Canada, what are you waiting for? Getting Trikafta to those who need it now.

Background

A new study shows the incredible positive impact that the breakthrough cystic fibrosis drug Trikafta could have on Canadians living with CF, but only if it becomes available in the near futureThis study examines the potential health outcomes for the Canadian cystic fibrosis (CF) population by the year 2030 and illustrates the impact by assessing three scenarios: getting access to the drug in 2021, getting access to the drug in 2025 and not getting access at all.

The study shows that early introduction of Trikafta would result in life-changing health improvements by 2030 comparative to no drug available, including:

- The estimated median age of survival for a child born with CF would increase by 9.2 years
- 60% fewer people living with severe lung disease
- 18% increase in people with mild lung disease
- 19% fewer hospitalizations or home intravenous courses for chest infections
- 15% fewer deaths, and
- A reduction in the number of transplants that are required for severe lung disease.

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.

Trikafta: A Game-Changing Drug for CF

Trikafta is a new life-changing drug for up to 90% of people with cystic fibrosis. It represents the single biggest advancement in treating cystic fibrosis in the history of the disease and has been proven to significantly improve health outcomes. *The Washington Post* named it number one of nineteen good things that happened in 2019[1]. Trikafta was approved six months faster than expected in the United States and was fast-tracked for approval in the UK and Europe and will be available to patients soon.

But Canadians can't get it. And people are dying while waiting. Access to the drug has been delayed and it is at risk of not coming to Canada at all. When asked why the drug has not been brought to Canada, the manufacturer, Vertex Pharmaceuticals, raised concerns about the regulatory changes Canada is poised to adopt.

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

PMPRB Guidelines Blocking Access to Life-Changing Drug
Changes to the Patented Medicine Prices Review Board (PMPRB) regulations have introduced new factors to determine whether a medicine is sold at an "excessive" price. By some estimates these changes will require drug manufacturers to reduce their prices by 45% - 75%, making Canada an outlier among OECD countries and a less attractive market in which to launch innovative therapies.

The impact these changes have had on access to new medicines 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%[2].

Thanks to advocacy efforts of the cystic fibrosis community, the original implementation date of July 1, 2020 was delayed until January 1, 2021 and a new set of guidelines were issued for consultation. Cystic Fibrosis Canada's submission expressed our support for lower drug prices as well as our belief that the changes go too far and will limit access to innovative therapies, and we provided our recommendations for a path forward.

The New PMPRB Guidelines: Our Recommendations
Recent changes to the new guidelines are encouraging but additional changes should be made to remove barriers to an immediate submission to Health Canada by the manufacturer, and to remove access barriers for future medicines. We continue to apply pressure to the manufacturer to immediately submit the drug for approval, while encouraging all relevant bodies to align drug review and approval processes.

We are calling on the federal government to fix these parts of the guidelines**. We ask that you speak to the caucus about supporting these recommendations and also to the Minister of Health to implore her to implement the following recommendations now:**

- Take a phased approach to implementing the PMPRB regulations by immediately adopting the changes to the comparator countries. This will bring about an average 20% price reduction on drugs.

- Remove barriers in the PMPRB guidelines by amending the Health Canada approval deadline for "Gap Medicines" to an application deadline of January 1, 2021. This small change could help secure a Health Canada application for Trikafta. Cystic Fibrosis Canada will continue to pressure the manufacturer to apply.

- Appoint an independent third party to evaluate the impact of the revised economic criteria to be used in the PMPRB analysis and ensure that they do not raise unintended barriers to access for Canadians.

- Establish formal mechanisms for engaging patient representatives in PMPRB decision-making and processes to ensure patient voice, choice and representation.

No Time to Wait: We Need Access Now
With this new evidence in hand, **Cystic Fibrosis Canada implores all levels of government to work with all relevant bodies to expedite Trikafta's immediate entry into Canada and public access nationwide.**

Canadians with cystic fibrosis don't have time to wait.

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2 https://financialpost.com/opinion/price-controls-imposed-by-clueless-politicians-keep-new-life-saving-drugs-out August 21, 2020