Getting Trikafta to those who need it now.

Background
Following a year of tireless advocacy from the Canadian cystic fibrosis (CF) community Cystic Fibrosis Canada has learned that Vertex Pharmaceuticals has committed to bringing new CF medicines to Canada. We understand this to mean that an application to bring Trikafta to Canada is underway, however this can only be confirmed when Health Canada publishes the information on their website.

Throughout the past year, Cystic Fibrosis Canada has worked with our community, our partners, and our clinicians to call on the government and manufacturer to bring this innovative treatment to Canada. Countless Members of Parliament from all parties supported us and championed access to this game-changing therapy, and in a recent meeting with Cystic Fibrosis Canada, the Federal Minister of Health committed to fast track Trikafta through Health Canada for approval and for pricing review.

This is a milestone we fought hard for as a community, one that should have come sooner, but is here now thanks to the relentless will and determination of the cystic fibrosis community. Our fight,however, is not over: while a Health Canada application is in the works, there is much work to do to ensure that Trikafta gets to Canadians with CF who need it fast and fairly.

Why Trikafta?
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 fast-tracked for access by the U.S Federal Drug Agency (FDA) and the European Medicines Agency (EMA). Trikafta has received regulatory approval in 32 countries, 27 within the EMA centralized regulatory approval in addition to receiving approval in the United States, United Kingdom, Norway, Iceland and Liechtenstein. Trikafta has received public reimbursement in the United States, United Kingdom, Ireland, Austria, Denmark, Germany, and Slovenia.

Canadian research[2] released in August 2020 demonstrates that if Trikafta was brought to Canada quickly, as it has been in the United States, the United Kingdom and parts of Europe already, it could result in extraordinary health benefits by 2030, including 15% fewer deaths, 60% fewer people living with severe lung disease and an increased estimated median age of survival for a child born with cystic fibrosis of 9.2 years.

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1 https://www.washingtonpost.com/opinions/19-good-things-that-happened-in-2019/2019/12/17/719f50d6-2025-11ea-86f3-3b5019d451db_story.html
August 21, 2020

2 https://www.cysticfibrosis.ca/news/new-research-shows-15-reduction-in-cystic-fibrosis-deaths-by-2030-if-trikafta-is-made-available-now?p=1
November 11, 2020.

Where is it at in the approval process?
To our knowledge, the manufacturer is submitting Trikafta to Health Canada, however we don't yet know the details of their application. There are opportunities for rapid reviews. For example, the Health Canada review could be significantly quicker if the manufacturer requests and is granted a priority review, and if the manufacturer consents to an 'aligned review', several government review bodies will work in parallel, at the same time, instead of sequentially.

Specifically, Health Technology Assessment done by the Canadian Agency for Drugs and Technologies in Health (CADTH) and the Institut national d'excellence en santé et en services sociaux (INESSS) would align with the Health Canada review, as would the Patented Medicine Prices Review Board's (PMPRB) pricing review. All in, an aligned review can decrease the time, taking 12 months or less.

If an aligned review is not granted it could take up to two years or more to get the drug through the drug review and reimbursement system, and CF can't wait. Trikafta comes too late for too many in our community. We need public access to Trikafta now. Even 12 months is too long.

In addition to ensuring quick access to Trikafta, we must also ensure that all who can benefit from it can get it. For example, in the U.S.A. the drug is indicated for those who are 12 years of age and older[3] who have at least one F508del mutation, regardless of their second mutation. This makes sense both morally and scientifically. A broad indication allows clinicians to prescribe the medicine to any patient who can benefit from it. A broad indication may also help secure broader coverage, which will require further engagement with our public and private payers to secure.

Recommendations
We are encouraged that, in a meeting with Cystic Fibrosis Canada, the federal Minister of Health committed to fast-track the drug through Health Canada for approval and pricing review by the PMPRB. We need to also ensure that the Health Canada indication is broad and encapsulates all who can benefit from Trikafta, as noted above.

To that end, we are calling on **the Minister of Health to:**

- Provide clarity from Health Canada regarding what kind of review Trikafta will have and how long it will take;
- Reaffirm the Minister's commitment to fast track Trikafta;
- Commit to ensuring that the Health Canada indications represent the broadest population possible, like the Federal Drug Agency (FDA) has done;
- Work with our federal and provincial public drug programs through the pan-Canadian Pharmaceutical Alliance (pCPA) to:
    - Negotiate Trikafta as part of the current price negotiations for Orkambi and Kalydeco;
    - Expedite these negotiations; and to,
    - Provide broad access to Trikafta immediately thereafter.

[3] The safety and effectiveness of Trikafta in patients with cystic fibrosis younger than 12 years of age have not been established, but clinical trials are underway in 6-11 year olds.

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