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Name	Meredith Chiasson
Organization	Nova Scotia Health Authority
Job Title	Respirologist
Address	Halifax, NS

I am a respirologist, with cystic fibrosis care as an area of focus. While as a taxpayer I agree with cost containment measures around medications, what I do not think the government is aware is that orphan condition medications come with a hefty price tag. Pharmaceutical companies are a business, and they want to make money. If they cannot make money in a certain disease category, then they will not invest money in that area. Meaning that orphaned diseases would not be an area of interest for pharmaceutical companies. This unnecessarily punishes people with rare diseases. Especially when the disease are life limiting, such as with cystic fibrosis.

I want to make sure all stakeholders in these guidelines are aware of what will happen, and already is happening due to these guidelines. There are CF modifier drugs created by Vertex pharmaceuticals, they are novel in what they do, and come with a hefty price tag. Most countries in the world have agreed to fund them, Canada has not. Vertex has now decided that they will not apply to Canada for approval of the most recent drug, Trikafta, due to these guidelines. Trikafta is a drug that would benefit more than half of our CF patients, resulting in a substantial increase in their lung function. This enables them to get back to work and school and delays or prevents the need for them to get a lung transplant.

With Kalydeko, the drug was approved, and the provinces agreed to pay. All patients with the appropriate mutations are now on this life changing drug. The next two drugs to come out of the pipeline (Orkambi and Symdeko) were approved for sale in Canada, but not covered by provincial programs. Only our patients with private coverage have been able to benefit from these drugs. This has been frustrating, but as these drugs were not the greatest, and did not show the same benefit as Kalydeko most CF doctors agreed we would not push for universal access.

With Trikafta, it is a game changer. It would have dramatic life changing effects for our CF patients, in the same range as Kalydeko did. But, with the new guidelines we cannot get Vertex to apply to Canada. Vertex has created a special access program, such that the very sickest of our patients can gain access. Most of the patients who have an application submitted have been approved, but don't let this fool you into thinking most patients have been approved. The criteria are very restrictive and only the sickest qualify. We need ALL cystic fibrosis patients to be able to access the medication. Please review your policy. You are hurting patients, those with orphaned diseases in particular.

Thank you