Key Messages: Stop the PMPRB Changes and Fast-Track Access to Trikafta

1. Cystic fibrosis (CF) is the most common fatal genetic disease affecting children and young adults in Canada. There is no cure. CF mainly affects the digestive system and lungs with progressive loss of lung function. The majority of people (85%) with cystic fibrosis die from respiratory failure.

2. Exacerbations of pulmonary symptoms frequently lead to hospitalization of CF patients.

3. The COVID-19 pandemic puts people with cystic fibrosis at even greater risk of hospitalizations.

4. Helping people with cystic fibrosis be healthier so they can better fight off infections, avoid pulmonary exacerbations and stay out of hospital is of particular importance now, at a time when hospital resources are dwindling in the wake of COVID-19.

5. A new life-changing medicine called Trikafta can significantly improve health outcomes for 90% of Canadians with cystic fibrosis. This drug targets the root cause of the cystic fibrosis and helps people fight infections and avoid pulmonary exacerbations.

6. This drug was approved 6 months faster than expected in the United States is fast-tracked for review in the UK and under review in Europe. It is not available in Canada nor expected to be available any time soon due to regulatory changes that are creating barriers to drug access in Canada.

7. In a humanitarian effort to help Canadians with cystic fibrosis through this health crisis and preserve badly needed in-patient resources, we call on all governments and the manufacturer to remove the barriers and to fast track access to this drug.

Background:

8. In May of 2017, Health Canada proposed an update to several aspects of the Patented Medicine Prices Review Board (PMPRB) regulations that govern patented medicines. These changes incorporate new factors in the determination of whether a medicine is being or has been sold at an “excessive” price.

9. Since the changes to the Patented Medicines Regulations were first proposed in 2017, the Canadian patient community, including Cystic Fibrosis Canada, have consistently raised concerns that these changes will negatively impact access to new medicines for Canadian patients.

10. The PMPRB, and the federal government, have repeatedly assured Canadian patient groups that access to new medicines will not be impacted by the proposed regulatory changes.

11. Unfortunately, evidence shows that access to new precision medicines like the new drugs for cystic fibrosis has not only been delayed, but may be <u>denied altogether.</u>

12. The new PMPRB regulations will require patented drug manufacturers to significantly reduce their prices. By some estimates, price drops between 45-75% will be required. In addition to

significant price reductions, other changes to the regulations raise new concerns for drug manufacturers operating in a global market.

13. The decreased prices and increased barriers make Canada an outlier among OECD countries and a much less attractive market in which to launch innovative therapies, including precision medicines that can alter the course of devastating diseases like cystic fibrosis.

14. The manufacturer of Trikafta has clearly identified concerns about the regulatory changes the PMPRB is poised to adopt as the barrier stopping access in Canada.

15. Trikafta was granted expedited approval in the United States, is fast tracked for approval in the UK, and is on track for approval in Europe. It represents the single biggest advancement in treating cystic fibrosis in the history of the disease.

16. Cystic fibrosis patients, all Canadian patients, deserve a system that enables access to new life-changing medicines. Not one that unfairly and unnecessarily delays or denies access to these medicines.

17. We want to be clear about our position. Cystic Fibrosis Canada supports the policy goal of lowering drug prices in Canada. We expect pharmaceutical manufacturers to bring their products to market at a reasonable price. But we also expect the government to ensure that the regulatory environment in Canada does not unnecessarily limit our ability to access new life-saving therapies.

18. This situation should be a wake up call for all Canadians. Cystic fibrosis patients may be the first to feel the impact of these proposed changes, but we won't be the only ones. Other pharmaceutical manufacturers have begun to delay introducing new medicines to Canada because of the proposed changes.

19. Canadians should expect better. Canadians deserve better.

20. Recent discussions with the leadership of the PMPRB indicate that there is a desire to 'get this right" and an intent to find a way to lower drug costs while also ensuring access to innovative, life-changing medications. This is encouraging. But we know that 'getting this right' will take some time. And we are rapidly running out of time before these changes come into effect on July 1st.

21. Thousands of Canadians living with CF are running out of time. We are facing a respiratory virus crisis and need all the available tools to stay healthy and out of hospital.

Key asks:

22. On behalf of the thousands of Canadians living with CF, we call on the federal government to immediately stop implementation of the proposed changes to the PMPRB so that access to new drugs like Trikafta will not be delayed or denied. To go ahead with implementation of these changes while many parts of government have come to a halt is unfair and potentially dangerous to Canadian patients waiting for life-changing and life-saving medicines.

23. In addition, we ask that Health Canada, the PMPRB, CADTH, the pCPA , the provinces and Vertex use all available tools to fast-track the review and approval process for Trikafta to get it to those who need it now. The lives of thousands of Canadians are depending on you, especially during this time of extraordinary need.

24. We also need the support of the provinces. We are calling on all premiers to ask that the federal government stop the implementation of the proposed changes to the PMPRB and to ensure that their jurisdictions will support the cost of life-changing and life-saving medicines as soon as they become available to provincial formularies.

25. We also ask that the provincial governments immediately negotiate with the manufacturer for the drugs already approved by Health Canada for cystic fibrosis, so that people with CF who could benefit from other disease-modifying drugs can access these medicines now.