The Honourable Tyler Shandro
Minister of Health

March 5, 2021

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Subject: Alberta brothers battling Cystic Fibrosis. Brother born in USA gets lifesaving drug / Brother born in Canada doesn't have access. Please push to unconditionally fund Trikafta (CF Modulators)

As the parents of two sons afflicted with Cystic Fibrosis (CF), the most common fatal genetic condition, we feel obliged to share our story of coping with this insidious disease, and the impact it has had on our family. We appreciate that your attention, as Minister of Health, is understandably focused on the current pandemic, and we respectfully ask for your indulgence as we characterize our daily life of living with the prospect of potentially losing one, if not both of our sons. Importantly, we also want to share our hope for a solution that will not only save our family, but also prevent other families from having to live our life, as described below, for the last 19 years.

If our lives were a movie, it could be classified as a tense drama, sprinkled with precious, uplifting moments in a plot filled with dark twists and turns. It's a rollercoaster ride that never stops, one that takes you from the highest of highs to stomach-churning lows of despair. It's a place where a doctor's simple utterance can in one moment, like a pinprick to a balloon, deflate one's spirit, morale and hope.

Imagine going to bed every night with a knot in your stomach, sick with worry for the health of your children. That has been the norm for us ever since our two beautiful boys, now handsome, "honour-roled", young men, Zachary and Brett, came into our lives. From the moment they arrived in this world, we have never known for certain what tomorrow would bring, when the next 2:00 a.m. trip to Emergency would occur, or whether hours of chest therapy would reduce their discomfort of constant rasping and wheezing. Imagine trying to be brave for your child as you hold him while he coughs and struggles to breathe, whispering that everything will be okay when you're honestly not sure that it will. Then you wake up and try to be productive at work the next day. This is our reality.

There are days when you simply scan the news and search the Internet, looking for signs of hope, a breakthrough, or relief. Your emotions are raw, your energy more shallow than low tide, yet you try to be positive and optimistic. Simply put, watching your children suffer is excruciating.
Knowing their next bout of pneumonia could send them into a downward spiral toward lung transplantation leaves you feeling helpless, powerless to safeguard those you are supposed to protect as a parent.
Adding to our pain described above, is knowing that a life-saving drug is available to CF patients. A miracle developed by Vertex Pharmaceuticals called ***Trikafta*** has changed the lives and long-term outlook for CF patients worldwide. The silver bullet that can slay the CF monster and provide a chance at a normal life for loved ones. Then you learn that it is approved for use and funded in most countries in the western hemisphere, *except for Canada*.

We have now seen the results of Trikafta firsthand, in one of those uplifting moments in the movie of our life…and the results have been miraculous.

Our oldest son, Zach (19) has been plagued with an unresponsive, highly resistant gram-negative bacteria over the past five years. During this time, he's used colistin (the "antibiotic of last resort") twice daily, converting an IV med into an inhalant to reduce the threat of a deadly bacteria ending his life. A few weeks before Christmas 2020, Zach was hospitalized on IV antibiotics. His FEV1 measured at 40%. It was devastating news for all of us. We thought the moment we had dreaded since his birth may have arrived. CF was finally going to win.

Desperate times called for desperate measures. We were determined to get Zach Trikafta no matter what. While hospitalized in Calgary, a clinic in the USA took the generous step of allowing a virtual visit and assessment and prescribed Trikafta on the condition that he was "in the process of moving" to the United States. Shortly afterwards we took the COVID risk and flew to the United States for Zach's first month's supply.

It was one week after Zach's lung function (FEV1) came in at 40% that he began taking Trikafta. Three weeks later, after starting Trikafta, Zach's FEV1 was 71% and his CF team in Calgary stopped his antibiotics. As parents that have been expecting the early death of a child, the feelings we had upon seeing these results were nothing less than euphoric. To know that your child now has a realistic future in front of him is indescribable, and we wish we had better words to capture how we felt upon hearing the results. The rollercoaster we had been riding for 19 years had brought us to an inexpressible peak. Our lives have been forever changed. Zach's life has been changed forever.

Trikafta (or it's successor) is a drug that Zach will have to be on for the rest of his life. In order to keep receiving Trikafta, Zach had to move to the USA. Zach has a U.S. passport because he was born in California while we were working as expats - but he grew up in Canada. Our family is Canadian. Our 16-year-old son Brett, born in Canada, also has CF. Our two sons are best friends, bound together by their love of family and a shared medical journey that those outside of the CF community can't begin to imagine.

And now they are separated in terms of the chance of experiencing a normal life and physically.

Just before leaving us, we spent a weekend together as a family in the mountains where Zach was able to do what he loves best – climb a peak and ski his favourite pitch. His stamina and fragile lung capacity were preventing him from fully enjoy these pursuits; that is, prior to taking Trikafta.

In those final few days together, after Zach had been treated with Trikafta, he climbed and skied like he was born to. We'll never forget his smile when he told us he was never out of breath. For a CF family, this is bigger than winning the lottery. Money can't buy you happiness or life, but Trikafta can deliver both. It's been a disturbing revelation that we've sent our eldest son to the USA so he could gain access to better health care.

We agreed to share this private part of our lives because, as parents, we feel obligated and determined to help our son Brett, along with other Canadian CF patients (and their parents) by sharing our story.

Trikafta works! The proof of this can be found in the incredible results we have personally witnessed, and the number of countries where it has been swiftly approved and is readily available to its citizens.

The latest update includes Zach's lung function continuing to climb and the resistant bacteria that have plagued him for five years are gone – despite stopping antibiotics (and other drugs). He's gaining weight and abdominal pain he's suffered with his whole life is gone. Canadian CF patients and their families are seeing stories like Zach's roll in from around the world while they and their children suffer.

Trikafta was fast-tracked for access by the U.S Food and Drug Administration (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.

Minister Shandro, all is not lost, as hope is on the way for Canadian families and for those living with CF. Health Canada has confirmed that in December 2020 Trikafta was accepted for priority review with a timeline of 180 days. Further, the Canadian Agency for Drugs and Technologies in Health (CADTH) is also reviewing the cost effectiveness of the drug, that we hope will lead to being listed on Alberta's public drug plan. Unfortunately, this process is excruciating slow – and Trikafta's predecessor took nearly 5 years from Health Canada approval to gain funding! The problem with this is these drugs are PREVENTATIVE. Excessive delay or worse – placing a funding condition on a patient needing to have low lung function - defeats the purpose of the drug - and is inhumane. And CF eats away at patients' lungs each and every day. Time is not on our side.

As loving parents, as loyal Albertans, and proud Canadians, we ask that you speak with the Ministry's drug program managers about expediting price negotiations for Trikafta. Our Allies are taking care of those with CF. Alberta/Canada: please keep up this time. We'd be grateful to speak with you and/or your staff so we might relay other details not contained in this letter. Please let us know if a call may be scheduled.

Sincerely,

Chris and Michelle Bushell, Calgary, AB

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Summary

- **Request:** Unconditionally fund Trikafta now and figure out what's broken in our novel medicines systems later. Don't make patients and their families suffer while Provincial and Federal agencies try to fix our targeted and novel medicines angle. This should have been thought of years/decades ago. Don't make us suffer for it. Improvements to our novel medicine systems will take years and the Liberal-formed PMPRB review is taking the wrong path anyhow. How can we negotiate drastic price reductions when representing .2% of the world's population? The answer is to join consortiums – not to try to negotiate from a position of weakness at the expense of treating citizens humanely. Changes take years and we can't wait.

- **Political Implication:** Trikafta is the most visible major disease miracle-drug being widely distributed everywhere in main countries except Canada. It's a crystal ball into what will happen when a miracle cure for cancer comes about. Providing access to Trikafta will help with public support.

- **Humanitarian Implication:** We're a G7 nation and we're letting people degrade and die when they're being saved in 32 other countries.