



April 4, 2024

Senator James Coleman
Chair, Committee on State, Veterans, & Military Affairs
Colorado General Assembly
200 E Colfax RM 346
Denver, CO 80203
james.coleman.senate@coleg.gov

Re: SB24-060; Prescription Drug Affordability Board Exemption for Orphan Drugs

Dear Senator Coleman:

Aimed Alliance is a not-for-profit health policy organization that seeks to protect and enhance the rights of health care consumers and providers. We appreciate the opportunity to comment on Senate Bill 24-060, Prescription Drug Affordability Board Exempt Orphan Drugs. **Aimed Alliance supports exempting orphan drugs from review by the Colorado Prescription Drug Affordability Board (PDAB).**

Orphan drugs are prescription drugs developed to treat rare diseases or conditions that are characterized by their low prevalence, typically impacting less than 200,000 individuals living within the United States.¹ The National Institute of Health has recognized that of the nearly 7,000 rare disorders identified in the United States, only 500 have approved treatments.² Consequently, millions of individuals across the United States lack access to treatments for their conditions, resulting in patients, caregivers, and providers focusing on symptom management rather than addressing the root cause of these disorders.

The investment, research, and development of treatments for rare disorders are frequently hindered by concerns regarding the limited populations affected by these conditions.³ Fortunately, federal programs such as the Orphan Drug Act help incentivize pharmaceutical companies to invest in this type of research and drug development through tax credits, user fee exemptions, and market exclusivity protections.⁴ For individuals and families living with rare disorders, these incentives are essential for advancing research and development efforts aimed at tackling the underlying causes of these conditions. Ultimately, they hold the potential to improve life expectancy and enhance the quality of life for individuals with rare disorders.

The imposition of upper payment limits on rare disease drugs by the Colorado PDAB could present a significant risk to the development of rare disease treatments and is unlikely to

¹ Orphan Drug Act, <https://www.fda.gov/industry/designating-orphan-product-drugs-and-biological-products/orphan-drug-act-relevant-excerpts>.

² NIH, *Rare Disease*, <https://www.nih.gov/about-nih/what-we-do/nih-turning-discovery-into-health/promise-precision-medicine/rare-diseases#:~:text=Advances%20in%20diagnosis%20of%20rare.about%20500%20have%20approved%20treatments..>

³ Nathan Yates & Jennifer Hinkel, *The economics of moonshots: Value in rare disease drug development*, <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC9010265/>.

⁴ *Id.*



improve access to already approved rare disease treatments. A study conducted in 2019 found that commercial health plans provided coverage for orphan drugs without restrictions in 70 percent of cases, while 29 percent were subject to some form of benefit utilization policies.⁵ Ultimately, the study revealed that only one percent of orphan drugs were not covered by plans, compared to nearly six percent of non-orphan drugs.⁶ Therefore, given that many rare disease drugs are already covered by health plans, setting upper payment limits on the cost of orphan drugs may have limited impact on improving access and coverage for these treatments.

While an upper payment limit may not affect consumers already benefitting from available rare disease treatments, its imposition could substantially impede ongoing research and development efforts for other rare disorders. This is particularly concerning given that over 90 percent of rare disorders lack approved treatments. Limiting investments in rare disease treatments could have devastating consequences for patients reliant on these treatments to target the underlying causes of their conditions. By exempting orphan drugs from affordability reviews and price setting, Senate Bill 24-060 would safeguard access to these treatments for individuals with rare diseases in Colorado.

In conclusion, we urge you to support Senate Bill 24-060 and take action to protect the health of Colorado residents who rely on orphan drugs and ongoing advancements in rare disorder treatments. We appreciate your time and consideration of this important issue.

Sincerely,

Ashira Vantrees
Counsel

⁵ James D. Chambers & Ari D. Panser, *Variation in US Private Health Plans' Coverage of Orphan Drugs*, <https://www.ajmc.com/view/variation-in-us-private-health-plans-coverage-of-orphan-drugs>.

⁶ *Id.*