BRIEFING NOTE: Access to CF Medicines

Background

- Timely access to medications is crucial to maintaining – and in some instances improving – the health and well-being of Canadians with cystic fibrosis (CF). There are now new highly effective medicines that treat the basic defect of CF, rather than just the symptoms.

- First-in-kind, genetically tailored disease-modifying therapies such as Kalydeco, Orkambi and Symdeko are precision medicines approved by Health Canada and have been shown to work well in some people with CF. There are over 2000 mutations associated with CF.

- Currently, only some Canadians that could potentially benefit from these drugs can access them, primarily through private insurance, through the manufacturer's compassionate care program or on a restrictive limited access case by case basis such as in Alberta, Ontario and Saskatchewan or through its patient d'exception program in Québec.

- All Canadian provinces provide coverage for Kalydeco for those with the G511D mutation on an exceptional, case-by-case basis. In 2019, the pCPA and the manufacturer completed price negotiations for additional Kalydeco mutations, which are not covered by all public drug programs.

- With respect to Orkambi, in 2019, Alberta along with Ontario and Saskatchewan put criteria in place for access to Orkambi. Access may be considered for pediatric patients between 6-17 years old who have experienced at least a 20 percent relative decrease in ppFEV1 in the last six months sustained for at least six weeks despite appropriate treatment.

- Although this was a step forward, we were nonetheless disappointed that these criteria are not more inclusive. Cystic fibrosis is no longer a childhood disease: 64 percent of Canadians living with CF are adults, and 16 percent are adults over 40.

- In June 2020, the pan-Canadian Pharmaceutical Alliance (pCPA) and Vertex Pharmaceuticals agreed to start negotiations on the drugs Orkambi and Kalydeco. The goal of these negotiations is to come to an agreement on price, which Cystic Fibrosis Canada hopes will lead to improved public coverage of Kalydeco and Orkambi, and which may pave the way for access to Trikafata, a new, game changing treatment for cystic fibrosis.

- Symdeko was approved by Health Canada in June of 2018 and has not yet been reviewed by the Canadian Agency for Drugs and Technologies in Health (CADTH). The drug is available through some private drug programs but the manufacturer has not yet submitted a dossier to CADTH. Until the manufacturer does, our public drug programs won't consider providing access.

Trikafta: The Biggest Advancement in Treating CF

- Trikafta is a new life-changing drug that can treat up to 90% of people with cystic fibrosis. The Washington Post named it number one among nineteen good things that happened in 2019.

- 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. Cystic fibrosis was identified as a disease in 1938. The drug does not yet have Health Canada approval and therefore cannot be marketed in Canada until it is approved.

- The manufacturer has cited regulatory changes to the Patented Medicines Prices Review Board (PMPRB) as the reason the drug has not been submitted to Health Canada for approval. Thanks to advocacy efforts of the cystic fibrosis community and others, implementation of these changes were delayed until January 1, 2021 and a revised set of guidelines were issued. Cystic Fibrosis Canada is reviewing the new guidelines to determine if they will help or hinder access.

Impact of CF Drugs and the Canadian System for Precision Medicines

- Kalydeco, Orkambi, Symdeko and Trikafta can: i) improve lung function, ii) reduce the rate of pulmonary exacerbations, which can lead to hospitalizations and accelerated lung disease, and iii) improve nutritional status. Exacerbations lead to frequent hospitalizations - hospitalizations interfere with school, and jobs, for both adult patients and the parents of children with CF. Collectively, CF patients spent over 26,500 days in the hospital in 2018. That is 72 years.

- Unlike many other countries, Canada does not have a fast-track process for drugs for rare diseases or precision medicines. This has resulted in limited and delayed access to disease modifying therapies such as Orkambi and Kalydeco. The current system takes years and is duplicative and onerous for precision medicines and drugs for rare diseases.

- In order to market Trikafta in Canada, the manufacturer must apply for and receive Health Canada approval, and have the maximum price for the drug set by the PMPRB. It must then undergo health technology assessment through CADTH and its Quebec counterpart, Institut national d'excellence en santé et en services sociaux (INESSS), and will also need to clear some separate jurisdictional reviews. Only then must the drug go to the pan-Canadian Pharmaceutical Alliance (pCPA).

- These processes are not quick; they take years to complete. Cystic fibrosis is a progressive, fatal disease. Canadians with cystic fibrosis can't wait.

Cystic Fibrosis Canada's Recommendations

- Cystic Fibrosis Canada is calling on the provincial government to:

    - Provide leadership by working in collaboration with other jurisdictions to fast track access to Trikafta. There must be a collaborative effort to ensure a pathway to access comes quickly for those who need it now.

We also call on government to immediately:

- Expedite negotiations at the pCPA and provide improved public access for Kalydeco and Orkambi. There is no time limit on pCPA negotiations. CF is a progressive, fatal disease. People with CF don't have time to wait. They need access to these drugs now.