Submission to the Patented Medicines Prices Review Board
Price Review Approach During the Interim Period following publication of Amendments to the Patented Medicines Regulations

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Introduction

Cystic Fibrosis Canada is pleased to provide feedback to the Patented Medicines Prices Review Board (PMPRB) on its proposed approach for conducting interim price reviews while the PMPRB consults its stakeholders on new 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 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 for the 4,332 Canadian children and adults living with cystic fibrosis through treatments, research, information and support. Despite our remarkable progress together, we are not yet done. Not when half of the Canadians with cystic fibrosis who died in the past five years were under the age of 37. 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.

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 protects timely access by Canadians to new medicines, especially precision medicines.

We believe the PMPRB can achieve reasonable pharmaceutical price reductions in ways that do not impede timely access.

Implementation of New Comparator Countries

From the outset of the proposed changes to the to its guidelines in 2018, the PMPRB sought to implement a change in comparator countries, which it estimated would result in savings of 20% of the cost of patented medicines year over year.

Cystic Fibrosis Canada supported this change then, and we support it now, as do many other stakeholders. In our submissions and communications to the PMPRB, Cystic Fibrosis Canada repeatedly called on the PMPRB to immediately implement the change in comparator countries so Canada could start reducing drug expenditures while the rest of the proposed changes were

being challenged. We noted that approximately 20% in savings could have been realized if the change in comparator countries was implemented in 2018, when the regulatory change process began.

In response to the new PMPRB regulatory changes that took effect on July 1, 2022, the Parliamentary Budget Officer (PBO) undertook a study on the potential long-term impact that changing comparator countries could have on expenditures by Canadian consumers. This study was initiated by the PBO and was not done at the request of an external body. The PBO stated that they undertook the analysis due to Parliament's interest in the matter.

On June 14, 2022, the PBO released its report: *Canadian Patented Drug Prices: Gauging the Change in Reference Countries*. Using 2018 as the representative year, the PBO found that the PMPRB11 could have reduced Canadian expenditures by approximately 19% representing a $2.8 billion reduction. More specifically, if more frequent reassessments are used, the PBO concludes that the proposed change may, over the long-term, lower expenditures on patented drugs reaching 19% if reassessment of prices occurs more frequently. The report outlines that almost 66% of the 19% reductions could be attained from attaining median prices of the PMPRB7 through more frequent reviews.

Cystic Fibrosis Canada is disappointed that the PMPRB did not implement the change of comparator countries sooner, particularly given that the goal of the regulatory and guideline changes was to reduce drug prices in Canada and that so many stakeholders, including industry, seemed to support this change. That this led to $1-$3B in annual savings being left on the table raises questions about the PMPRB's price reform objectives and its ability to implement cost-savings strategies in timely and meaningful ways.

Proposed Interim Guidance

Cystic Fibrosis Canada appreciates that the PMPRB requires regulations and guidelines to operate, and that it is limited by the Superior Court of Quebec's February 18, 2022 ruling that the proposed economic factors and disclosure of confidential rebates were unconstitutional. Cystic Fibrosis Canada was an intervenor in this case, as the uncertainty that the 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 noted that it was hesitant 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. Almost 40 countries provided access to Trikafta before Canada.

When the drug did finally make its way to Canada for those 12 years of age and older, it took just 11 months to go from Health Canada application to being covered by all of Canada's public drug plans, a pace and reach rarely seen in our country. The efficacy of this medicine is so great that, in a rare move, the Canadian Agency for Drugs and Technologies in Health (CADTH)

changed its draft-recommendation to improve access to the drug and recently issued a new recommendation for our public drug programs to expand access to anyone six years of age or older, removing an upper lung function initiation criterion that could have prevented approximately 27% of those 12 years of age and older from accessing the drug. At least 70% of children aged 6-11 years old could have been left behind if this restrictive criterion was applied to them.

Moreover, the pan-Canadian Pharmaceutical Alliance (pCPA) worked behind the scenes to negotiate a price with the manufacturer that clearly all jurisdictions were comfortable with. This allowed many jurisdictions to move quickly to reimburse almost immediately after CADTH's recommendations and shows that those who actually have jurisdiction to regulate drugs – the provinces and territories – are quite capable of driving drug costs down.

Trikafta is changing the trajectory of cystic fibrosis in Canada. More importantly, it is changing people's lives. If the guidelines were not delayed and ultimately stripped of unconstitutional overreach, we could still be fighting for this drug, more people would get sicker, and more would have died waiting.

Given the impact that the PMPRB's overreach had on Canadians with cystic fibrosis, we are gravely concerned with the PMPRB's decision to not conduct a price review of any new patented medicines until new guidelines are implemented. We know from experience that this could take several years. Manufacturers are less likely to launch innovative products in markets of uncertainty, and this costs people their health and, for some, their lives.

While it didn't focus on access specifically, the Parliamentary Budget Officer's report also raised this issue, noting that (pg. 11):

> *Another issue that remains relevant is linked to reasons for Canada's timely access to some drugs. If current Canadian prices for those drugs was the main reason for that access, then it would likely be curtailed (e.g., Spicer and Grootendorst, 2020; Palmer, 2019). This means that Canada would only have access to some new drugs as they became more widely available internationally.*

Cystic Fibrosis Canada calls on the PMRPB to conduct reviews under the current regulatory and guideline framework until new guidelines are adopted to create more certainty in the market. Years of uncertainty cost Canadians their health and their lives.