UNIVERSITY OF SASKATCHEWAN
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Saskatchewan Health Authority

14 February 2020

To: The Patented Medicines Price Review Board (PMPRB)

To whom it may concern at the PMPRB,

I am a pediatric respirologist in Saskatoon, Saskatchewan and the Clinic Director of the North Saskatchewan Cystic Fibrosis Clinic.

As a physician, a taxpayer, and a proud Canadian, I am grateful that under our medical system, my patients have access to world-class clinical CF care. CF outcomes including life expectancy, transplant survival, and nutritional status in Canada lead the world at present. But this could change.

We have a patient who has just been approved for the Special Access Program for Trikafta, but the number of delays and barriers set up for access have been incredible. In addition, Special Access is only available for the sickest patients at this time.

I need you to take pause and consider how the PMPRB can help Canadians living with cystic fibrosis (CF).

Trikafta is a game-changing drug that targets the basic defect of cystic fibrosis. It can treat up to 90% of Canadians with CF. On October 21, 2019 the Food and Drug Administration (FDA) in the U.S. approved Trikafta for sale in the United States six months ahead of schedule. That's how important this drug is. When the Washington Post wrote about 19 good things that happened in 2019 - #1 was the announcement of Trikafta.

Recent changes to the PMPRB could result in very large price reductions being imposed on patented medicines, even innovative, life-changing medicines like Trikafta. Some sources claim that the changes could lead to price reductions between 70%-90%. No business wants that to happen to their product.

However, I have also spoken with representatives of the company and encouraged them to act in good faith in price negotiations with provinces and Canadian government agencies, to ensure fairness and accessibility without causing undue burden to the system. I have asked them to please proceed with submission for Health Canada approval and not leave our patients behind.

Companies, especially those with drugs for rare diseases like Trikafta for cystic fibrosis, say that they are worried by potential changes in Canada. The PMPRB regulatory changes may make it even more difficult to get life-changing and life-sustaining medicines like Trikafta to the people who need them. Putting further restrictions on medicines at the point of entry to our country will lead to longer wait times, if manufacturers choose to bring their medicines here at all. CF is a progressive, fatal disease and people with CF can't wait.

The federal government has committed to improving access to medicines for Canadians with rare disease by implementing a rare disease strategy. However, the changes proposed to the PMPRB could result in no new, innovative medicines for rare diseases being brought to Canada. I ask that the PMPRB reforms be placed on hold for further consideration until their impact on rare disease medications and

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drugs like Trikafta can be properly assessed and changes made to ensure that drugs such as this one are quickly made available to Canadians.

Canadians with cystic fibrosis should not have to die because they don't have access to these medications. **Canadians with rare diseases need access to future medications to live productive lives and contribute to our society.**

Sincerely,

Martha L. McKinney, MD MPH FRCPC
Associate Professor
University of Saskatchewan
Director, Northern Saskatchewan Pediatric Cystic Fibrosis Clinic