RAREi
THE CANADIAN FORUM FOR
RARE DISEASE INNOVATORS

RAREi Submission

House of Commons Health Committee's 2023 PMPRB Study

May 12, 2023

Thank you for the opportunity to offer a rare disease innovator perspective to the committee's current study on the Patented Medicine Prices Review Board (PMPRB).

The Canadian Forum for Rare Disease Innovators (RAREi) is a network of 17 biopharmaceutical companies dedicated to improving the lives of patients with rare diseases by developing and commercializing treatments.

During the past several years, RAREi has actively engaged in the Health Canada and PMPRB consultation processes on both the regulations and the guidelines. RAREi has provided input on recent health committee studies regarding the PMPRB and rare disorders in previous parliaments. All its submissions are available on the RAREi website (www.rarei.ca).

Background on Canadian rare disease treatment landscape

Let's begin by providing some background on the rare disease treatment landscape and some of the challenges faced by rare disease patients. Canadians affected by often severe and debilitating rare disorders already struggle to access needed treatments. This is in addition to the multiple other challenges faced by rare disease patients, including the very long journey to reach a diagnosis, small patient populations, fewer health care resources available to treat the conditions and many unknowns about the disease. For many rare disease patients, there are no medicines available yet to treat their condition. That is why it is important to ensure that those that are developed can be made available to patients who need them as soon as possible.

The new rules proposed by the PMPRB up to this point – including the guidelines that were the subject of consultations last year – would have significantly exacerbated the challenges faced by rare disease patients, researchers, clinicians, developers and health systems.

At the moment, no element of the Canadian medication review and approval process, including regulatory review and approval, pricing review, health technology assessments, product negotiation processes and funding frameworks is set-up to evaluate these treatments fairly. In contrast, many developed countries – including in Europe, the United States, Japan and Australia – have implemented tailored review processes and incentives to encourage manufacturers to develop and commercialize rare disease treatments.

Evidence exists already to demonstrate that rare disease therapies are launched in Canada much later than in the United States and/or Europe and, in many cases, are not even available for patients in Canada. To put things in perspective, fewer than two-thirds of the rare disease treatments approved in Europe were submitted to Health Canada for regulatory approval, and among those approved by Health Canada, the rate of public reimbursement is

low.[1] Meanwhile, only 54% of rare disease treatments approved by Health Canada are subsequently reimbursed by public drug plans in Canada.[2]

The reality is that developing treatments for rare disorders is an extremely risky and costly endeavour. Investments made in creating a new rare disease treatment must be recouped from a smaller pool of patients compared with medicines for common diseases. On the other hand, the current and projected public spending for rare disease medicines is small as a proportion of total costs, and is projected to be manageable in the coming years.

RAREi has supported ground-breaking research on this issue which has been published in posters and journals and subject to peer review.[3] These analyses found that in 2021, spending on non-cancer rare disease medicines accounted for just 3.2% of the $16.5 billion spent on medications by governments. That proportion is forecast to rise to 8.3% by 2025. Moreover, the actual spending on rare disease medicines is certainly much lower because the analysis does not account for the value of confidential manufacturer discounts or health outcomes-related offsets, which brings down total spending very significantly. The authors conclude that limiting access because of this growth is not aligned with Canadian patient or societal values.

Why the PMPRB's approach is so problematic

RAREi would like to use this opportunity to highlight some of the key challenges with respect to the PMPRB's 2022 Guidelines proposal, which was the subject of much of the discussions during the committee's hearings in April and May 2023.

- PMPRB acting outside of its mandate: From the outset of the PMPRB reform process, the board has consistently sought to regulate list prices of new medicines as low as possible despite several clear court rulings that limit its regulatory scope to protecting Canadians against excessive prices in the context of the time-limited market exclusivity offered by patent protection. The board also has offered no explanation as to why its proposed new benchmarks are the appropriate limits between non-excessive and excessive pricing.

- Significant market uncertainty: The most recent proposed guidelines offer patentees no clarity regarding what a compliant price might be and would give PMPRB staff almost unlimited discretion to determine when and how a product should be investigated and considered for a hearing recommendation. As a result, RAREi members believe the reforms sought by the board to date would contribute to substantial market uncertainty, effectively delay or prevent patient access to important new vaccines and therapeutics, undermine Canada's life sciences ecosystem and impose unreasonable new bureaucratic barriers to entry for all medication developers. The proposed changes would be particularly challenging for rare disease treatment innovators.

- Inconsistent with broader government objectives: The board's approach appears to be at odds with other federal government policies that appear designed to improve access to medicines and grow the life sciences sector in this country. In fact, when Minister Duclos announced the most recent amendments to the *Patented Medicines Regulations* in April 2022, he stated "the Government of Canada is committed to improving access

[1] Ward et al, *An international comparative analysis of public reimbursement of orphan drugs in Canadian provinces compared to European countries*. Orphanet Journal of Rare Diseases, Issue 17, Article 113, March 4, 2022): https://doi.org/10.1186/s13023-022-02260-6.

[2] Rawson, N, *Availability and Accessibility of Essential Drugs for Rare Disorders in Canada*. Canadian Health Policy, October 13 2021: https://doi.org/10.54194/HFEB4050 www.canadianhealthpolicy.com.

[3] Lech et al, Orphanet Journal of Rare Diseases – 08Oct22 - https://ojrd.biomedcentral.com/articles/10.1186/s13023-022-02534-z

to quality medicines for Canadians."[4] In addition, during the past few years, the government has undertaken consultations related to the development of a new pan-Canadian genomics strategy and launched both a national rare disease drug strategy and a national biomanufacturing and life sciences strategy. To be clear, none of these other federal initiatives will be optimized if the board's proposed new approach to price review is pursued as currently framed.

In an effort to help explain specifically how and why the approaches are so problematic for rare disease medicines, RAREi supported a number of peer-reviewed case studies..[5],[6],[7] Other research published last year shows the clear connection between public list prices and level of access to innovative medicines.[8]

Conclusion and Recommendations

In light of the above, **RAREi respectfully submits the following recommendations for adoption by the committee in its report:**

1. **Call on the PMPRB to address the above-noted considerations in any future guidelines proposal, specifically, to ensure that guidelines are consistent with the PMPRB's legislative and regulatory mandate, that they reduce uncertainty for rare disease developers who seek to research and provide medicines in Canada and that they align with and support broader government objectives related to the life sciences sector, rare disease policies, genomics strategies, etc.**
2. **Recommend that the PMPRB create a technical working group, comprised of medicine developers and other key stakeholders, to help inform the development of new guidelines, and undertake a case study approach to better understand how any draft price review processes would work in practice.**

This is an extremely exciting time for the rare disease community given the incredible scientific and technological advances underway. However, the benefits of many of these technologies will not reach Canadian patients in a timely manner if the PMPRB guidelines are not crafted carefully. That is why it is vital for the government and the PMPRB to find a better approach that would position Canada as an example to follow internationally in terms of providing timely access to rare diseases treatment and a place to pursue real innovation. In doing so, we hope that the PMPRB will work collaboratively with RAREi, industry associations and other health system stakeholders to find a better way forward

Thank you for your kind attention.

---

[4] Health Canada, *Statement from minister of health on the coming-into-force of the regulations amending the Patented Medicines Regulations*, April 14, 2022: https://www.canada.ca/en/health-canada/news/2022/04/statement-from-minister-of-health-on-the-coming-into-force-of-the-regulations-amending-the-patented-medicines-regulations.html.

[5] https://www.canadianhealthpolicy.com/product/new-patented-medicine-regulations-in-canada-case-study-of-a-manufacturer-s-decision-making-2/.

[6] https://www.canadianhealthpolicy.com/product/new-patented-medicine-regulations-in-canada-updated-case-study-en-fr-2/.

[7] https://www.canadianhealthpolicy.com/product/effect-of-amended-patented-medicine-regulations-on-industry-decisions-to-launch-new-drugs-in-canada/.

[8] Grootendorst P & Spicer O, *An empirical examination of the Patented Medicine Prices Review Board price control amendments on drug launches in Canada,* Working paper 200003, Canadian Centre for Health Economics, July 2020: https://www.canadiancentreforhealtheconomics.ca/wp-content/uploads/2020/08/Spicer-Grootendorst-2020.pdf.

About RAREi

RAREi is a network of biopharmaceutical companies dedicated to improving the lives of patients with rare diseases by developing and commercializing treatments. This network includes the following members: Alexion Pharma Canada Corp, Amicus Therapeutics Canada Inc, argenyx Canada, Astellas Canada, Biogen Canada Inc, BioMarin Pharmaceutical (Canada) Inc, Boehringer Ingelheim Canada Ltd., GlaxoSmithKline Canada, Horizon Therapeutics Canada, Ipsen Biopharmaceuticals Canada Inc, Janssen Canada, Mitsubishi Tanabe Pharma Canada Inc, Recordati Rare Diseases Canada Inc, Sanofi Genzyme, Sobi Canada, Inc, Ultragenyx Pharmaceutical Inc and Vertex Pharmaceuticals (Canada) Inc. For more information, please visit www.rarei.ca.