Submission to the Patented Medicine Prices Review Board
Consultation on the Guideline Monitoring and Evaluation Plan

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Introduction

Cystic Fibrosis Canada has dramatically changed the cystic fibrosis story. We have advanced research and care that has more than doubled 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, and donors. We work together to change lives through treatments, research, information, and support. Despite our remarkable progress together, we are not yet done. Not when half of the people with cystic fibrosis who died over the past three years were younger than 34. Not when a child born with cystic fibrosis today still has only a 50% chance of living to 54. We will keep pushing, keep going further until all people with cystic fibrosis can and do experience everything life has to offer — and enjoy everything life has to offer.

We work closely with our patient community to advocate to improve their health and well-being. In 2020, Cystic Fibrosis Canada's National Advocacy Network consisted of 250 well-trained advocates and a basket of tools to help them in their efforts. We have been able to help the cystic fibrosis community by amplifying their voices through coordinated efforts that have addressed both national and regional priorities.

Cystic Fibrosis Canada's contributions have led to significant improvements in care and quality of life for people living with cystic fibrosis. As a result, Canada has one of the highest median ages of survival in the world.

About Cystic Fibrosis

Cystic fibrosis is the most common fatal genetic disease affecting children and young adults in Canada. There is no cure. Cystic fibrosis causes various effects on the body, but mainly affects the digestive system and lungs. The degree of cystic fibrosis severity differs from person to person; however, the persistence and ongoing infection in the lungs, with progressive loss of lung function will eventually lead to death in most people with cystic fibrosis. Respiratory failure causes eighty-five percent of cystic fibrosis fatalities.

Patients may suffer frequent pulmonary exacerbations (PEx) requiring weeks of hospitalization and I.V. antibiotics. PEx cause rapid decline of lung function and more rapid disease progression and are associated with a greater risk of death (Stanford, G. E., Dave, K. & Simmonds, N. J., 2021)[1]. Other consequences of having cystic fibrosis include malnutrition and very low BMI, and cystic fibrosis-related comorbidities like cystic fibrosis-related diabetes (CFRD) and cystic fibrosis-related liver disease.

Cystic fibrosis is a complex disease caused by mutations in the gene for the Cystic Fibrosis Trans-membrane Conductance Regulator (CFTR). There are over 2,090 known mutations. Cystic fibrosis has a tremendous impact on the people who live with it, their loved ones, and on society. Every week in Canada, two people are diagnosed with cystic fibrosis, one of them through newborn screening. Every week in Canada, one person with cystic fibrosis will die.

Thanks to significant progress in treatment and care, the majority of children with cystic fibrosis will reach adulthood. The estimated median survival of Canadians with cystic fibrosis in 2019 was 54.3 years of age.[2] Half of the Canadians who died from cystic fibrosis in 2019 were under 42 years, compared to 33 years in 2018 and 28 years in 2000.

[1] Stanford, G. E., Dave, K. & Simmonds, N. J. Pulmonary Exacerbations in Adults With Cystic Fibrosis: A Grown-up Issue in a Changing Cystic Fibrosis Landscape. *Chest* **159,** 93–102 (2021).

[2] Canadian Cystic Fibrosis Registry 2019 Data Report, https://www.cysticfibrosis.ca/registry/2019AnnualDataReport.pdf

Information Sources

This submission follows input Cystic Fibrosis Canada provided to the PMPRB in August and February 2020 on draft Guidelines, as well as on proposed reforms, provided in February 2018 and June 2017, and input in October 2016 regarding Health Canada's PMPRB Guidelines Modernization Discussion Paper. In addition, Cystic Fibrosis Canada has had several communications with the PMPRB. We have provided correspondence and participated in the PMPRB's stakeholder briefing sessions. This submission draws on our recommendations from all of these sources, as well as others.

Background

The PMPRB is proposing to measure the impact of its regulatory and guidelines changes through its Guidelines Monitoring and Evaluation Plan (GMEP), which will focus on measuring impact in four areas: price, access, pharmaceutical ecosystem, and processes.

Cystic Fibrosis Canada supports lower drug prices, but feels that the proposed changes to the PMPRB guidelines go too far, too fast and will compromise access to life-changing and life-saving medicines for CF. We expressed these concerns in our past submissions and feel that our concerns went unheard and were not valued.

Throughout these communications and consultations, we learned that feeling unheard and under-valued is a common thread among many patients and patient groups when it comes to engaging with, and being consulted by, the Patented Medicines Prices Review Board.

Patients and patient groups are primary stakeholders in healthcare, particularly with respect to access to medicines. As primary stakeholders, we worked hard to demonstrate the opportunities and challenges we saw with respect to the implementation of the PMPRB changes. Throughout the consultation periods, we stressed, again and again, that we agreed with the goal of lowering drug prices and the changes to the comparator countries, but we had concerns that the additional pharmacoeconomic elements being proposed would negatively impact access. The PMPRB told us, again and again, that our fears were unfounded, but little to no changes were made to the guidelines to address our concerns.

More recently we learned that the PMPRB deemed the cystic fibrosis community to be an opponent and it was suggested that our community was spreading disinformation through organized public relation campaigns.

Cystic Fibrosis Canada, its partners and community members are stakeholders of the PMPRB, a federal drug regulatory agency in Canada, whose guidelines have been under revision for several years. Cystic Fibrosis Canada has at times been vocal about the parts of the PMPRB's new guidelines that we do not support. Our job is to speak up when we believe there are barriers that limit access to new innovative, life-changing medicines for our community. We have provided our feedback to the PMPRB and outlined the parts of the guideline changes we support and areas for improvement. The fact that we support some parts of the guideline changes seems to continually fall on deaf ears which illustrates to us that we are not being listened to with any real intent. Rather, the PMPRB continues to marginalise and invalidate the voices of the CF community. This is a grave concern to us as it is indicative of conduct that does not comply with the standards Canadians should expect from a regulatory agency.

The PMPRB's approach to evaluation has been to primarily focus on the use of administrative, commercial, and internal data sources in its decision-making. Given the PMPRB's distrust of the patient community, the lack of integration of the patient community in the proposed GMEP plan is not a

surprise. For instance, the PMPRB appears to have given no consideration to the rich data available through organizations like Cystic Fibrosis Canada in tracking the impact of the Guidelines.

Recommendations

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.
Given its record on patient engagement and consultation, Cystic Fibrosis Canada has serious concerns about the approach and conduct of the PMPRB and does not believe it is the best interest of Canadians to have this body monitor and evaluate the impact of its own regulatory and guideline changes.

In our June 2020 submission to the PMPRB, we called for an independent, third-party evaluator to develop and lead the GMEP process for transparency and impartiality purposes. Specifically, we recommend that the Government of Canada:

- Implement an impartial, independent third party, such as CIHI, to evaluate the impact of the revised economic criteria on the availability of medicines in Canada specifically to inform any decision on whether, when and how to implement the use of the new economic criteria for innovative, precision, and other high-cost medicines. Until this is completed, and the value of these measures is demonstrated, no such measures should be adopted.

To our knowledge, no formal mechanism for meaningful and continuous engagement of patient representatives has been established since our first mention of this recommendation in our February 2020 submission.

There are examples of meaningful patient engagement in drug access decision-making. There is no reason why the PMPRB cannot involve patients in meaningful ways when so many other jurisdictions have. Given the significant impact of the PMPRB's work on patient's health, it is essential they do so.

The GMEP also outlines that well-placed, thoughtful Health Technology Assessments (HTA) can play an important role in helping to determine the costs and benefits of medicines and health technologies. Canada's HTA processes, as currently structured, do not serve the rare disease community well. Quebec is making progress here, but the rest of Canada needs to catch up.

Ensuring fair and equitable access to new rare disease and precision medicines requires a new way of thinking and a new way of evaluating innovative medicines. To that end, Cystic Fibrosis Canada recommends that:

- The PMPRB immediately establish a formal mechanism for meaningful and continuous engagement of patient representatives in its drug evaluation and decision-making process to ensure patient voice, choice, and representation and so that all medications are assessed not only on their cost-per-QALY, but on their true holistic value to patients, families, and society.

In closing, given the PMPRB's demonstrated bias against select patient communities and the cystic fibrosis patient community in particular, Cystic Fibrosis Canada is highly skeptical about the ability of the PMPRB to conduct an impartial monitoring and evaluation process of its own guidelines. We repeat,

with force, our recommendation and call for an independent, third-party evaluator to develop and lead the GMEP process for transparency and impartiality purposes. As noted recently by Killam Prize holder Dr. Donald J. Savoie, OC ONB FRSC, Canadian public administration, and regional economic development scholar:

> *"...from a machinery of government and given the requirements tied to a quasi-judicial agency, it will be important to ensure a non-bias perspective in PMPRB's work. If it is unable to both retain and promote this capacity in its work, its decisions will be challenged by one side or the other. This in turn will force stakeholders to spend time and resources to monitor the agency's work and subject PMPRB to continuing challenges. It would also violate a basic requirement of public administration – quasi-judicial agencies should go about their work free of any bias, political or otherwise. - Donald J. Savoie*

Should you have any questions about this submission, please contact:

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