CYSTIC FIBROSIS FOUNDATION

May 8, 2024

House Health & Human Services Committee
Rhode Island State House
82 Smith Street
Providence, RI 02903

Dear Honorable Members of the House Health & Human Services Committee:

On behalf of the people living with cystic fibrosis (CF) in Rhode Island, the Cystic Fibrosis Foundation writes to provide comments on H8220. We appreciate the need to address rising prescription drug spending to ensure sustainability of the state's health care system, while also maintaining access to critical therapies for Rhode Islanders who depend on them. We recommend the following amendments to H8220 as outlined below to ensure the Drug Cost Review Commission puts the needs of people living with a disease, including CF, at the center of the discussion when selecting drugs for review, conducting reviews, or considering upper payment limits (UPL).

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 110 people in Rhode Island, 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 one in Rhode Island.

Goals of Drug Cost Review Commission
We caution that H8220 may be working towards two separate aims that require separate consideration and policy solutions: reducing drug costs for Rhode Island's health care system and reducing drug costs for consumers. For instance, we note that one definition of excess costs in H8220 is "costs of appropriate utilization of a prescription drug product that are not sustainable to *public and private health care systems* over a ten (10) year timeframe." However, section 5-19.3-8 states that the commission shall set a reimbursement cap if it determines that a drug creates excess costs for "payors and consumers." Policies that cap reimbursement for drugs may not ultimately impact what consumers pay at the pharmacy counter and it is important to recognize this distinction as lawmakers proceed with this legislation. Any ambiguity about whether the commission is reviewing excess costs for health care systems or for consumers can create confusion about how the commission should review drugs and appropriate policy remedies.

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Due to the complexity of the U.S. health care system, there are many factors and entities involved in determining what patients pay for their drugs. In the case of CF, most people living with the disease rely on multiple forms of insurance as well as third-party assistance to cover the cost of their medications. Navigating these plans and assistance programs is burdensome and time consuming, but often means that people can afford the cost-sharing for their most expensive therapies.

Far too many people with CF still struggle to afford all of their care—which includes an extensive treatment and care regimen—but their affordability challenges are not always driven by the cost of one specialty drug. We share this information to highlight the complexity of prescription drug spending in this country and note that affordability challenges for the system do not always align with affordability challenges for consumers. We ask that lawmakers clarify the definition of excess costs and the circumstances in which reimbursement caps should be considered with these nuances in mind.

Reimbursement Level Setting

H8220 requires the commission to establish the level of reimbursement that payors, wholesalers, providers, and consumers may be billed if the commission determines that the drug creates excess costs for payors and consumers. However, we ask that lawmakers amend H8220 to give the commission the option to determine whether setting reimbursement levels is appropriate or whether another course of action is more prudent. For example, the law that established Colorado's Prescription Drug Affordability Board (PDAB) gives them the option to either set an upper payment limit, make policy recommendations to the legislature to improve prescription drug affordability, or take no further action. Capping reimbursement rates may not always be the best solution to lower costs for consumers, depending on specific drug and market considerations. H8220 should provide the commission with the freedom to consider all economic and political levers to determine what would be most effective and appropriate to lower prices for consumers.

Stakeholder Engagement

All boards conducting reviews on prescription drugs must have processes for stakeholders, especially people living with a disease who may be impacted by potential commission action, to meaningfully engage and inform processes and decision-making. We urge you to amend H8220 to include a robust stakeholder engagement process through which people living with a disease, their caregivers, and the clinical providers who care for them can provide input on drug reviews and proposals to set upper payment limits.

First, we ask that H8220 direct the commission to provide diverse opportunities for stakeholder involvement during drug selection, the review process, and upper payment limit determinations to address time and technology limitations for some people. For example, we encourage the commission to provide multiple opportunities for involvement 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. Avenues for public engagement can include online surveys, written comments, oral testimony, 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 of these processes should be conducted not only during drug selection and reviews, but also if the commission begins any determinations of upper payment limits.

We also request that H8220 require the commission to consider input from clinicians when conducting reviews. Clinicians who specialize in the treatment of certain diseases and conditions bring a critical perspective about the benefit of therapies and availability of alternative treatments, and the commission should be required to solicit their input. The commission should meet with clinicians at least

once during the review process to discuss the drug under review and their experience caring for people living with the condition treated by the drug.

Transparency of the Process

H8220 should require the commission to be transparent about their processes, methods, and utilization of value assessments. For example, any eligible drug list or dashboard developed by the commission – with the exception of proprietary information – should be visible to the public, and the commission should only have discussions about reviews during public meetings. The Foundation also emphasizes the importance of explaining the process in a lay friendly manner to ensure the public can understand the process and authentically engage with the commission.

H8220 should also require the commission to educate people living with a disease, providers, and other members of the public about its process and timeline for selecting drugs for review, how the review will be conducted, and any potential outcomes from the review. The commission 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.

Commission Composition

The commission should be statutorily required to include diverse membership, including members with expertise on the systemic impacts of market interventions recommended by the commission. For example, a health economist would help the commission understand and consider the potential implications of setting a UPL on the health care system overall, as well as on coverage and access for people who take the prescription drug. We appreciate that H8220 requires members to have "expertise in health care economics or clinical medicine." However, we request that the legislature amend this language to specify that both of these perspectives must be represented on the commission.

Drug Selection and Review Criteria

Orphan drug status

The CF Foundation urges the committee to include orphan drug status as a criterion when the commission selects drugs for review, conducts affordability analyses, and 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. The commission should consider orphan drug status alongside other existing factors already outlined in H8220 to ensure a more comprehensive view of the treatment and access landscape for people living with a rare disease.

Availability of therapeutic alternatives

We urge you to include the availability of therapeutic alternatives as a criterion the commission must consider when selecting drugs for review, conducting the affordability analysis, and determining whether to set an upper payment limit. 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. The commission must consider the availability of therapeutic alternatives as there are unique access concerns for drugs without alternatives.

Length of time on market

H8220 should establish a minimum period of time that drugs must be on the market before they are eligible for commission 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 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 review as these treatments may delay or halt disease progression, thus impacting healthcare utilization, productivity, and the overall trajectory of cystic fibrosis. As such, H8220 should establish a minimum time that drugs must be on the market before they are eligible for an review.

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, H8220 should require that the commission seek out patient-reported data for reviews and 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.

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Thank you for the opportunity to comment on H8220. 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 Rhode Islanders. Please contact Amanda Attiya, State Policy Specialist, at aattiya@cff.org with any questions about this issue.

Sincerely,

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Cystic Fibrosis Foundation