CYSTIC FIBROSIS FOUNDATION®

February 6, 2025

Health and Government Operations Committee
Maryland General Assembly
240 Taylor House Office Building
Annapolis, Maryland 21401

Dear Honorable Members of the House Health and Government Operations Committee:

On behalf of the people living with cystic fibrosis (CF) in Maryland, the Cystic Fibrosis Foundation writes to provide comments on HB 424. We have appreciated the opportunity to engage with the Maryland Prescription Drug Affordability Board (PDAB) as it developed the Upper Payment Limit (UPL) Action Plan and support many elements of the approved plan. However, we believe that there are several provisions that should be added to ensure that the needs of people living with a disease, including CF, are at the center of the discussion when considering whether and how to set an UPL. We recommend additional provisions as outlined below for your consideration as you authorize the PDAB to move forward with implementing UPLs.

About Cystic Fibrosis & the Cystic Fibrosis Foundation
Cystic fibrosis is a progressive, genetic disease that affects the lungs, pancreas, and other organs. There are close to 40,000 children and adults living with cystic fibrosis in the United States, including more than 570 people in Maryland, and CF can affect people of every racial and ethnic group. 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 and Transparency
We are glad to see that the approved UPL Action Plan includes stakeholder processes through which people living with a disease and their caregivers can provide input on the PDAB's consideration of policy solutions to address drug unaffordability, including setting UPLs. We recommend that the legislature amend HB 424 to also require the PDAB to provide diverse opportunities for stakeholder involvement to address time and technology limitations at all stakeholder engagement points, including when a preliminary determination is made, during the policy review process, and when considering a UPL. For example, we encourage the legislature to require the Board to provide multiple hearings at a variety of times to accommodate adults living with a disease and adult caregivers that are working and unable to join a meeting during business hours. Other avenues for public engagement can include online surveys and focus groups. It is also crucial that people living with a disease and caregivers be involved in the development of survey and focus group questions. All these processes should be conducted if the PDAB begins any development of policy recommendations or determinations of upper payment limits.

We also appreciate that the approved UPL Action Plan requires the PDAB to be transparent about their processes and methodologies for determining whether a drug has led to or will lead to an affordability

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challenge, whether to set a UPL or pursue other policy actions, and what amount the UPL should be. We emphasize the importance of explaining the process in a lay friendly manner to ensure the public can understand the process and authentically engage with the PDAB throughout the proposed policy review process and any UPL determinations. HB 424 should also require the PDAB to educate people living with a disease, providers, and other members of the public about its process and timeline for setting a UPL, how the UPL will be calculated, and any potential outcomes. The PDAB should be transparent to the public about how data and information collected, especially from people living with a disease, will be used in the decision-making process and with whom it will be shared as well.

Consideration Criteria

While we appreciate that the approved UPL Action Plan includes various criteria the Board must consider when setting UPLs, we request that HB 424 include additional criteria as outlined below.

Availability of therapeutic alternatives

We urge you to include the availability of therapeutic alternatives as a criterion the PDAB must consider when determining whether to set an upper payment limit, as this is a critical factor in understanding the market for a particular therapy and deciding whether to establish a UPL. In CF care, treatments are finite, and therapeutic alternatives are often not available. For example, a class of drugs called CFTR modulators only works for individuals with certain genetic profiles; they are not interchangeable and there are currently no generics or therapeutic alternatives. When evaluating the market for a particular drug and whether to establish a UPL, the PDAB must consider the availability of therapeutic alternatives as there are unique access concerns for drugs without alternatives.

Moreover, given that the PDAB only has authority over state and local government spending, the board has limited leverage for drugs without therapeutic alternatives. In contrast to Medicare price negotiation—where manufacturers would be hard pressed to walk away from all Medicare beneficiaries—the PDAB is dealing with a relatively small market, and a single source manufacturer may be willing to give up the state market rather than accept a UPL. Upper payment limits may hold more promise for drugs with therapeutic equivalents; it is feasible that a state could achieve savings in this scenario while preserving access for patients.

Orphan drug status

The CF Foundation urges the legislature to include orphan drug status as a criterion when the PDAB determines whether to set an upper payment limit. The small number of people in rare disease populations can create unique challenges for drug development and present different market considerations compared to other therapies. This limited market size for these kinds of drugs is a factor in determining the price of these therapies, and it is important to preserve financial incentives to bring more drug developers into this space as there are many rare diseases without any approved treatment. For instance, some CF treatments are indicated based on specific genetic variants and therefore even for a disease like CF with many approved treatments, there must still be incentives to continue investing in this space. The PDAB should consider orphan drug status alongside other existing factors already outlined in the approved UPL Action Plan to ensure a more comprehensive view of the treatment and access landscape for people living with a rare disease.

Length of time on market

HB 424 should establish a minimum period of time that drugs must be on the market before they are eligible for a UPL. 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 people living with a disease 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 affordability review as these treatments may delay or halt disease progression, thus impacting healthcare utilization, productivity, and the overall trajectory of cystic fibrosis. As such, HB 424 should establish a minimum time that drugs must be on the market before they are eligible for a UPL.

Lived experiences of people living with a disease
Cost-effectiveness methodologies cannot accurately measure value if they do not include data on the experiences, preferences, and outcomes reported by people living with a disease. To that end, HB 424 should require that the PDAB seek out patient-reported data for UPL determinations, including patient surveys, focus groups, presentations from patient-focused drug development meetings, and registry data. This is essential to complement data from clinical trials, claims data, and other sources and give a full picture of how a therapy works for people living with a disease.

Additional Policy Recommendations
As the legislature considers a range of policy remedies to address drug affordability, the Foundation also recommends consideration of measures related to accumulator programs and PBM reform. We supported legislation during Maryland's 2024 legislative session that would ban accumulator programs by requiring insurers to apply patient assistance to their health plan cost-sharing requirements. [1] Patients with chronic diseases like CF often struggle to afford their care and rely on copay assistance to access vital medications. We recognize that copay assistance is problematic—allowing pharmaceutical companies to charge payers high prices, while shielding many individual patients from the costs—but banning copay accumulator programs helps ensure patients' health and financial wellbeing are not sacrificed in the ongoing debate between payers and pharmaceutical companies about prescription drug prices. The Maryland State Senate is currently considering similar legislation—SB 773—and the Foundation encourages you to pass this bill should it advance to the General Assembly.[2]

Additionally, many states are pursuing reforms of pharmacy benefit managers (PBMs) to address drug affordability, including establishing fiduciary requirements, prohibiting spread pricing, increasing transparency, and pharmacy network reforms. We are encouraged to see that the Maryland legislature has previously considered legislation that would implement some of these provisions.[3] These measures will create more pressure on plans and PBMs to put the interests of the patient first and can have a direct impact on consumer costs.[4]

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[1] Cystic Fibrosis Foundation letter supporting SB 595 that would require insurers to apply patient assistance to their health plan cost-sharing requirements. February 27, 2024. Available at: https://www.cff.org/statements/2024-02/maryland-ban-co-pay-accumulators-alternative-funding-plans
[2] SB 773. Available at: https://mgaleg.maryland.gov/2025RS/bills/sb/sb0773F.pdf
[3] SB 896. Available at: https://mgaleg.maryland.gov/2023RS/bills/sb/sb0896F.pdf
[4] U.S. Government Accountability Office, *Prescription Drugs: Selected States' Regulations of Pharmacy Benefit Managers*, March 18, 2024. Available at: https://www.gao.gov/products/gao-24-106898

Thank you for the opportunity to comment on HB 424. The Cystic Fibrosis Foundation stands ready to serve as a resource as the legislature explores solutions to improve access to and affordability of care for Marylanders. Please contact Amanda Attiya, State Policy Specialist, at aattiya@cff.org or 240-482-2879 with any questions about this issue.

Sincerely,

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