September 20, 2023

The Honorable Mary Cavanagh
Chair
Senate Committee on Finance, Insurance, and Consumer Protection
Michigan Senate
6500 Binsfeld Office Building
Lansing, Michigan 48909

Dear Chair Cavanagh and Members of the Senate Committee on Finance, Insurance, and Consumer Protection,

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 for changes to SB 483 (Camilleri) and its companion bills, SB 484 (Klinefelt) and SB 485 (McDonald Rivet). As written, SB 483 would establish a Prescription Drug Affordability Board (PDAB) within the State Department of Insurance and Financial Services, as well as the Prescription Drug Affordability Stakeholder Council (PDASC). Together, these entities would have the authority to set an upper payment limit for prescription drugs determined to create affordability challenges to health care systems or patients within the State of Michigan, 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 as a whole. For Americans living with a rare disease, high drug prices can hinder their ability to access needed therapies. Although rare disease patients face significant health and financial obstacles regardless of whether a disease-specific treatment option exist 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 recommendations for changes to SB 483 and its companion bills, SB 484 and SB 485.

Recommendation 1: Recognize the unique value orphan drugs bring to patients with 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, SB 483 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

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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 any FDA approved therapy. This means that millions of Americans living with a rare disease are left with significant unmet medical needs. 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.[1]

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 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. By definition, rare diseases impact small patient populations[2] 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 ODA, which established a set of incentives to encourage rare disease drug development.[3] According to the FDA's orphan drug product database, since the passage of the ODA, manufacturers have obtained 6596 orphan designations,[4] 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 1166 orphan indications[5] 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 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.

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[1] https://pubmed.ncbi.nlm.nih.gov/35414039/
[2] https://www.fda.gov/industry/designating-orphan-product-drugs-and-biological-products/orphan-drug-act-relevant-excerpts
[3] Ibid.
[4] https://www.accessdata.fda.gov/scripts/opdlisting/oopd/listResult.cfm
[5] Ibid.

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 selected 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.

Recommendation: NORD recommends amending the language in §11 subparagraphs (a) to (j) of subdivision (4) of Senate Bill 483 such that orphan drug status is one of the key factors considered in affordability reviews. NORD also strongly urges consideration for its inclusion as a key factor in selecting drugs for review as provided in §11 subdivision (1) and explicitly outline how a products orphan drug status will be considered to bring consistency and transparency into the process.

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.

Currently, SB 483 has no mechanism for direct patient input into the selection, affordability review, or the upper payment limit setting processes. However, to be truly successful, any affordability review and possible upper payment limit determination should not only consider the savings that accrue in the near term on prescription drug costs, but also must be evaluated based on the impacts on patients, communities, and the broader health care system. 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. Rare disease patients are also 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 remain scarce, patients and providers are often the best experts from which to elicit such information related to rare disease treatments.

While NORD acknowledges the specific factors outlined in §11 subparagraphs (a) to (j) of subdivision (4) of SB 483 offer manufacturers the opportunity to submit for data for consideration in the affordability review itself, the factors outlined in subparagraphs (f) to (h) are also ones with which patients could have valuable data and insights. Additionally, subparagraph (h) references “relative financial impact to health, medical, or social service costs as can be quantified and compared to baseline effects of existing therapeutic alternatives, but it doesn’t require consideration of how a product will be treated that does not have a therapeutic alternative, which is often the case for many therapies that exist to treat rare diseases. Patient and caregiver input into these factors is essential.

Recommendation: Patient input must be an integral part of an affordability review but also in the prioritization of drugs for review to ensure any recommendations to address prescription drug costs truly reflect the often-complex needs of patients. Therefore, NORD strongly encourages the Committee to include robust opportunities for patients to provide critical input into their lived experience with, and/or without, access to a therapy to the PDAB and PDASC. FDA listening sessions, patient focused drug development meetings, and other FDA-led initiatives routinely collect meaningful patient experience data in ways that works for rare disease patients and families and can serve as another valuable guide and resource for the PDAB and PDASC in effectively engaging patients.

Recommendation 3: Pass legislation enacting the Michigan Rare Disease Advisory Council (HB 4167/Morgan) to be a resource for the selection and affordability review of products that may have orphan indications.

The State of Michigan has the opportunity to join the community of 27 other states that have given their rare disease patients a stronger voice in state government by creating a Rare Disease Advisory Council (RDAC).

Michigan's RDAC would be comprised of patients, caregivers, healthcare providers, clinical experts and researchers, amongst other rare disease community stakeholders. The Michigan Legislature has already approved a $70,000 appropriation for the establishment of an RDAC as part of the FY 2024 General Omnibus budget. Furthermore, on March 22, 2023, the Michigan House of Representatives passed HB 4167 (Morgan) which would establish the Council, seizing their opportunity to provide direction on how the Council should be implemented and the already appropriated funds utilized.

Recommendation: By passing HB 4167 through the Senate and sending it to the Governor's desk for approval, you are providing the PDAB and PDASC with a critical resource to tap into should a product be selected for an affordability review that has an orphan 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 Heidi Ross, Vice President, Policy and Regulatory Affairs at HRoss@rarediseases.org or Carolyn Sheridan, State Policy Manager, Eastern Region at csheridan@rarediseases.org.

Sincerely,

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CC: The Honorable Jeremy Moss, Majority Vice Chair
The Honorable Mark Huizenga, Minority Vice Chair
Mike Sitkauskas, Committee Clerk