

July 18, 2022

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

Dear Members of the PMPRB,

The Canadian Organization for Rare Disorders (CORD) is the national voice for the over 3 million Canadians living with rare diseases.

Since the PMPRB changes were first proposed in 2017, CORD has actively engaged in preparing constructive feedback and recommendations, recognizing that the goal of delivering optimal patient and societal outcomes can only be achieved if Canada simultaneously pursues three objectives. The first is to assure timely individual access to the medicine best suited to that person's need. The second is to deliver value to public, private, and individual payers for investment in a therapy. And the third objective is provide sufficient return to researchers and developers to incentivize continuous innovation and development. The balance of these three drivers of the price of medicines is the "sweet spot" that a well-functioning Patented Medicines Pricing Regulations should facilitate.

Persons living with rare diseases are highly sensitive to these three imperatives; any imbalance means we don't get therapies. Rare diseases tend to be severe, progressive, and life-threatening. About 95% have no approved therapies, so a new rare disease drug is often the first effective treatment for that condition. Patients should have access as soon as possible. Rare disease therapies are often more complicated than medicines for common conditions. Because the number of patients that could benefit from a specific drug is small or very small, the budget impact is often minimal or even miniscule. However, because the development of these therapies can be very complex and there are few patients, the individual cost can be high, even very high. Moreover, the small patient populations and the severity of the conditions often require small, non-traditional clinical trials. Major regulators, including Health Canada, have been able to adapt review processes to accommodate these innovative trials but the health technology assessors and the payers have mostly clung to HTA and pricing models that have proven inadequate to these innovative medicines.

The changes proposed by PMPRB starting in 2017 have been singularly focused on one objective, that is, driving prices as low as possible, going far beyond even their original mandate of assuring prices were not excessive as an abuse of patent and in comparison to other countries. This approach would have had a humongous impact on Canadians living with rare diseases, not only denying access to new medicines but actually forestalling their entry to Canada, starting with the shutdown or slowdown of clinical trials.

The Canadian Organization for Rare Disorders heralded the federal government's announcement of a commitment of \$1 billion to set up a national Rare Disease Drug Strategy and realizes it is essential to use this opportunity to redesign the rare disease infrastructure to optimally support prescribing and monitoring of rare disease drugs. To achieve optimal benefit for the person and the health system, we must start with timely and accurate diagnosis so patients are identified as soon as possible and referred to an appropriate specialist who can make the diagnosis, prescribe the best therapies, and provide oversight for the on-going collection of data to assure the drugs are delivering

the expected outcomes. At the same time, the collection of “real-world” data provides the information to determine value and long-term right pricing.

To these ends, CORD’s recommendations for the PMPRB and the Rare Disease Drug Strategy are aligned. Moreover, as a result of CORD consultations through many webinars, forums, and conferences over the past three years, we have achieved the engagement and consensus of nearly all stakeholders in endorsing CORD’s Rare Disease Plan and proposals for the Rare Disease Drug Program. Like the rest of the developed world, Health Canada and increasingly the HTA agencies (CADTH and INESSS) have developed more agile and adaptive review processes. Thus, drugs with high promise of effectiveness for patient populations with unmet needs can be made available with minimal clinical trial data and appropriate patients are “managed” through programs collecting real-world outcomes data, including biomarkers, clinical outcomes, and quality of life impact.

Because of the potential catastrophic impact of implementation of the previously proposed PMPRB changes, CORD deemed it essential to take a role as intervenor in the cases before the federal and Quebec courts. We were very heartened by the court decisions leading to a policy change by Health Canada to remove the unwieldy and unsubstantiated economic factors from the proposed pricing regime. However, this removal of a potential barrier is not sufficient to ensure that patients with rare conditions will have appropriate and timely access to the (new) medicines available elsewhere in the world. We need innovative pathways that are compatible with the innovative therapies, including new targeted therapies that provide truly life-changing treatment and even cures for serious rare conditions but also more common conditions such as cancer, diabetes, and cardiovascular disease.

To these ends, the patient voice needs to be both present and heard. We hope that the PMPRB’s consultation on the new guidelines will be more meaningful and have a different tone and approach from what we have experienced in the past.

Specifically, the PMPRB should explore the impact of its pricing policies on patients’ lives and build policies to support treatments getting to Canadian patients. In particular, the approach taken must enable federal and provincial rare disease strategies. This will only be possible if the PMPRB reviews how the changes will operate in practice and with case studies, which was a key recommendation from CORD representatives in both the steering committee and technical working group for earlier guidelines modernization efforts.

We hope that these considerations will inform your plans for the interim and final guidelines in the coming weeks and months. Ultimately, we all need to do our part to help Canadian patients with rare diseases, too many of whom are literally in the fight for their lives.

Sincerely,



Durhane Wong-Rieger, PhD
President & CEO