NORD®
National Organization for Rare Disorders

Alone we are rare. Together we are strong.®

January 29, 2024

The Honorable R. Creigh Deeds
Senator, Senate of Virginia
Chair, Committee on Commerce & Labor
Room 612
General Assembly Building
201 North Ninth Street
Richmond, VA 23219

The Honorable Jeion A. Ward
Delegate, Virginia House of Delegates
Chair, Committee on Labor & Commerce
Room 1009
General Assembly Building
201 North Ninth St.
Richmond, VA 23219

Re: Recommendations Relating to SB274/HB570, An Act Establishing a Prescription Drug Affordability Board (PDAB) in the Commonwealth of Virginia

Dear Chair Deeds and Chair Ward,

On behalf of the more than 30 million Americans living with one of the over 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) writes to respectfully offer recommendations relating to the possible establishment of a Prescription Drug Affordability Board (PDAB) in the Commonwealth of Virginia as proposed in Senate Bill 274/House Bill 570. The goal of these recommendations is to ensure the unique needs of the rare disease patient community are appropriately considered within the context of this legislation.

As written, SB274/HB570 establishes a Prescription Drug Affordability Board (PDAB, Board) within the State Department of Health, as well as a Stakeholder Council (Council). The Board, with input from the Council, would have a mandate to set an upper payment limit (UPL) for prescription drugs determined to create affordability challenges to health care systems or lead to high out-of-pocket costs to patients within the Commonwealth of Viriginia, which could have significant implications for the rare disease patient community NORD proudly represents.

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 (ODA), to formalize the coalition of patient advocacy groups that were instrumental in passing that landmark law. NORD's 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.

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. Although rare disease patients can face significant health and financial obstacles regardless of whether a disease-specific treatment option exists for their condition, more than 95% of rare diseases do not have an FDA-approved therapy. This makes continued research and innovation into new therapies especially important to the rare disease community. Therefore, it is critical that legislation 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. In a desire to reflect that balance, NORD respectfully submits the following considerations and recommendations for SB274/HB570 as it moves through the legislative process.

Recommendation 1: Recognize the unique value orphan drugs bring to patients with few or no other safe and effective treatment options and define how orphan drug status will be considered in the selection and affordability review processes.

As written, SB274/HB570 does not include any references to orphan drug status, which NORD believes should be clearly and explicitly factored into the selection, affordability review and upper payment limit setting processes. Specifically, we urge you to carefully consider the following aspects of the rare disease patient experience and therapy development and ensure they are appropriately weighted when selecting drugs for, and when conducting, an affordability review:

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 30 million Americans are living with a rare disease, but only about 5% of rare diseases have an FDA-approved therapy. This means that millions of Americans living with a rare disease are left with significant unmet medical needs and significant financial impacts. According to a recent Orphanet study that reviewed data for just 379 of the more than 7,000 known rare diseases, the total economic burden for the analyzed diseases amounted to $997 billion in 2019, with $449 billion in direct medical costs and $439 billion in non-medical costs.

Patients living with a rare condition that does have a safe and effective therapy need access to that therapy in order to achieve their optimal health and usually have few, if any, alternative treatment options. Timely access to these therapies 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 can face significant disease progression, increased out of pocket health care costs, and even die without access to treatment. Patient access to safe and effective therapies is critical to the rare disease community, but it also benefits broader society.

Research and development into therapies to treat rare diseases is particularly challenging. Rare diseases impact small patient populations 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 (ODA), which established a set of incentives to encourage rare disease drug development. According to the FDA's orphan drug product database, since the passage of the ODA, manufacturers have obtained 6749 orphan designations,[1] 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 1200 orphan indications[2] have been determined to be safe and effective and therefore, FDA approved. The ODA is working as intended, helping to spur research into the development of new and better therapies to treat rare diseases, but millions of Americans are still waiting and hoping for a treatment for their condition.

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 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 be considered in affordability review and upper payment limit setting processes 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: NORD recommends amending the language in Section § 32.1-276.16. Subsection C. such that whether a drug has an orphan designation tied to an indication, is one of the key factors considered by the Board when determining whether to conduct an affordability review on a prescription drug product. NORD also strongly urges consideration for its inclusion as a key factor in selecting drugs for review as provided in Section § 32.1-276.16. Subsection D, and explicitly outline how having a designation tied to an indication will be considered to bring consistency and transparency into the process.

[1] US FDA, Search Orphan Drug Designations
[2] US FDA, Search Orphan Drug Approvals

Recommendation 2: To ensure the effectiveness of the affordability review, establish robust engagement opportunities for patients and caregivers so they can meaningfully contribute their unique insights.

NORD acknowledges and appreciates that SB274/HB570 has outlined mechanisms for written and verbal public input during the PDAB's deliberation process. However, to most effectively serve the communities impacted by high-cost prescription drugs, direct engagement with patients, providers and caregivers with experience with the products under review is crucial to the determination of if a product is affordable. Patients and caregivers have key insights on issues such as out-of-pocket costs, determining the value of a therapy and how it compares to potential alternative treatment options, or if any alternative treatment options even exist.

Rare disease patients and their caregivers are often uniquely positioned to share the challenges associated with unmet medical needs - when there are no or very few options available to treat their condition - and the benefits to themselves, their families, and the community from a safe and effective therapy. Because published data to assess these specific uses remains scarce, patients and providers are often the best experts from which to elicit such information related to rare disease treatments.

Furthermore, rare disease patients frequently have unique experiences with products that may differ from those using the product for common indications. For example, the experiences of an individual using a product for rheumatoid arthritis (typically a non-rare disease), may differ significantly from the experiences of an individual using the same product for the treatment of pediatric Crohn's disease.

Preliminary results from other PDABs around the country, such as Colorado, have found soliciting patient input on the selected products to have tremendous impact on the final determination of affordability for a product. Limiting opportunity for engagement to open-ended public testimony and written submissions, rather than intentional engagement with the patient community and their advocates, risks excluding valuable insight from a broad range of those impacted by the selected product.

Recommendation: NORD recommends explicitly including patient, caregiver and provider listening sessions for those with experience with the products under review, during both the selection process and the affordability review.

Recommendation 3: The Virginia Rare Disease Advisory Council should be a resource for the prioritization and affordability review of products with orphan indications.

The Commonwealth established a Rare Disease Advisory Council (RDAC) in 2021.[3] The vision of Virginia's RDAC is to "serve as a voice for Virginia's Rare Disease Community by advising policymakers and empowering families and individuals affected by rare diseases."[4] This vision is in alignment with its statuary purpose to advise the Governor and the General Assembly on the needs of those affected by

[3] VA Code Ann. §32.1-73.14 Added by Acts 2021, Sp. S.I, c. 303, eff. July 1, 2021.
[4] Virginia Department of Health, Rare Disease Council.

rare diseases, identify challenges and barriers faced by those affected with rare diseases; and direct funding of research and supports for persons with rare diseases. The Virginia 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 health insurance industries. Also represented are rare disease patient organizations and those with an understanding of the rare disease research landscape.

Recommendation: Virginia's Rare Disease Advisory Council should explicitly be listed as resource to the PDAB and Council in product prioritization determinations and when a product be selected for an affordability review that has any orphan designations tied to an indication.

In conclusion, NORD believes any legislation to address prescription drug costs must reflect a careful balance between greater affordability and maintaining appropriate incentives for continued investment. This is especially critical in rare disease drug development given the tremendous unmet needs that still exist. Please consider NORD a resource as these bills move through the legislative process. For questions regarding NORD or our recommendations, please contact Carolyn Sheridan, State Policy Manager, Eastern Region at csheridan@rarediseases.org, or Mason Barrett, Policy Analyst at mbarrett@rarediseases.org.

Sincerely,

Carolyn G Sheridan MPH,
State Policy Manger, Eastern Region
National Organization for Rare Disorders

Mason Barrett
Policy, Analyst
National Organization for Rare Disorders

CC:
The Honorable Karrie K. Delaney, Delegate, Virginia House of Delegates
Tracy Eppard, Legislative Director for Sen. R. Creigh Deeds
Jacqueline Garry, Legislative Assistant for Del. Karrie Delaney
Randall Riffle, Legislative Assistant for Del. Jeion Ward