CYSTIC FIBROSIS FOUNDATION®

November 20, 2023

Maryland Prescription Drug Affordability Board
16900 Science Drive, Suite 112-114
Bowie, MD 20715

Dear Members of Maryland's Prescription Drug Affordability Board:

On behalf of people living with cystic fibrosis (CF) in Maryland, the Cystic Fibrosis Foundation writes to provide comments on 14.01.04 *Cost Review Study Process*. We appreciate the need to improve affordability of care for Marylanders and address the rising cost of prescription drugs to ensure sustainability of the state's health care system. However, as the Maryland Prescription Drug Affordability Board (PDAB) considers how to design its cost review study process, the paramount goal must be preserving access to care for patients. We recommend additional provisions, outlined below, to ensure that the PDAB centers the needs of patients, including those living with CF, when selecting drugs for cost review study and throughout the cost review process.

About Cystic Fibrosis & the Cystic Fibrosis Foundation
Cystic fibrosis is a life-shortening genetic disease that affects nearly 40,000 children and adults in the United States, including nearly 600 in Maryland. CF causes the body to produce thick, sticky mucus that clogs the lungs and digestive system, which can lead to lung damage, life-threatening infections, malnutrition, and other complications. Cystic fibrosis is both serious and progressive; lung damage caused by infection is often irreversible and can have a lasting impact on length and quality of life, resulting in extended hospitalizations, transplant, or premature death. As a complex, multi-system condition, CF requires targeted, specialized treatment and medications. There is no cure.

As the world's leader in the search for a cure for CF and an organization dedicated to ensuring access to high-quality, specialized CF care, the Cystic Fibrosis Foundation supports the development of CF clinical practice guidelines and accredits more than 130 care centers nationally – including two in Maryland.

Stakeholder Engagement Process
The Foundation appreciates the PDAB's inclusion of a stakeholder process through which Maryland patients, their caregivers, and the clinical providers who care for them can provide input on the PDAB's selection and cost review of prescription drugs. We recommend the following additions to the stakeholder engagement process to ensure that it is as accessible and transparent as possible and that it centers the patient voice throughout the process.

It is essential that patients, caregivers, and clinical providers who are experts in treating the diseases impacted by such reviews can participate in this process as early as possible and that their feedback is heard throughout the entire cost review. The PDAB should also take care to educate patients, providers, and other members of the public about its process and timeline for selecting drugs for cost review, how the study will be conducted, and any potential outcomes from the review. We ask that the PDAB provide diverse opportunities for patient involvement and recognize time and technology limitations that

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community members may see as barriers to engagement. For example, we encourage the Board to provide multiple opportunities for involvement at a variety of times to accommodate adults and caregivers that are working and unable to join a meeting during business hours. Avenues for public engagement can include online surveys, written comments, oral testimony, and patient-focused activities like focus groups or small group meetings. It is also crucial that patients be involved in the development of survey and focus group questions.

Transparency of the Process

The PDAB should be transparent about their processes, methods, and utilization of value assessments. It is important that the PDAB be transparent throughout the entire process by making the drug dashboard – with the exception of proprietary information – visible to the public and only having discussions about cost reviews during public meetings. The PDAB must also be transparent to the public about how data and information collected, especially from patients, will be used in the decision-making process and with whom it will be shared. The Foundation would also like to emphasize the importance of explaining the process in a lay friendly manner to ensure that the public can understand the process and authentically engage with the PDAB.

Cost Review Study Criteria

Orphan Drug Status

The CF Foundation thanks you for including rare disease designation as a data point that will be available to Board members on the dashboard. However, we urge you to also include orphan drug status as a criterion when the PDAB selects drugs for cost review, conducts cost review studies, and determines whether the drug poses an affordability challenge. The small number of patients in rare disease populations can create unique challenges for drug development and present different market considerations compared to other therapies. The PDAB should consider orphan drug status alongside other existing factors already outlined in 14.01.04 *Cost Review Study Process* to ensure a more comprehensive view of the treatment and access landscape for patients.

Length of time on market

It is important the PDAB establish a minimum period of time that drugs must be on the market before they are eligible for PDAB review. While data from clinical trials is important for establishing safety and efficacy, it can take years to fully understand the benefits of a given drug. For instance, collection of real-world evidence is vital to understand how a drug impacts patients in a real-life setting. Such data also allows researchers to capture information on additional outcomes beyond those evaluated in a clinical trial, such as patient-reported outcomes related to quality of life, productivity, and well-being. For diseases with complex care regimens such as cystic fibrosis, it is also important to also give adequate time to study the impact of a new therapy on other aspects of care. These studies require ample time to assess changing existing care in response to new treatments. Collection of real-world evidence takes time as well and may not be available until a drug has been on the market for a number of years.

Moreover, in cystic fibrosis, the Food and Drug Administration initially approved CFTR modulators for people with certain genotypes ages 12 and up. As sponsors collect additional data, the labels have been expanded to include additional genotypes and younger age groups. As a progressive disease, understanding the impacts of CFTR modulators on younger populations is essential for a comprehensive cost review as these treatments may delay or halt disease progression, thus impacting healthcare utilization, productivity, and the overall trajectory of cystic fibrosis. For these additional populations, it will take time to understand the full benefit of CFTR modulators on life-expectancy, as one example. As

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such, 14.01.04 *Cost Review Study Process* should establish a minimum time that drugs must be on the market before they are eligible for a cost review.

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Thank you for the opportunity to comment on 14.01.04 *Cost Review Study Process*. The Cystic Fibrosis Foundation stands ready to serve as a resource as the PDAB explores solutions to reduce prescription drug costs for Marylanders. Please contact Amanda Attiya, state policy specialist, at aattiya@cff.org with any questions about this issue.

Sincerely,

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