



October 23, 2023

Christina Shaklee  
Health Policy Analyst Advanced  
Maryland Prescription Drug Affordability Board  
16900 Science Drive, Suite 112-114  
Bowie, MD 20715

Dear Ms. Shaklee,

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) would like to thank you for your commitment to ensuring that all Marylanders have access to affordable medications. We are writing today with a request to actively participate in the work of the Prescription Drug Affordability Board (PDAB) as it stands up the infrastructure to begin its first set of affordability reviews and potential upper payment limit setting. We understand the enormity of the task and believe we are well suited to lend our expertise as the PDAB continues its work.

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, to formalize the coalition of patient advocacy groups that were instrumental in passing that landmark law. Our mission has always been, and continues to be, to improve the health and well-being of people living with rare diseases by driving advances in care, research, and policy.

**Recommendation 1: NORD urges more explicit consideration of orphan drug status in both the prioritization and selection of therapies and affordability review phase.**

NORD is encouraged that a drug's orphan status will be considered when the PDAB is conducting its initial drug selection. NORD believes consideration of orphan status should ultimately reflect a delicate balance between affordable patient access to often life-changing therapies, and vital incentives for continued research in rare disease drug development. However, we are concerned about the lack of clarity in the proposed regulation regarding how orphan drug status will be considered.

As currently written, orphan status will only be considered during the initial drug selection phase. While there are some important factors for consideration during the affordability review phase that are particularly impactful for the rare disease community, such as therapeutic alternatives, utilization management policies, and patient out of pocket cost, the fact that a product treats orphan diseases can factor into PDAB considerations in a number of nuanced ways. As such, NORD requests greater clarity and transparency into how products with orphan designations will be considered in the prioritization and selection of therapies for the affordability review.

Research and development into therapies to treat rare diseases is particularly challenging. By definition, rare diseases impact small patient populations<sup>1</sup> that are often geographically dispersed. Limited natural history data and knowledge of disease progression combined with heterogeneous disease presentation mean that clinical trials

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

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, which established a set of incentives to encourage rare disease drug development.<sup>2</sup> According to the FDA's orphan drug product database, since the passage of the Orphan Drug Act, manufacturers have obtained 6634 orphan designations,<sup>3</sup> 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 1175 orphan indications<sup>4</sup> have been determined to be safe and effective and therefore, FDA approved. Even with the ODA's incentives in place, more than 95% of rare diseases do not have an FDA-approved therapy<sup>5</sup> and often there are no therapeutic alternatives available when there is an existing rare disease therapy.

The tremendous unmet need that exists in the rare disease community has 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 5% of rare diseases have an FDA approved therapy, leaving millions of Americans with significant unmet medical needs. According to a study published in *Orphanet* in April 2022, a review of just 379 of the more than 7,000 known rare diseases showed a total economic burden of \$997 billion in 2019, with \$449 billion in direct medical costs and \$439 billion in non-medical costs.<sup>6</sup> 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, which 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 face significant disease progression, increased out of pocket health care costs, and even die without access to treatment. Timely patient access to safe and effective therapies is critical to the rare disease community, but is also beneficial to broader society.

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 currently 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 become eligible for affordability reviews and upper payment limit setting 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.

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<sup>2</sup> Ibid.

<sup>3</sup> <https://www.accessdata.fda.gov/scripts/opdlisting/oopd/listResult.cfm>

<sup>4</sup> Ibid.

<sup>5</sup> <https://rdcu.be/dh0XV>

<sup>6</sup> <https://pubmed.ncbi.nlm.nih.gov/35414039/>

**Recommendation 2: NORD urges the Board to provide opportunities for more robust patient engagement and protect confidentiality.**

NORD appreciates the PDAB providing forums for patients to submit information on a drug or drugs that have caused or are causing affordability issues for the individual. However, we are concerned that the lack of clarity on how patient data will be treated could limit individual participation. To ensure that patients feel comfortable that their data will be protected, we recommend clarifying explicitly that patient submitted information will be treated as “proprietary data and information” and not subject to public availability.

In addition to the opportunity to provide written comments, NORD recommends the PDAB provide the opportunity for patients and caregivers to submit information live through patient listening sessions. Live questions and testimonials can elucidate additional dimensions of value that may otherwise not be captured in written submission. Live patient testimonials can also help patients with accessibility challenges participate in a process they may otherwise not be able to. Colorado’s PDAB has had success recently in receiving both written and verbal patient input and could provide a roadmap for how the Maryland PDAB could engage with patients. Colorado’s stakeholder engagement guide can be found [here](#). Additionally, FDA’s [condition-specific meeting reports](#) on patient experience can be used to help fill in the gaps to help inform patient listening sessions.

**Recommendation 3: NORD recommends formalizing inclusion of the RDAC in the drug selection and affordability review process as an advisory voice.**

On May 3, 2023, the Governor Wes Moore signed into law legislation creating a Rare Disease Advisory Council (RDAC) in Maryland, to begin work in 2024. The stated objective of the RDAC is to study and make recommendations on matters relating to individuals with rare diseases in the state, as well as solicit public comment from the public on the needs of rare disease patients, caregivers, and health care providers. In addition to providing access to a panel of rare disease experts, the RDAC will likely be able to provide valuable input from Maryland’s rare disease patients and caregivers.

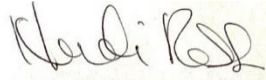
**Recommendation 4: Engage with NORD and other patient advocacy groups early and often in the process to collect a robust patient perspective.**

NORD is an umbrella organization for more than 330 rare disease-specific patient advocacy groups and does not engage in disease-specific or treatment-specific policy or advocacy work. Therefore, NORD is not seeking an opportunity to discuss the specifics of any of the potential selected products, but wants to more broadly discuss issues such as data scarcity that can make it challenging to conduct a comprehensive affordability review and/or set an upper payment limit, the access challenges many of our patients face, as well as the significant unmet medical need that necessitates continued investment in drug development. We hope that we can be a resource in determining how to best factor these realities into the determination of affordability and upper payment limits.

NORD is uniquely positioned to provide data and to connect patients directly to stakeholder opportunities. We are eager to serve as a resource to the PDAB it conducts its work throughout the affordability review process. As you develop patient stakeholder opportunities such as surveys and listening sessions, we would welcome the opportunity to meet with you and provide resources that will assist in your review. Please contact Heidi Ross, Vice President of Policy and Regulatory Affairs, at [HRoss@rarediseases.org](mailto:HRoss@rarediseases.org), Mason Barrett, Policy Analyst, at [MBarrett@rarediseases.org](mailto:MBarrett@rarediseases.org), or Carolyn Sheridan, State Policy Manager, at [csheridan@rarediseases.org](mailto:csheridan@rarediseases.org) to schedule a meeting at your earliest convenience.

Thank you for your consideration of our request, and we look forward to hearing from you.

Sincerely,

Handwritten signature of Heidi Ross in black ink.

Heidi Ross, MPH  
Vice President, Policy and Regulatory Affairs  
National Organization for Rare Disorders

Handwritten signature of Mason Barrett in black ink.

Mason Barrett  
Policy Analyst  
National Organization for Rare Disorders

Handwritten signature of Carolyn Sheridan in black ink.

Carolyn Sheridan, MPH  
State Policy Manager  
National Organization for Rare Disorders

CC: Members of the Maryland Prescription Drug Affordability Board

