Submission to the Patented Medicines Prices Review Board
2022 Proposed updates to the PMPRB Guidelines

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Introduction

Cystic Fibrosis Canada is pleased to provide feedback to the Patented Medicines Prices Review Board (PMPRB) regarding the 2022 proposed updates to the PMPRB guidelines.

Cystic fibrosis is the most common fatal genetic disease affecting 4,332 Canadian children and young adults. There is no cure. Of the Canadians with cystic fibrosis who died in the past five years, half were under the age of 37. Cystic fibrosis is a progressive, degenerative multi-system disease that affects mainly the lungs and digestive system. In the lungs, where the effects are most devastating, a build-up of thick mucus causes severe respiratory problems. Mucus and protein also build up in the digestive tract, making it difficult to digest and absorb nutrients from food. In addition to the physical effects of the disease, mental health concerns are emerging; anxiety and depression are common among this population. Double lung transplants are the final option for patients with end-stage disease; most fatalities of people with CF are due to lung disease.

Cystic Fibrosis Canada has dramatically changed the cystic fibrosis story. We have advanced research and care that has more than tripled life expectancy. Since being founded by parents in 1960, Cystic Fibrosis Canada has grown into a leading organization with a central role engaging people living with cystic fibrosis, parents and caregivers, volunteers, researchers and healthcare professionals, government, industry and donors. We work together to improve the lives of Canadians living with cystic fibrosis through treatment, research, information and support.

Cystic Fibrosis Canada supports efforts to lower the costs of prescription drugs for Canadians. We believe that this can and must be done in a way that ensures timely access by Canadians to new medicines, especially precision medicines. We also support meaningful stakeholder engagement, as well as transparent and accountable practices. We do not support the proposed guideline changes and approach to implementation because they do not support these principles.

Meaningful and Timely Stakeholder Engagement

Stakeholder engagement was neither meaningful nor timely given the potential impact of the proposed guideline changes. The tight timeframe for consultations on the guidelines and the plan to implement on January 1, 2023, when feedback will only have been received on December 5, 2022 is rushed, and comes during the holiday break. It seems unlikely that the ministry will be able to fully consider stakeholder input during this time, leaving us to question the sincerity of the consultations.

The guidelines must be developed in full consultation with patients and their representatives. The consultation process must be extended and implementation must be deferred to allow for respectful consideration of stakeholder feedback prior to the implementation date.

Assessing the Impact on Access

Early access to new, innovative treatments serves the health needs of Canadians and will improve health outcomes. Canada's cystic fibrosis community is an excellent example of how transformative medicines can save and change lives. However, the uncertainty that the previous PMPRB guidelines created in the Canadian pharmaceutical environment directly and negatively impacted access to game-changing therapy Trikafta, the single greatest innovation in the history of cystic fibrosis.

The manufacturer was clear that it hesitated to bring the drug to Canada due to the uncertainty the proposed changes created in the market. Canadians with cystic fibrosis grew sicker and some died while watching their international counterparts access this life-changing medicine. When the guidelines were paused and the Gap Medicines clause was extended, the manufacturer finally launched the drug in Canada. In less than two years, Trikafta twice passed Health Canada approval and HTA assessment and was reimbursed by all of Canada's public drug programs; first for the 12 years and older cohort, followed by the 6-11 year old cohort.

Trikafta is changing the trajectory of cystic fibrosis. More importantly, it is changing lives. This drug is getting people off the lung transplant list and on with living, and it is giving kids hopes and dreams they never thought they'd have. If the guidelines were not delayed and ultimately stripped for constitutional overreach, we could still be fighting for access to this drug, more people would have grown sicker, and more would have died waiting.

The new proposed guidelines grant significant powers to staff to assess drug prices and to commence investigations, leading to greater uncertainty, but there has been no assessment of how such an approach may impact access. Efforts at reducing costs of medicines in Canada should not slow down patient access to potentially life-saving medications**.**

An independent third-party should be hired to conduct a formal impact assessment of the guideline changes on access to medicines (including access to clinical trials) in Canada and across therapeutic areas.

Accountability and Transparency

Cystic Fibrosis Canada has serious concerns about granting additional drug pricing powers to PMPRB staff without increased accountability and transparency. The risks this issue imposes are exacerbated by the PMPRB's move toward self-assessment through its Guidelines Monitoring and Evaluation Plan (GMEP).

In its own words, the PMPRB notes that through the GMEP, it will seek to monitor and evaluate trends in the pharmaceutical market that may be impacting patentees, as well as the consumers, patients, and payers that it is mandated to protect. We believe it is critical to the best interests of Canadians to have an independent body monitor and evaluate the impact of the PMPRB's regulatory and guideline changes.

Moreover, staff should not be given such a wide berth on drug pricing without additional clarity, accountability and transparency. To do so leaves a significant number of decisions open to the "opinion of PMPRB staff" and thereby grants them powers that are not clearly outlined or limited. Recent court decisions in multiple jurisdictions in Canada have limited the powers of the PMPRB. If left unchecked, increasing the power that staff has on drug pricing will lead to greater market uncertainty, which may prevent new medicines from coming to Canada.

To improve accountability and transparency, the PMPRB must establish formal mechanisms for meaningfully and continuously engaging stakeholders, including patient representatives, in its decision-making and evaluation processes.

Furthermore, the guidelines must provide greater clarity on staff powers and limitations as they relate to drug pricing and triggering investigations. The guidelines must also include greater accountability and transparency mechanisms with respect to staff powers and limitations.

High-Cost vs High Value

The guidelines as written speak only to price, not to the value or cost/benefit ratio of a medication. To consider only price tells only part of the picture. This is especially important when it comes to drugs for rare diseases, which may come at a high cost while also providing great therapeutic value.

The guidelines must put value to patients first – not cost.