RAAP
RARE ACCESS ACTION PROJECT

July 26, 2023

Colorado Prescription Drug Affordability Board &
Prescription Drug Affordability Advisory Council (PDAAC)
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Members of the Colorado Prescription Drug Affordability Board &
Members of the Prescription Drug Affordability Advisory Council (PDAAC):

Thank you for the opportunity to comment. As part of this process, we request that all the comments be made public.

On behalf of the Rare Access Action Project (RAAP), I am writing to express strong concerns with the inclusion of rare therapies in the upcoming Prescription Drug Advisory Board (PDAB) process, which are part of the recommendations from the Prescription Drug Affordability Advisory Council (PDAAC). We understand that the PDAAC will be preparing recommendations for the PDAB and requesting community input on July 31st.

RAAP is a coalition of life sciences innovators and patient stakeholders that explore creative policy solutions to address structural issues in access and coverage. Our priority is to help ensure rare disease patients have access to the care and treatments they need.

The Orphan Drug Designation Program provides orphan status to drugs and biologics which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the US, or that affect more than 200,000[1] persons but are not expected to recover the costs of

[1] Department of Health and Human Services, Food and Drug Administration (FDA). Office of Orphan Drug Development. https://www.fda.gov/about-fda/office-clinical-policy-and-programs/office-orphan-products-development. Accessed March 13, 2021.

developing and marketing a treatment drug.[2] Rare diseases include more familiar conditions, such as cystic fibrosis, Lou Gehrig's disease, and Tourette's syndrome, as

well as less familiar conditions, such as aromatic L-amino acid decarboxylase (AADC) deficiency, Duncan's Syndrome, Madelung's disease, and acromegaly/gigantism. These conditions are complex and often not well understood, which causes great challenges to the diagnosis, treatment, and research efforts.

Rare disease treatments range from curing the disease, modifying how the disease functions, or treating the symptoms. Truly curative treatments are uncommon. Disease-modifying therapies target the underlying pathology of a disease to prevent it from worsening. Symptomatic treatments seek to temper symptoms or to maintain physical, emotional, and mental functioning.

Approximately 5% of rare diseases have a treatment approved by the Food and Drug Administration (FDA) and for one-third of individuals with a rare disease, it can take between one and five years to receive a proper diagnosis. Patients with rare diseases often seek treatment in clinics where the condition has never been seen before and have symptoms that are absent, masked, misunderstood, or confused, which often leads to delayed diagnosis further complicating the patient's and family's arduous journey. Half of all patients diagnosed with a rare disease are children, and as many as 3 in 10 children with a rare disease will not live to see their 5th birthday[3]. RAAP understands that there are several barriers patients face with gaining access to these orphan designated drugs across our healthcare system and are often faced with a patient journey filled with misdiagnosis and lack of treatment options.

By nature, these smaller patient population therapies are going to be higher cost products. In 2021, the National Organization for Rare Disease (NORD) found that spending for rare disease therapies made up approximately 11 percent of invoicing for all medications in the United States. Since 2010, the share of orphan drug spending has increased by five percentage points, likely due to the increasing number of approved orphan products. During this same time, specialty drug spending has also risen by 22 percentage points and now accounts for 47% of all medical invoice spending. However, specialty drugs and orphan drugs are not the same,

[2] 87 Fed. Reg. at 28,195.

[3] Slade, A., Isa, F., Kyte, D., Pankhurst, T., Kerecuk, L., Ferguson, J., Lipkin, G., & Calvert, M. (2018). Patient reported outcome measures in rare diseases: a narrative review. *Orphanet journal of rare diseases*, *13*(1), 61. https://doi.org/10.1186/s13023-018-0810-x

and IQVIA found 77% of specialty drug spending is for the treatment of common conditions and not rare diseases.[4]

And, most notably, approximately 95 percent of rare diseases have no FDA approved therapies, leaving patients in Colorado and across the United States with a significant unmet medical need.

Recently, the Everylife Foundation released a study that explored the burden of rare diseases in the United States, highlighting that most rare patients have no approved therapies, and the costs of rare disease are significant. But it is also worth considering the impact on Colorado patients who could have their treatments disrupted due to untested and unproven policies. According to the report:

"Besides the direct medical costs associated with RD, there are significant costs to society and individuals, including indirect costs associated with productivity losses, non-medical costs such as spending on home or motor vehicle modifications, and certain healthcare costs not covered by insurance. Many individuals with RDs have high medical needs requiring that they miss work, retire early, and utilize the assistance of a caregiver for activities for daily living ([5])([6]). Caregivers also experience work productivity losses to fulfill their caregiving responsibilities[7]. As such, the economic burden of RD is likely to be significant, for patients, unpaid family caregivers, and society."

The PDACC's determination to include rare therapies in this experiment could have unintended consequences for rare families and communities. Upper Payment Limits (UPL) could begin to create barriers to access where currently such barriers do not exist for rare patients. Increased administrative burdens can make navigation of coverage more time consuming. Further, if the cost of the product exceeds the mandated UPL, a rare patient currently being successfully treated could face uncertain continuity of care where in many cases there are no other treatment options. The federal government,

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[4] NORD publications, https://rarediseases.org/new-report-finds-medical-treatments-for-rare-diseases-account-for-only-11-of-us-drug-spending-nearly-80-of-orphan-products-treat-rare-diseases-exclusively/?pt=post&taxonomy[category]=research, March 2021.

[5] Forshaw R. What is the cost of living with rare disease? https://rarem ark.com/articles/what-does-it-cost-to-live-with-a-rare-disease--572. Accessed 29 Jan 2021.

[6] Therapies, Shire Human Genetic. Rare disease impact report: insights from patients and the medical community. Shire Human Genetic Thera- pies, Tech. Rep; 2013.

[7] Mighiu C, O'Hara S, Ferri Grazzi E, Murray KF, Schattenberg JM, Ventura E, Karakaidos M, Taylor A, Brrang H, Dhawan A, Willemse J, Finnegan A. Impact of progressive familial intrahepatic cholestasis on caregiv- ers: caregiver-reported outcomes from the multinational PICTURE study. Orphanet J Rare Dis. 2022;17(1):32. https://doi.org/10.1186/ s13023-022-02177-0.

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through enactment of the Inflation Reduction (IRA), recognized the difficulty in developing medicines used to treat rare diseases by exempting certain orphan drugs from negotiation. The Colorado PDAB statute includes no such exemption.

With the advent of new technologies, such as cell and gene therapy, we have seen rare patients (children and adults) facing life threatening and debilitating diseases being offered a life and a future. We urge the Governor, the PDACC and the PDAB to remove orphan therapies from the priority list. Price controls and limited access for rare patient communities cause disruptions to the continuity of care, creating a long-term negative impact on a vulnerable community where one size fits all policymaking doesn't meet the needs of rare and ultra-rare patients and communities.

Sincerely

Michael Eging
Executive Director
RAAP

Cc:

The Honorable Jared Polis, Governor
The Honorable Michael Conway, Commissioner, Division of Insurance
The Honorable Kim Bimestefer, Executive Director, Department of Health Care Policy and Financing