Death by Regulation: Stop Policies Putting Canadians with Cystic Fibrosis at Risk

Background

In May of 2017, Health Canada proposed changes to the Patented Medicine Prices Review Board (PMPRB) regulations. These changes include new factors to determine whether a medicine is being sold at an "excessive" price. Since these changes were first proposed, the patient community has repeatedly raised concerns about the negative impact on access to new medicines.

The new regulations require patented drug manufacturers to reduce their prices by 45% - 75%, by some estimates, making Canada an outlier among OECD countries and a much less attractive market in which to launch innovative therapies. Therapies like new precision medicines that can alter the course of devastating diseases like cystic fibrosis.

Cystic fibrosis is a fatal genetic disease that mainly affects the digestive system and lungs with progressive loss of lung function. Exacerbations of pulmonary symptoms frequently lead to hospitalizations. Eighty-five percent of people with cystic fibrosis die from respiratory failure. The COVID-19 pandemic puts people with cystic fibrosis at even greater risk of hospitalizations.

The impact the new regulatory changes have had is chilling. From November 1, 2019 to February 29, 2020 Health Canada registration of new clinical trials decreased by 60% compared with the average of four years prior. Moreover, in 2019 the approval of new drugs in Canada either prior to or within a year of their approval in the United States fell by more than two-thirds from previous levels (2015-2018), from 49% to 15% (1).

The PMPRB and the federal government have repeatedly assured patient groups that access to new medicines will not be impacted by these changes. We now know that access to new drugs for Canadians with cystic fibrosis has not only been delayed, but may be denied altogether.

Trikafta: A Game-Changing Drug for CF

Trikafta is a new life-changing drug for up to 90% of people with cystic fibrosis. Access to the drug has been delayed and is at risk of not coming to Canada at all. When asked why the drug has not been introduced in Canada, the manufacturer, Vertex Pharmaceuticals, raised concerns about the regulatory changes Canada is poised to adopt.

This drug was approved 6 months faster than expected in the United States, is fast-tracked for review in the UK and is under review in Europe. Ireland and Switzerland have agreed to cover it after health technology assessment approval. Trikafta represents the single biggest advancement in treating cystic fibrosis in the history of the disease (identified as a disease in 1938) and has been proven to significantly improve health outcomes for 90% of CF patients. *The Washington Post* named it number one among nineteen good things that happened in 2019.

But Canadians don't have access to it. And people are dying while waiting ~~for access to this drug~~.

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[1] https://business.financialpost.com/opinion/price-controls-imposed-by-clueless-politicians-keep-new-life-saving-drugs-out March 5, 2020

[2] https://www.washingtonpost.com/opinions/19-good-things-that-happened-in-2019/2019/12/17/719f50d6-2025-11ea-86f3-3b5019d451db_story.html March 5, 2020

Canadians with CF Need Access Now

Helping people with cystic fibrosis be healthier so they can better fight off infections, avoid pulmonary exacerbations and stay out of hospital is of particular importance now, at a time when hospital resources are dwindling in the wake of COVID-19.

Cystic fibrosis patients, all Canadian patients, deserve a system that enables access to the best new life-changing medicines available. Not one that unfairly and unnecessarily delays or denies access to these medicines. Vertex has made its sixth offer to the pCPA and has yet to receive constructive feedback from the provinces on the first five proposals. The company has not received feedback on the first five offers.

Cystic Fibrosis Canada supports the policy goal of lowering drug prices in Canada. We expect pharmaceutical manufacturers to bring their products to market at a reasonable price. But we also expect the government to ensure that the regulatory environment in Canada does not unnecessarily limit our ability to access new life-saving therapies.

This situation should be a wake-up call for all Canadians and our elected members. Cystic fibrosis patients may be among the first to feel the impact of these proposed changes, but we won't be the only ones. Other pharmaceutical manufacturers have begun to delay introducing new medicines to Canada because of the proposed changes. Canadians expect better. Canadians deserve better.

Recent discussions with the leadership of the PMPRB indicate that there is a desire to 'get this right' and an intent to find a way to lower drug costs while also ensuring access to innovative, life-changing medications. This is encouraging. But we know that 'getting this right' will take some time. And we are running out of time before these changes are set to come into effect.

We are facing a respiratory virus crisis and need all the available tools to stay healthy and out of hospital. Thousands of Canadians living with CF are running out of time. On their behalf, we call on the federal government to:

- **Stop the implementation of the proposed changes to the PMPRB and reconsider the changes so that access to new drugs like Trikafta will not be delayed or denied.**

In a humanitarian effort to help Canadians with cystic fibrosis through this health crisis and preserve badly needed in-patient resources, we call on governments, Health Canada, the PMPRB, CADTH, and the pCPA to:

- **Remove the barriers and fast track access to Trikafta to get it to those who need it now.** Lives of thousands of Canadians are literally depending on you.

And we are calling on Premiers and provincial health ministers to:

- **Tell the federal government to stop implementation of the proposed changes to the PMPRB and reconsider the changes so that access to new drugs like Trikafta will not be delayed or denied.**

- **Immediately negotiate with the manufacturer on the sixth proposal they have made to the provinces for access to disease-modifying drugs already approved by Health Canada for cystic fibrosis, so that people with CF who could benefit from other disease-modifying drugs can access these medicines now.**