

**Joint Submission to Health Canada:
National Strategy for High-Cost Drugs for Rare Diseases**

Introduction

Canadians with rare disorders need the federal government to implement a comprehensive rare disease strategy – one that prioritizes patients and improving their quality of life and wellbeing. Instead, what has been proposed by Health Canada is a strategy for cost-containment. This is worrisome and unacceptable. But it is just one of several recent and ongoing regulatory and policy reforms being undertaken that will slow access to important life-changing and life-saving medicines for Canadians.

Since the changes to the Patented Medicines Pricing Review Board (PMPRB) regulations were first proposed in 2017, the patient community has consistently raised concerns that these changes will negatively impact access to new medicines and clinical trials for Canadians. We strongly believe that the PMPRB changes, as they currently stand, as well as any other policies that focus solely on cost-containment are an impediment to patients achieving fair and quick access to the medicines they need. In particular, narrowly focusing on cost-containment deprioritizes rare disease patients and ultimately, puts their lives, and the lives of all Canadians who are waiting for access to new and innovative drugs, at risk. While the government's approach to PMPRB reforms and its Strategy for High-Cost Drugs for Rare Diseases may succeed in saving government drug plans money, it will be at the expense of patients' quality of life, and may cost others more than that.

On behalf of our collective organizations, we respectfully submit the following recommendations, which reiterate those put forward by the individual participating organizations and which are of vital importance to Canadian patients.

Protecting Access to New Medicines

Our collective organizations, and the patients we represent, support efforts to lower the cost of prescription drugs for Canadians. We expect pharmaceutical manufacturers to bring their products to market at a responsible price, but we also expect the government to ensure that the regulatory environment in Canada does not unnecessarily limit our ability to quickly and fairly access new therapies that hold promise to improve the health of Canadians.

Efforts to lower drug prices must be balanced in a way that encourages ongoing innovation and the launch and uptake of new medicines, including drugs for rare disorders, into the Canadian market. However, we are concerned that the current emphasis by government on cost-containment – over access and seeing patients benefit from scientific innovation – will greatly disadvantage millions of Canadians who are waiting for new therapies.

We are particularly concerned about the impact that the current cost-containment policies will have on companies offering precision medicines and innovation in the rare disease space. Small and large pharmaceutical companies have indicated that it will be very difficult to adapt to regulatory changes such as the PMPRB and, as a result, new therapies for rare diseases may face barriers in Canada which will slow, or prevent, access to those new medicines. Canada needs a fair regulatory environment and process for drugs for rare diseases, but the proposed policy changes will make Canada an unfavourable market for launching new drugs and clinical trials¹.

¹ <http://www.raredisorders.ca/content/uploads/CORD-submission-to-HESA-on-PMPRB-Nov-6-20.pdf>

The government has dismissed Canadian patients' concerns by insisting these regulatory changes and cost-containment policies will not create barriers to access to new medicines and clinical trials. Unfortunately, the facts don't support this claim. New regulations have already created a chilling regulatory, review and reimbursement environment in the Canadian market. For instance, Canadians living with cystic fibrosis have [died waiting for access](#) to a new therapy that could have saved their life, but which is still not available in Canada because the manufacturer delayed bringing the drug to Canada as a result of the proposed regulatory changes – and there are other patients living with other diseases across Canada that are similarly struggling to access therapies currently available in other countries.

The lack of incentives for manufacturers to conduct clinical trials or commercialize new drugs, including drugs for rare diseases in Canada has ultimately resulted in access issues. Right now, it is estimated that only 60% of the drugs approved by the US FDA are available in Canada and most that are get approved up to six years later than the US or Europe². Canadian patients are not only being left behind; their lives are being put at risk.

Despite claims to the contrary, government knows the proposed changes will delay access to new therapies, which is why it exempted COVID-19 vaccines and treatments from its new regulatory regime. In doing so, it has implicitly acknowledged that the proposed changes will delay access to new medicines for Canadians.

We appreciate and support the need to expedite access to these life-saving COVID-19 vaccines and therapeutics, but there are millions of Canadians living with life-threatening conditions or who may benefit from transformational treatments who also need fast access to new medicines and for whom time is running out. But no such exemptions exist for them. Canadians deserve quick and fair access to all new medicines, not just when it comes to COVID-19.

Canadians value fairness and equity. It is the very foundation of universal healthcare. Canada does not apply a monetary cap to each patient's healthcare services, nor would governments ever suggest that needed, potentially life-saving services should be withheld from a Canadian because it is expensive. Yet, this is precisely what is being proposed when we reduce drugs, particularly those for rare diseases, to their economic impact.

RECOMMENDATION: *Develop a Drugs for Rare Diseases strategy that has at its core the value of bringing innovative medicines to impacted Canadians as quickly and as equitably as possible.*

RECOMMENDATION: *Establish a multi-stakeholder advisory group to evaluate the impact of PMPRB changes on availability of medicines, in particular those for rare diseases, and specifically to inform any decision on whether and how to implement the use of new economic criteria. Until this is completed, and the value of these measures is demonstrated, no such measures should be adopted.*

² Herder, M. and Krahn, T. M. Some Numbers behind Canada's Decisions to Adopt an Orphan Drug Policy: US Orphan Drug Approvals in Canada, 1997-2012. Healthcare Policy. Longwoods, 11(4), May 2016. Last accessed March 22, 2021. <https://www.longwoods.com/content/24626/some-numbers-behind-canada-s-decision-to-adopt-an-orphan-drug-policy-us-orphan-drug-approvals-in>

Importance of Involving Patients

One of the biggest challenges the patient community has faced as the government seeks to make substantive changes with regards to pharmaceutical policy and regulations has been government's flawed process. The lack of meaningful engagement of groups representing patients has resulted in flawed policy. The government has failed to strike the right balance between lowering drug costs and ensuring new life-changing medicines are still made available to Canadians, but just as importantly, it has failed to respect and really listen to patient groups.

While government may claim that it has fully and meaningfully consulted with Canadians, including patients, the organizations that represent them and the clinicians who treat them, posting open consultations and accepting submissions should not, and cannot, be the limit of participation. Patients must be given a voice through meaningful and ongoing involvement in policy development and decision-making – a role that recognizes that ultimately, the groups whose lives are most affected by these changes are those of patients. Instead, government is repeating a flawed and broken process that proves the patient perspective is not truly valued. Until government makes an inclusive and robust process, one that reflects that patients' interests matter as much as governments cost-containment objective, government is and will continue to fail patients.

What's more, government cannot continue to treat and engage on complex pharmaceutical policy reforms in siloes. These policies are not mutually exclusive but rather, intertwined in how they interact with one another and most importantly, the impact that they have on access to new medicines and therefore, the lives of patients. Patients must be at the table and meaningfully engaged in conversations and decision making that looks at pharmaceutical policy reforms and their impacts in their totality.

Going forward, we are calling for the establishment of a formal mechanism for ongoing and continuous engagement of patient representatives. Patient Advisory Councils aimed at improving access for themselves and others are utilized in many of Canada's comparator countries. Now that policies such as the PMPRB and Strategy for High-Cost Drugs for Rare Diseases will be weighing in on the economic value of patient lives, patients need to be at the table and weigh in on the value of their lives too.

RECOMMENDATION: *Require a formal mechanism for meaningful and continuous engagement of patient representatives in the drug decision-making process be established to ensure patient voice, choice, and representation.*

About Protect Our Access

Protect Our Access is a group of leading health charities and patient groups that represent the needs of patients across Canada, working together to raise concerns about draft guidelines proposed by the PMPRB. We came together out of frustration that the perspective of patients was not being fully valued by government in this process.

Our objective is to communicate to government and the public the importance of protecting timely and equal access to innovative medicines for patients; striking the right balance between reducing costs and ensuring Canadians continue to have access to new medicines.

The participating organizations of Protect Our Access are:

ALS Society of Canada
Canadian Cancer Survivor Network
Canadian Hospice Palliative Care Association
Coalition Priorité Cancer au Québec
Colorectal Cancer Canada
Cure SMA
Cystic Fibrosis Canada
Fighting Blindness Canada
Lung Health Foundation
MitoCanada
Ovarian Cancer Canada
PROCURE – The Force Against Prostate Cancer
Québec Breast Cancer Foundation
The Leukemia & Lymphoma Society of Canada