March 19, 2025

Patented Medicine Prices Review Board (PMPRB)
333 Laurier Avenue West, Suite 1400
Ottawa ON, K1P 1C1

Subject: PMPRB Guidelines consultation (2025)

Dear PMPRB Board Members,

The Canadian Organization for Rare Disorders (CORD) is the national alliance of over 100 rare disease patient organizations and representing approximately 3 million Canadians affected by rare diseases.

On behalf of our patients and families, we commend the PMPRB for taking a refreshed perspective in its latest proposed guidelines. We are particularly encouraged by the return to its core mandate. This new approach will help remove some of the previous barriers that discouraged the introduction of new therapies in Canada. This gives us hope that we can turn the page on what has been a difficult and frustrating chapter for CORD and the rare disease community.

However, while this is a step in the right direction, we remain concerned about how the revised guidelines will impact the overall availability and accessibility of rare disease treatments in Canada.

Rare disease patients face unique challenges in accessing new medicines. These treatments are highly specialized, developed for small patient populations, and require significant investment in research and development. Overly restrictive pricing measures can make it financially unviable for manufacturers to introduce these therapies in Canada, resulting in limited availability and delayed access.

The reality is already troubling. Between August 2019 and March 2025, only 57.6% of new medicines approved in the U.S. were authorized for use in Canada. Even more concerning, from the start of 2024 to now, only 14 out of 49 approved U.S. treatments became available to Canadian patients. Many of these missing therapies address serious and rare conditions, including refractory neuroblastoma, Duchenne muscular dystrophy, and other rare genetic disorders.

While the move to a clearer approach, including criteria for in-depth reviews is a positive step, it must be accompanied by additional measures to ensure that Canada remains an attractive and viable market for new drug launches. Without further action, patients with rare diseases will continue to face unnecessary delays and be left without access to critical, life-saving therapies.

In this context, as the PMPRB begins to reflect on its guidelines approach, we encourage you to consider the following key questions:

1. How will the PMPRB changes enable the implementation of Canada's rare disease drug strategy?
2. How will its new processes for annual and in-depth reviews impact Canada's attractiveness as a destination for new medicine launches and research?
3. Do the latest guidelines provide the requisite level of clarity and predictability to ensure developers can confidently launch their treatments in Canada?
4. How can the PMPRB align its pricing policies with international best practices to attract new therapies earlier? For instance, other countries, such as France, have implemented "zero-time" access models, ensuring that new therapies become available as soon as they receive regulatory approval.

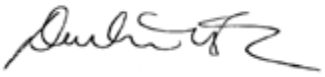
We also encourage the PMPRB to introduce a formal mechanism to monitor and evaluate the real-world impact of its new pricing system on patient access. Without a structured monitoring process, there is no way to determine whether the new pricing regime is helping or harming Canadian patients. The PMPRB must track key access metrics such as the number of new drug launches in Canada compared to international markets, the time-to-approval for new therapies, and the impact on rare disease treatments.

If data reveals that the new guidelines are discouraging pharmaceutical companies from bringing essential therapies to Canada, the PMPRB must be willing to make policy adjustments accordingly. A rigid framework that fails to adapt to real-world outcomes will only exacerbate the existing challenges in drug access.

By implementing a structured evaluation system, the PMPRB can ensure that its policies are meeting their intended objectives without creating additional barriers for patients.

Thank you once again for the opportunity to provide input.

Sincerely,



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