and are presently working to advance more than 200 rare disease development programs, many of which would be the first ever FDA-approved therapy for patients with a given rare disorder.[1] Our goal is to inform policymakers of the unique challenges facing the biopharmaceutical companies that are working to take these rare disease therapies from discovery to approval and finally, and most importantly, to patients.

The RDCC is concerned this rule will create potentially insurmountable economic barriers for innovator pharmaceutical companies to pursue the difficult and long road of scientific discovery of new therapies to treat the more than 7,000 identified rare diseases. We know that rare disease patients have a significant unmet medical need and have limited to no treatment options, which is why it's imperative that the Board revise the proposed rule to address the provisions that will have detrimental consequences for rare disease patients.

Summary

We strongly urge the PDAB to consider the unique circumstances of rare disease patients and therapies as it crafts its policy proposals. The RDCC cautions against punitive measures that would have an outsized impact on rare diseases, chilling future investment and development of rare disease treatments

[1] Rare Disease Company Coalition. https://www.rarecoalition.com/.

that would further disadvantage rare disease patients. As such, the Coalition has significant concerns with the Board's proposed rule as it could potentially:

- Restrict patient access to innovative treatments for rare diseases and stifle future development; and
- Undervalue rare disease treatments by applying misaligned cost effectiveness frameworks that are not patient-centered, not calibrated for smaller patient-populations, nor meaningfully account for the advances in science and evidence-based research associated with rare disease drug development.

Our specific comments and concerns, as further detailed in the sections below, are:

- The proposed rule fails to adequately account for the unique challenges of developing treatments for rare diseases;
- Assessing value of treatments for rare diseases requires different approaches but the proposed rule applies a one-size-fits-all framework to all drugs being evaluated; and
- Treating rare diseases requires special expertise, and engaging scientific or medical professionals with the necessary experience, as well as the Colorado Rare Disease Advisory Council, during affordability review is critical to mitigating potential negative impacts on rare disease patients.

Developing treatments for rare diseases presents unique challenges

In the United States, a rare disease is defined as a condition that affects fewer than 200,000 people. There are over 7,000 identified rare diseases that impact an estimated 25 to 30 million Americans.[2] These diseases are devastating and often life-threatening: 80 percent of rare diseases are genetic in origin, 50 percent impact children,[3] with many rare diseases resulting in premature deaths of infants and young children.[4]

When taken as a whole, Colorado's PDAB law has the potential to disproportionately impact rare disease patients and rare disease drug development. In recognition of this, some states that have enacted similar laws – like Oregon and Washington – have entirely exempted rare disease drugs from the affordability review and upper payment limits process to protect innovation and access for patients living with a rare disease, who have limited to no treatment alternatives. Thus, it's critical that the proposed rule fully take into account the unique challenges in the development of rare disease therapies, including small patient populations, progressively debilitating and variable disease courses, and the fact that many of the individuals affected are children.

Congress has long recognized these unique challenges and equipped the US Food and Drug Administration (FDA) with the tools needed to modernize and adapt rare disease drug development and regulatory pathways. Since implementation of the Orphan Drug Act (ODA) in 1983, the FDA has approved over 600 orphan products to treat rare diseases.[5] Prior to enactment of the ODA, only 38

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[2] National Center for Advancing Translational Science and Genetic and Rare Diseases Information Center. *What is a rare disease?* Updated January 26, 2021. Accessed April 2022 at https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases.

[3] Batshaw ML, Groft SC, Krischer JP. Research into rare diseases of childhood. *JAMA*. 2014; 311(17): 1729-30.

[4] Institute of Medicine (US) Committee on Accelerating Rare Diseases Research and Orphan Product Development; Field MJ, Boat TF, editors. *Rare diseases and orphan products: Accelerating research and development*. Washington (DC): National Academies Press (US); 2010. Available from: https://www.ncbi.nlm.nih.gov/books/NBK56189/ doi: 10.17226/12953

[5] U.S. Food and Drug Administration (FDA). Developing Products for Rare Diseases & Conditions: About OOPD. Content Current as of May 12, 2022. Accessed at https://www.fda.gov/industry/developing-products-rare-diseases-conditions

therapies were available to treat rare diseases.[6] It took decades after the passage of the ODA to see meaningful results for rare disease patients and attract the investment needed to develop these breakthrough therapies. Despite this progress, more than 90 percent of rare diseases are still without an FDA-approved treatment option and our commitment to advancing rare disease therapies must remain steadfast.

The underlying Colorado law (section 10-16-1406(4), C.R.S.) requires the consideration of orphan drug status during affordability review. We appreciate that the proposed rule adds further considerations by the Board of the patient population served by the drug, the extent to which the drug fills an unmet need, and if the drug treats a rare or serious disease (and where there are limited therapeutic alternatives available). However, given a rare disease drug has minimal utilization due to inherently small patient populations, and will likely not meaningfully address budget concerns, subjecting products that treat rare diseases to affordability reviews runs counter to the overall intent of the PDAB and therefore we urge the Board to consider incorporating into the rule a de-prioritization process for the review of rare disease therapies. The Board should strike the right balance when selecting drugs to review and realize that conducting reviews of rare disease therapies could 1) create headwinds to the progress that has been made in rare disease drug development and 2) will jeopardize access for vulnerable Coloradans suffering with a rare disease who are waiting for that next innovation to address their rare disorder. The challenges for many patients with rare disease can be made exponentially more difficult by socioeconomic, ethnic, and/or race factors as well. Therefore, consideration of the unique nature of rare disease and therapies must be a critical piece to Colorado's affordability review process.

Treatments for rare diseases require different value assessment approaches

The proposed rule seeks to consider the estimated cost-effectiveness of prescription drugs and determine the "relative financial effects of the price on broader health, medical, and/or social services costs." The Board intends to determine these costs by reviewing "relative financial effects literature" and the use of information that leverages "a quality-adjusted life year analysis to evaluate relative financial effects" of a prescription drug. Existing value-assessment frameworks, including health technology assessments (HTAs) or cost effectiveness analyses (CEAs), are not designed to adequately address the unique considerations of rare diseases and rare disease therapies. These frameworks typically assess value to the payer, not patient, and only consider two core inputs: quality-adjusted life-years (QALYs) gained and net cost. Both metrics have significant shortcomings when assessing the value of rare disease therapies.

- The QALY is a single measure that combines how much a treatment extends life and improves quality of life. This flawed measurement assigns a lower value to patients with disabilities or debilitating conditions than those without;[7] a point that the Board recognizes in the proposed rule. Valuing a person's life at a lesser value because they were born with a genetic disease or need a wheelchair is discriminatory and unethical. The National Council on Disability has urged Congress to ban the use of QALYs because this metric devalues those living with disabilities and

[6] National Organization for Rare Disorders (NORD). Orphan Drugs in the United States: An Examination of Patents and Orphan Drug Exclusivity. Published March 25, 2021. Accessed May 2022 at https://rarediseases.org/wp-content/uploads/2021/03/NORD-Avalere-Report-2021_FNL-1.pdf

[7] FTI Consulting. Challenges in Preserving Access to Orphan Drugs Under an HTA Framework. Published December 2, 2021. Accessed May 2022 at https://www.fticonsulting.com/insights/reports/challenges-preserving-access-orphan-drugs-hta-framework

negatively impacts access to treatment.[8] The inherent inequity of a QALY analysis is expressly why the legislature included in the law a strict prohibition on the use of QALY by the Board in determining an applicable upper payment limit. The affordability review is the gateway to which potential upper payment limits will be established so by allowing analyses that use QALY to be part of an affordability review, this flawed metric will inherently influence the determination of any subsequent upper payment limit, running contrary to the intent of the law. We urge the Board to remove the consideration of QALY from the proposed rule and any part of the Board's work.

- Net costs – direct medical costs to the payer – measure the opportunity for a new therapy to save money to the payer and are calculated by utilizing the cost of the current treatment paradigm. But for many rare diseases, costs incurred by the payer are often not as high as the direct and indirect costs borne by patients and their families, given limited availability of treatments to begin with. There are many other costs directly attributed to treating rare disease borne by the patient and family, not the payer, including familial and societal costs, which are not quantitatively accounted for in traditional value frameworks.[9]

If the Board were to decide to conduct an affordability review of a rare disease therapy, it must utilize a value-assessment framework that is patient-centered, incorporating diverse inputs that are missing from traditional CEAs or HTA frameworks. For an equitable review of a rare disease therapy, it's essential that the Board incorporate key considerations for valuing rare disease treatments including disease progression, symptom control, a treatment's impact on productivity, ability of a patient to participate in activities of daily living, impact on caregiver burden and the ability to remain in the workforce, improvement over alternative treatments, impact on public health, and health equity.[10]

Engaging scientific or medical professionals with experience treating rare diseases is critical

The RDCC appreciates that the Board is including input from patients, caregivers, and individuals with scientific or medical training in their affordability review process. We also appreciate the Board considering information voluntarily provided by a manufacturer prior to any decisions. Input from stakeholders is critical to ensure all potential factors are considered before determining eligibility for affordability review, especially because this first step in establishing an upper payment limit could negatively impact patient access to life altering medications if implemented. More specifically, we urge the Board to include scientific and medical professionals with experience treating the disease(s) that the therapy is indicated for – or at the very minimum, a specialist in the therapeutic area (neurology, metabolics, etc.) – when making any affordability review decisions. Additionally, racial and ethnic minorities with a rare disease face additional challenges in access to care and are underrepresented in research and clinical trials.[11] These disparities make timely diagnosis and adequate treatment exponentially harder, resulting in poorer health outcomes for these patients. Because of the complex nature and heterogeneity of rare diseases, all too often, policy decisions that impact the rare disease

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[8]National Council on Disability. Quality-Adjusted Life Years and the Devaluation of Life with Disability. Published November 6, 2019. Accessed May 2022 at https://ncd.gov/sites/default/files/NCD_Quality_Adjusted_Life_Report_508.pdf

[9] https://everylifefoundation.org/wp-content/uploads/2021/02/The_National_Economic_Burden_of_Rare_Disease_Study_Summary_Report_February_2021.pdf

[10] ISPOR. Novel Elements of the Value Flower: Fake or Truly Novel? Published April 2021. Accessed May 2022 at https://www.ispor.org/publications/journals/value-outcomes-spotlight/vos-archives/issue/view/navigating-the-changing-heor-publishing-landscape/novel-elements-of-the-value-flower-fake-or-truly-novel

[11] The Rare Disease Diversity Coalition. *About the Coalition*. Accessed April 2022 at https://www.rarediseasediversity.org/about-the-coalition.

communities are made by professionals that do not have the background or experience necessary to fully understand the policy's impact to the rare community.

Finally, we recommend that the proposed rule include a requirement for the Board to consult with Colorado's Rare Disease Advisory Council (RDAC) if it undertakes an affordability review of a rare disease therapy. The RDAC was created from the enactment of SB22-186 and will consist of residents with expertise and experience in treating or being treated for a rare disease. Consultation with the RDAC will ensure another formal avenue for the rare disease community to offer input and help the Board incorporate broader criteria into a value assessment that will be the basis for an affordability determination.

Conclusion

We appreciate the opportunity to provide comments on Colorado's PDAB draft proposed rule. The RDCC is greatly concerned about the negative impact the proposed affordability review rules will have on the rare disease community. We ask the Board to weigh the potential impacts the proposed rule could have on the rare disease community in Colorado, as innovating and maintaining access to prescription drugs can be a matter of life, quality of life, and hope for the millions of individuals with rare diseases.

Should you have any questions, please feel free to contact me at amanda@rarecoalition.com.

Sincerely,

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Executive Director
Rare Disease Company Coalition