Cystic Fibrosis Canada | FURTHER

BRIEFING NOTE: Access to CF Medicines

Background

- Timely access to medications is crucial to maintaining and improving the health and well-being of Canadians with cystic fibrosis (CF). There are 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.

- Collectively, Canadian CF patients spent 25,246 days in hospital in 2019, equivalent to almost 70 years. There are now 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.

- There are over 2000 mutations associated with CF. 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. Trikafta is the newest disease-modifying therapy. It can treat up to 90% of the CF population and is currently under review by Health Canada with a decision due on or before June 23, 2021.

- Currently, only some Canadians who could benefit from these drugs can access them,primarily through private insurance, through the manufacturer's compassionate care program oron 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 G551D mutation) on an exceptional, case-by-case basis. In 2019, the pan-Canadian Pharmaceutical Alliance (pCPA) and the manufacturer completed price negotiations for an additional eight mutations,which disappointingly are still not covered by most public drug programs. This is a form of genetic discrimination.

- In 2019, Alberta along with Ontario and Saskatchewan, put very restrictive criteria in place for access to Orkambi. Access may be considered only 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 sustainedfor 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 not more inclusive. Cystic fibrosis is no longer a childhood disease: 62 percent of Canadians living withCF are adults, and 18 percent are adults over 40. Providing access to modulators to these adults couldhelp them live much healthier and productive lives.

- In June 2020, the pCPA and the manufacturer started price negotiations on the drugs Orkambi and Kalydeco. Cystic FibrosisCanada believes these negotiations should improve public coverage of Kalydeco and Orkambi, and pave the way for access to Trikafta.

- Symdeko has yet to be submitted to CADTH and INESS by the manufacturer. Until CADTH and INESSS review the drug, our public drug programs have told us that they won't consider covering it.

Trikafta: The Biggest Advancement in Treating CF

- Trikafta is a new transformational drug that can treat up to 90% of people with cystic fibrosis. Fast-tracked for access by the U.S Federal Drug Agency (FDA) and the European Medicines Agency (EMA), it has received regulatory approval in 35 countries, 28 within the EMA centralized regulatory approval in addition to receiving approval in the United States, UnitedKingdom, Norway, Iceland, Liechtenstein and Australia. Trikafta has received public reimbursement in the United States, United Kingdom, Ireland, Austria, Denmark, Germany, Slovenia, Luxembourg and Malta.

- A true game-changer, England's National Health Service finalized negotiations with the manufacturer and agreed to fund the drug *before* regulatory approval. This deal secured access to the drug across other UK nations through similar agreements shortly thereafter. Patients in England were accessing Trikafta within a week of its approval by the EMA. We want nothing less for Canadians living with CF.

- Canadian research published in the Journal of Cystic Fibrosis shows that access to Trikafta in 2021 would result in profound health benefits for Canadians living with cystic fibrosis. By 2030, Trikafta is projected to 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

- Following a year of tireless advocacy from the Canadian cystic fibrosis community, Trikafta has been submitted to Health Canada and has been granted a priority review, with a decision due no later than June 23, 2021.

- Through an aligned review process, CADTH and INESSS have officially started their health technology assessment reviews. The CADTH recommendation will be issued to the manufacturer between June 28-June 30, 2021. INESSS has not yet announced their recommendation window, though it usually falls shortly after CADTH's decision.

- Trikafta also needs to complete pCPA negotiations, which have no set deadlines, as well as a review from BC Pharmacare, the start of which has not been announced. CADTH, INESSS, and the pCPA all work on behalf of the provinces. Those decisions must not be allowed to stand between the drug and Canadians with CF who need Trikafta now. CF can't wait.

Cystic Fibrosis Canada's Recommendations

We are asking provincial elected officials to call on their Ministers of Health and Premiers to commit to:

- Funding Trikafta: To fund Trikafta immediately upon Health Canada approval with the broadest prescribing criteria possible.

- Funding Kalydeco and Orkambi: To fund these drugs immediately upon Health Canada approval of Trikafta with the broadest prescribing criteria possible.