CF GET LOUD

An Open Submission in response to the Proposed Guidelines of the Patented Medicine Pricing Review Board and their Implications on the Cystic Fibrosis Community

Submitted to:
Patented Medicines Regulations Consultations
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We would like to thank you for considering our submission as part of the official consultation period on the proposed PMPRB guidelines.

It is an exciting time for people who live with cystic fibrosis (CF). We are now approaching a new era when it comes to our health and prognosis. Over 4400 Canadian families are for the first time cautiously optimistic about our future living with CF. We stand alone in absence of support from our Canadian health care system and government, but very much together as we rally in hope for a future when 60 families per year are spared the devastation that comes from the preventable death of a loved one. This disease is ruthless. We have been hopeful for this moment for over 3 decades since the CF gene was discovered in 1989 at our very own Sick Kids Hospital in Toronto.

About CF Get Loud

CF Get Loud Canada was founded by a few patients, on IV poles fighting for their lives. HOPE was ignited in the CF community when Trikafta gained FDA approval in the United States. We watched helplessly while our CF community south of the border was given a second chance at life while we continued to deteriorate. We refused to sit idle and wait. We are used to losing physical battles with CF and dealing with the toll that slowly losing each battle takes on our bodies. However, we were unprepared for the mental and emotional toll of watching months go by, slowly dying, still without Trikafta even having an application submitted to Health Canada for approval. Until recently, we truly believed that Canada provided the best opportunities and outcomes for a CF patient.

Our mission is to help EDUCATE our community, ELEVATE our voices, and EMPOWER advocates.

Throughout these last 8 months we have rallied together a group of 4,000 people, willing and ready to fight on our behalf. We have united a community of advocates and allies. We have awakened a patient group that for too long has felt isolated and hopeless. We have meticulously researched and sought to understand the obstacles in the path to access for gene modulators. We strive to help others like us understand the challenges ahead due to the inefficient processes that are inadequate and unacceptable for the rare disease community who require precision, life-saving medicine. We encourage them to speak up for themselves and their families in hopes that we are heard and acknowledged.

The Guidelines

This process has been challenging. As the CF landscape has progressed, we never thought as Canadians we would have to fight so hard for these innovative breakthrough therapies. CFTR modulators have already had a challenging battle when it comes to public funding in our country,

but never did we think that there would be mandated regulations keeping the future of precision medicine beyond the reach of Canadians and denying us access to innovative therapies.

The undertaking of these guidelines reflects a policy decision detached from the PMPRB's original mandate and has lost touch with the present. It ignores the future of pharmaceutical innovation, as well as the pride that is our Canadian health system. We are caught at a crossroads of policy changes not informed by up to date data or "fixing something that is broken", but rather a one size fits all makeshift solution that ignores several unintended consequences.

Taking a macro perspective, there is no justification or reason why Canada must contribute human capital (lives lost), in order to send a message to the pharmaceutical industry. As Canadians, we are proud of our health system and will not, and cannot, compromise on being a leader among nations in bringing innovative therapies to our patient community.

The language highlighted this year due to the pandemic emphasizing "listen to the experts" and "let medical professionals decide what is best because THEY know best" illustrates why it is tone deaf and wrong to allow health economists decide who lives and who dies.

These decisions represent life or death to our community, and to many other Canadian families outside of the CF community. We ask that you not take the impact on our and their lives lightly.

If someone you loved faced a rare disease diagnosis, would you not be grateful if your country stood by them and their community in their time of need? If you lost a loved one, can you think of how many people would be affected by their death? How many people would show up at your daughter's, or son's funeral? 50, 100? If you count our CF community and calculate the effect on 100 people per every CF patient, 440,000 Canadians will be impacted by these changes. That is only in the CF community and does not take into account that every other rare disease and some cancers are in the crosshairs if the PMPRB implements these guidelines with the opportunity to alter/adjust if needed. This is a flippant and dismissive response when lives are on the line, and they are.

You have the power to change all of this.

We hope that our idealism does not fall on deaf ears. Arguing specific economic factor formulas, gap vs new medicine categories, and therapeutic criteria all have their place in this problem. Other organizations like Cystic Fibrosis Canada and the Canadian Organization for Rare Disorders (CORD) have all done an excellent job highlighting the crux of the problem and have provided recommendations for a path forward. The recent court ruling, and the upcoming legal challenge put forth by the CF Treatment Society adds yet another layer to the problematic and unreasonable nature of the proposed guidelines.

Our goal is to highlight the human capital your decisions will be expending. So many Canadian lives are at stake. We respectfully ask that you make the necessary adjustments as if it was your family being directly affected by these guidelines

The Consultation Process

While we appreciate the opportunity to make this submission, the overall consultation process lacks in depth and engagement. The patient community has not been engaged or invited into decision making processes that claim to consider patient voices but ultimately fail to create an environment for proactive feedback. The language used in the guidelines and the lack of a timely release of the backgrounder only adds frustration to the understanding of the proposed guidelines and create a gap between the PMPRB and the public. Health policy experts and other professionals in the field have had trouble interpreting the guidelines. How do you expect the average Canadian to understand and in turn give any feedback on these guidelines?

The process is one dimensional and completely arbitrary, very much like the guidelines themselves. The public consultation webinar streamed in July 2020 used data from the 2017 PMPRB annual report and it was only until most recently that the 2018 PMPRB annual report was tabled in parliament. The PMPRB's mandate is to both report and keep prices in line, but these changes are being rolled out long before the relevant report substantiates (or not) the proposed changes. It is a statutory requirement to release the annual reports and you are 2 years behind. How can you propose to bring forth a change in regulations without the evidence to support it? Additionally, it is difficult for us to trust the data used in the public webinar as it is outdated. Specific to the CF slide, it is demonstrably false and misguided. You have failed to understand the current CFTR modulators and the number of patients who will benefit from them by a large margin. If your data is categorically wrong when it comes to CF, how are we as Canadians to trust the rest of your data and figures?

Letters for Lives Initiative

As part of this consultation period, we were faced with a big challenge. The guidelines are nearly incomprehensible to our community, as we are not health economists. They require interpretation and much help in understanding what they mean to the CF community and our fight for access to life-saving medication. Our role and mission in this battle is to help our community understand these changes, to support and educate others as to what the implications are, and to help elevate our community's voice. We strive to empower every Canadian that is affected by these changes to speak up for themselves and make a stand for their lives and/or the lives of their loved ones.

We were compelled to make it as easy as possible for Canadians to express their feelings and what these changes mean to them in the form of a submission letter template and an easy way to participate in the stakeholder consultation process through our website (cfgetloud.ca).

We ask you to consider each submission as a unique voice. They are the feedback of individual taxpaying Canadians. They should be considered as such, and not as part of an initiative or

amalgamated into one umbrella. We have received 11,191 submissions to date and we respectfully ask that you consider each one.

Our Ask

Our ask is simple: we need Trikafta in Canada NOW.

Please take into consideration ALL Canadians before implementing policy changes that ultimately decide who lives and who dies.

Thank you for your consideration.

CF GET LOUD

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