BRIEFING NOTE: Access to CF Medicines

Background

- Timely access to medications is crucial to maintaining – and in many 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, and which significantly improve the health outcomes and quality of life of people diagnosed with CF.

- There are over 2000 mutations associated with CF. Based on the Canadian Cystic Fibrosis Registry, a baby born with cystic fibrosis in Canada in 2018 has only a 50% chance of living 52 years and will endure significant health-related disabilities. Collectively, CF patients spent over 26,500 days in the hospital in 2018, equivalent to 72 years. There are drugs that can dramatically improve and extend the lives of people with CF, but Canada's drug review and reimbursement system stands in the way.

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

- Currently, only some Canadians who 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 the patient d'exception program in Québec.

- All Canadian provinces provide coverage for Kalydeco for those with a specific mutation (the G511D mutation) on an exceptional, case-by-case basis. In 2019, the pan-Canadian Pharmaceutical Alliance (pCPA) and the manufacturer completed price negotiations for additional Kalydeco mutations, which unfortunately are not still not covered by most public drug programs.

- With respect to Orkambi, in 2019, Alberta along with Ontario and Saskatchewan, put restrictive 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. The Quebec criteria are not public.

- Although this was a small step forward, we are disappointed that these criteria are so restrictive and 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. Providing access to modulators to these adults could help them live healthier and more productive lives.

- In June 2020, the 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 Trikafta, a newer, more impactful?, 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) or Institut national d'excellence en santé et en services sociaux (INESSS) because the manufacturer has not yet submitted it for review

by these bodies. The drug is available through some private drug programs. Until the manufacturer submits Symdeko to CADTH and INESSS our public drug programs won't consider providing access.

Trikafta: The Biggest Advancement in Treating CF

- Trikafta is a new transformational drug that can treat up to 90% of people with cystic fibrosis. 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 published in the Journal of Cystic Fibrosis demonstrates that access to Trikafta in 2021 would result in profound health benefits for Canadians living with cystic fibrosis. By 2030, Trikafta could reduce the number of people living with severe lung disease by 60% and reduce the number of deaths by 15%.

- The findings show a significantly slower progression with an 18% increase in people with mild lung disease and 19% fewer hospitalizations or home intravenous antibiotics for pulmonary infection exacerbations. The estimated median age of survival for a child born with cystic fibrosis would increase by 9.2 years.

Impact of CF Drugs and the Canadian System for Precision Medicines

- Canada's approval and reimbursement system does not typically prioritize precision medicines for rare diseases like cystic fibrosis, which has resulted in limited and delayed access to disease modifying therapies. The current system can take years, and is duplicative and onerous for precision medicines and drugs for rare diseases.

- In order to get Trikafta to those who need it 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 or INESSS and will also need to complete pCPA negotiations and clear some separate jurisdictional reviews both which have no set deadlines. These processes are not quick: they take years to complete.

- To our knowledge, the manufacturer is submitting Trikafta to Health Canada and has been granted Priority Review. While this review shortens Health Canada approval to 180 days, there are no changes to the pace at which the rest of the system moves; a system that is slow. If the manufacturer consents to and is granted an 'aligned review', several government review bodies will work in parallel, at the same time to review the drug in between 8-12 months. Canadians with CF need Trikafta now. It must move through the review process quickly and receive expedited negotiations at the pCPA. CF can't wait.

Cystic Fibrosis Canada's Recommendations

We are calling all provincial ministries of Health to:

- Work with our federal and provincial public drug programs through the pan-Canadian Pharmaceutical Alliance (pCPA) to include Trikafta as part of the current price negotiations for Orkambi and Kalydeco.

- Make a commitment to expedite Trikafta through pCPA negotiations and provide immediate public access to Trikafta and the other CF medicines once negotiations conclude.