Canadian
Cystic Fibrosis
Treatment Society

November 6, 2020

Standing Committee on Health (HESA)
Sixth Floor, 131 Queen Street
House of Commons
Ottawa ON K1A 0A6

Subject: HESA's Study on the Patented Medicine Prices Review Board Reforms

Dear Members of the House of Commons Standing Committee on Health,

On behalf of the Canadian Cystic Fibrosis Treatment Society (CCFTS) thank you for holding this study, which is the first substantive parliamentary review of a regulatory system that is killing Canadians by blocking access to life-saving medicines. This is an issue with grave and imminent implications particularly for Canada's cystic fibrosis (CF) community and me, personally, as someone living with CF.

CCFTS is a leading patient advocacy organization aimed at supporting patients with CF. Whether it's advocating for medications, therapies, medical devices or public and private insurance, we do what it takes to ensure patients with cystic fibrosis are able to live longer and better lives.

In this context, CCFTS is extremely concerned about the potential consequences of the proposed PMPRB changes on patient access to needed medicines. There is every indication that the sheer scale and magnitude of the reforms, and the uncertainty they create, effectively prevent pharmaceutical companies from introducing new, promising, and often life-saving medicines to Canada.

This was most recently highlighted by our country's inability to access Trikafta – a ground-breaking new CFTR gene modulator therapy that every person with CF has dreamt of since being diagnosed with the deadly respiratory disease. Trikafta already available in the over twenty other countries has the potential to treat 90 percent of the population with CF, and therefore save and improve thousands of Canadian lives. Trikafta is not yet available in Canada, except via the manufacturer's compassionate use program and the company's willingness to provide access through the special access program ("SAP"). The manufacturer has cited the PMPRB changes as the primary reason are not launching the treatment in Canada.[1]

It is worth mentioning that CFTR modulators were invented by a Canadian researcher (Fred van Goor) and are the direct result of the 1989 discovery of the CF gene in Canada by Canadian researchers, with funds raised over years through a dedicated rare disease community in the hopes of bringing lifesaving changes to Canadian CF patients. It is an ultimate irony that access to drugs resulting from Canadian researchers and discoveries is now unavailable here because of PMPRB red tape. There is a significant risk that Canadian CF patients and the Canadian CF community lose confidence that the research they support will actually benefit Canadian patients. What hope do we have in this context that Canadian researchers will be part of finding an eventual cure for CF?

[1] https://globalnews.ca/news/7412136/patients-health-organizations-breakthrough-medical-treatments-patented-medicine/

While we support measures that improve access and affordability of medicines for patients, a balance is clearly needed. Medicine prices should be affordable and sustainable for patients and payers, but they should never become so low or unpredictable that companies are discouraged from launching new therapies in Canada. Unfortunately, this pendulum has swung too far in favour of obtaining the lowest possible medicine prices, with little regard for how the changes will affect our health system and patients.

The very real practical effect of the PMPRB changes is that by mandating extreme price cuts and requiring the use of QALY thresholds (cost-effectiveness evaluations) to set maximum drug prices, the regulations practically guarantee that at least some expensive rare disease drugs will not be made available to Canadians. When drug prices are fixed using systems designed to evaluate the most common drugs, drugs for rare diseases are disadvantaged. By removing the flexibility to negotiate prices, this reduces the ability of manufacturers and payers to arrive at mutually acceptable commercial terms, which hinders access. In this context, it is important to remember that pharmaceutical medicines are not products like any other; regulating prices does not only affect industry, it affects patients' lives.

From the outset of the PMPRB reform process, CCFTS has directly engaged with government officials and collaborated with other patient organizations to raise awareness of these issues. We believe the PMPRB changes were implemented too fast and with insufficient consideration of the feedback received from concerned stakeholders throughout the many stakeholder consultations held by Health Canada and the PMPRB.

This is one of the reasons why CCFTS became one of the primary intervenors in the ongoing constitutional challenge of the PMPRB changes in the Superior Court of Quebec. The issue at the heart of this legal challenge is whether the changes to the *Patented Medicines Regulations* are *ultra vires*, i.e. outside the scope, of the federal government's jurisdiction and impede on provincial authority to regulate medicine prices. It is important to note that several of the pharmaceutical companies leading the challenge affirmed that the regulations will force them to delay entry of new innovative medicines onto the Canadian market and might even result in some drugs not being offered for sale in Canada at all. This has also been confirmed by survey data published earlier this year.[2]

Moving forward, we strongly recommend that the PMPRB regulations be revised to ensure a better balance between medicine affordability and access, so that one does not come at the expense of the other. For this reason, we strongly encourage the removal of the proposed economic factors and the concept of QALY thresholds from the proposed regulations. This will help reduce the uncertainty associated with the current system, and provide a breath of hope for Canadian CF patients, who are nervously awaiting the outcome of these critical discussions.

In addition to submitting this input, the CCFTS has applied to appear before your committee in the context of this study, where we would be pleased to expand on our positions regarding the regulations, their impacts on Canadian patients, as well as the issues raised in our contributions to the constitutional challenge.

Sincerely,

Chris Macleod
Founder, CF Treatment Society

[2] https://lifesciencesontario.ca/wp-content/uploads/2020/02/Research-Etc.-PMPRB-Survey-02-03-20.pdf

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