November 6, 2020

Standing Committee on Health
131 Queen Street, 6th Floor
House of Commons
Ottawa, ON K1A 0A6
Canada

Dear committee members,

Subject : Brief on the PMPRB study from the Regroupement québécois des maladies orphelines (RQMO) to the Standing Committee on Health

On behalf of the Regroupement québécois des maladies orphelines (RQMO), we greatly appreciate the opportunity to contribute to the Standing Committee on Health's study on Patented Medicine Prices Review Board (PMPRB) reforms. This study is important for the future of health care in Quebec and Canada, especially for people with rare diseases.

We're extremely concerned that the PMPRB reforms will unduly and unreasonably limit access to life-saving or life-enhancing drugs for people with rare diseases. These patients are already penalized by our existing health care system, as Canada has no policy or program for rare diseases like other industrialized countries. Patients face long delays in getting a diagnosis, often sub-optimal care and difficulties in accessing treatment. Moreover, in 2020, barely 900 drugs were dedicated to treating specific rare diseases, even though approximately 7,000 rare diseases have been identified!

The RQMO launched a project to monitor orphan drugs submitted by companies for marketing in Canada and to track their authorization and reimbursement by public drug insurance plans. We began this project because Canada does not have orphan drug regulations like in the United States[1] or Europe[2]and government agencies do not use the "orphan" designation for drugs for rare diseases. According to our recent analysis for this project (July 2020), of the 837 orphan drugs approved by the Food and Drug Administration (FDA) in the United States since 1983, only 491 (59%) are available in Canada. One of the reasons for this — and the main reason — is that companies have not applied to market the drug in Canada. In addition, only 23% of these orphan drugs approved by the U.S. FDA are on the list of drugs reimbursed by Quebec's public drug insurance plan. Patients already face barriers accessing the treatment they need, and PMPRB's new pricing guidelines will only make the situation worse.

More specifically, the changes to the final guidelines do not adequately address our concerns about the impact on patient access to new drugs and clinical trials. We are extremely disappointed that the PMPRB and Health Canada did not address patient concerns in this regard, and that no analysis of the impact of these changes on access to drugs and clinical trials has been done. Changing the PMPRB's rules without

[1] *Orphan Drug Act*, Food and Drug Administration, United States: https://www.fda.gov/industry/designating-orphan- product-drugs-and-biological-products/orphan-drug-act-relevant-excerpts
[2] *Orphan Designation*, European Medicines Agency: https://www.ema.europa.eu/en/human- regulatory/overview/orphan-designation-overview

knowing the full implications of these changes is irresponsible, especially at this critical time when we need access to the latest drugs to keep patients out of hospitals as much as possible and fight COVID-19.

We're already seeing this impact. Revolutionary new drugs are unavailable in Canada because of price uncertainties created by the reform. Companies have begun to abandon the Canadian market for launching new drugs because they no longer consider it to be commercially viable.[3] For example, while the cystic fibrosis drug Trikafta is now available in the United States and Europe, the company has yet to file an application with Health Canada. As a result, patients' health is deteriorating rapidly, and some will die while waiting for the drug to reach Canada. The cystic fibrosis community is the first to be affected by the PMPRB reforms. However, if the new pricing guidelines are implemented, we're concerned that many other revolutionary treatments won't be available to Quebec patients, including therapies for amyotrophic lateral sclerosis (ALS), a disease related to vision loss, muscular dystrophy and cancer. Rare disease patients simply can't afford to wait any longer for access to new drugs.

In addition, the PMPRB's new pricing guidelines will block access to clinical trials. There is often no treatment for the rare diseases that patients suffer from, and patients eagerly await the chance to participate in clinical trials and gain access to new drugs under development. These trials are an essential part of the treatment of rare diseases, and patients rely on them to improve their condition and stay alive. Unfortunately, the PMPRB's new pricing guidelines on clinical trials and research in Canada are already having a negative impact.[4]

In addition to the impact on patients, reduced investment in clinical research in Canada could have two other major consequences for our health care system.

The first is the significant support this investment brings to Quebec's health care system. For many health care institutions, external funding of clinical research has led to comprehensive research departments and significant expertise. Having this infrastructure is extremely beneficial because it allows many research projects to be carried out in an efficient, timely and cost-effective manner by experts. Any significant reduction in external research funding threatens this infrastructure and, consequently, the existence and profitability of many other research activities.

The second major consequence of reduced investment in clinical research in Quebec is reduced access to leading medical experts for Quebec patients. Currently, thanks to the supportive environment for clinical research in Quebec, our leading clinicians and researchers can stay in the province serving our patients while having a rewarding and world-renowned research career. If access to research in Quebec is reduced, many of these experts will choose to relocate or pursue careers elsewhere, depriving Quebec patients of access to their expertise.

Finally, we're concerned that the federal reform will hamper the Quebec government's efforts to accelerate access to new drugs for rare disease patients. Quebec recently made changes in the evaluation of rare disease treatments and established an advisory committee for rare diseases. Its efforts will be greatly undermined by the federal reform, which will significantly restrict access to treatments for rare diseases.

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[3] IQVIA study, *New Medicine Launches: Canada in a Global Context*, June 2020, p. 14-15

[4] Nigel SB Rawson, Canadian Health Policy, April 2020, p. 4: https://www.canadianhealthpolicy.com/products/clinical-trials-in-canada-decrease--a-sign-of-uncertainty-regarding-changes-to-the-pmprb-.html?buy_type

In conclusion, as patients, we support the need to have access to the right medication. For rare disease patients, most of whom suffer from progressive and life-threatening conditions for which no effective treatment is currently known, this means having access to new drugs as quickly as possible. For this to happen, there must be access to clinical trials, rapid regulatory approval and streamlined funding decision-making.

We believe it is important to take a moment to register the impact that the PMPRB reform is already having and consider a different, more sustainable process developed in concert with patients and other health care stakeholders to ensure that Quebeckers, including those suffering from rare diseases, have stable, cost-effective access to prescription drugs.

We hope you will consider our views and recommendations as part of this study.

Sincerely,
[sgd]
Gail Ouellette, PhD.
Founding CEO and Science Director
Regroupement québécois des maladies orphelines

Email: gail.ouellette@rqmo.org Tel.: 819-437-6414

RQMO
2030 Pie-IX Boul., Suite 403
Montreal, QC H1V 2C8
www.rqmo.org

The Regroupement québécois des maladies orphelines was founded in 2010. It brings together 31 rare disease patient associations and individual members affected by rare diseases, parents of affected children and other caregivers. A rare disease is a disease that affects less than one person in 2,000. Half a million Quebeckers are affected by it.

RQMO's mission:

1. Provide information and support to people affected by rare diseases, their families and health professionals through its Centre iRARE, a rare disease information and support centre.
2. Share information on rare diseases and related issues.
3. Promote awareness of rare diseases among the public and the medical community.
4. Advance knowledge on various rare diseases by promoting partnerships between patients and researchers.

RQMO member associations:

1. Canadian Association for Familial Ataxias - Claude St-Jean Foundation
2. Association québécoise des personnes de petite taille
3. CORAMH (Corporation de recherche et d'action sur les maladies héréditaires)
4. Association d'acidose lactique du Saguenay-Lac-Saint-Jean
5. Groupe d'aide aux enfants tyrosinémiques du Québec
6. Leukodystrophy Foundation
7. Canadian Fabry Association
8. National Gaucher Foundation of Canada
9. Fondation Jean-Michel Dufour
10. Loeys-Dietz Syndrome Foundation Canada
11. Anti-NMDA Receptor Encephalitis Foundation
12. Fondation hypertension artérielle pulmonaire du Québec
13. Fondation du syndrome d'Angelman du Québec
14. Canadian PKU Canada (phenylketonuria)
15. Eeyou Awaash Foundation (Cree encephalitis and leukoencephalopathy)
16. Muscular Dystrophy Canada
17. Communauté Morquio du Québec
18. Association de la neurofibromatose du Québec
19. Soutien hétérotopie nodulaire périventriculaire
20. Sclérodermie Québec
21. Association d'anémie falciforme du Québec
22. Aplastic Anemia and Myelodysplasia Association of Canada
23. Association des patients immunodéficients du Québec
24. Association du syndrome de Turner du Québec
25. Association québécoise de la névralgie du trijumeau
26. Vivre avec la fibrose kystique
27. Association québécoise du syndrome de Rett
28. Association du spina bifida et de l'hydrocéphalie du Québec
29. Regroupement des aidantes et aidants naturels de Montréal
30. Fondation La Force
31. Fondation Simon-le-zèbre