Canadian Organization for Rare Disorders

Submission to the Patented Medicine Prices Review Board

Shaping the Future: A Discussion Guide for PMPRB Phase 2 Consultations on New Guidelines

September 2024

The Canadian Organization for Rare Disorders, on behalf of the approximately 3 million Canadians affected by one of hundreds/thousands of rare diseases, is pleased to submit this response to the Patented Medicine Prices Review Board (PMPRB) regarding its Phase 2 Consultations on New Guidelines.

The rationale for the existence of the PMPRB is to ensure that Canadians have the best possible access to the best available therapies. If all Canada wants is to spend as little money as possible, it would be simplest to just say “no” to most new drugs, regardless of their value, and not use the pretext of pricing guidelines. hen the PMPRB adopts guidelines that have an ultimate “implicit or explicit” objective of spending as little as possible on procuring therapies, it not only fails to achieve the goal of optimizing patient benefit, it also fails to achieve the mandate of incentivizing investment in pharmaceutical research in Canada. If Canada chooses to participate in the drug ecosystem primarily as a consumer or buyer without concomitant revenue streams derived from sale of knowledge, intellectual property, data, or goods, it cannot optimize the return on investment of drug procurement. Over the past couple of decades, even in advance of the revisions, the PMPRB has narrowed to nearly a singular operational focus, that is, driving new drug prices as low as possible.

Moreover, the proposed new guidelines will significantly negatively impact patient access to life-altering and life-saving medicines for serious chronic conditions, such as cancer, heart disease and diabetes. For patients with rare conditions delayed or few innovative therapies will be disastrous. If we continue our current access pattern, Canadian patients will eventually have access to about 60% of the breakthrough innovative and advanced therapies available in the USA, Europe, or other developed countries, but most will come six months to several years later and sometimes with a narrower indication. Rare disease patients cannot wait!

After two decades of advocacy, Canadians with rare diseases, got the first glimmer of hope in 2019 when the federal government announced $1.5 billion commitment to a national Rare Disease Drug Strategy, Following four more years of advocacy and consultation, , the federal government finally announced that $1.4 billion of the monies would be allocated through bilateral agreements between the federal government and provincial or territorial governments. CORD is encouraged by a recent milestone The good news is that the federal government signed the first bilateral agreement with British Colombia on July 23, 2024, obliging the province to spend 50% of the funding on at least one of 12 “common” rare therapies. The bad news is that the agreement requires the province or territory to fund minimally only one of the 12 “common” rare disease therapies over the first two years of the agreement.

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Delayed access means higher net costs!

From the outset, CORD has advocated for the Drug Strategy funding be spent not only on purchase of drugs but invested in sustainable Rare Disease Strategy. But, as usual, CORD is not just advocating but collaborating as a partner to mobilize action toward desired outcomes. To those ends, we were thrilled to announce on this year's Rare Disease Day, February 29, 2024, the launch of the Canadian Rare Disease Network (CRDN), a pan-Canadian, cross-disciplinary network that unites the country's leading clinical, scientific and patient expertise to address the unique challenges faced by Canadians affected by rare diseases and to advance rare disease care, research, and innovation both in Canada and worldwide.

Great progress is being made, but great challenges remain to fulfill the promise and the potential of the investment in Canada's Rare Disease Drug Strategy. Our goals are aspirational but achievable, namely, thatCanadians living with rare diseases will have access to all advanced, life-altering, and life-saving therapies at the same time as patients in the USA and other developed countries. The goal of "zero-time" to access (or minus zero as in France) requires all key components along the access pathway are prepared to implement real-world managed patient access at the time of (or prior to) regulatory and CDA assent with mechanisms for diagnosis, specialist care, and real-world data collection, synthesis, and analysis. The countries which can offer the shortest time to launch benefits from increased investment in research, clinical trials, managed access schemes, and demonstrated value assessment.

But Canada has a long way to go not just to achieve parity but to become a leader in the rare disease ecosystem. The chart below compares the number of new treatments authorized for sale in Canada versus the US since January 2020. As can be seen, just half (51.5%) of the 200 new medicines approved in the U.S. over this period (up to August 2024) were approved in Canada for use by Canadian patients.

Of special note, of the 20 new drugs approved in the U.S. so far this year, only one has been approved by Health Canada! Treatments not coming to Canada include those for serious and/or rare disorders including high-risk refractory or relapsed neuroblastoma, HER2+ breast cancer, rare conditions related to premature aging, Duchenne muscular dystrophy, multiple myeloma and Parkinson's disease.

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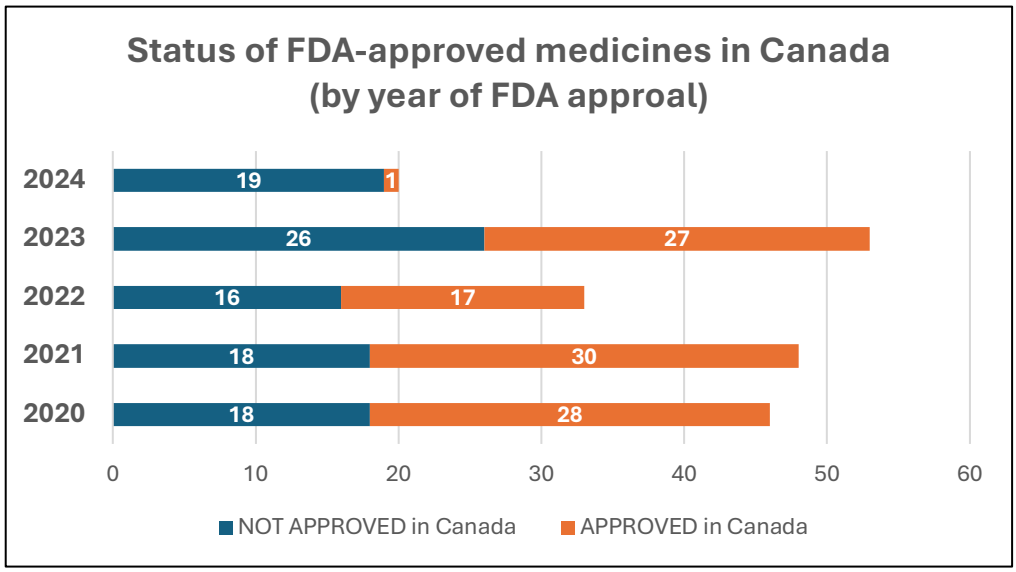

% approved in Canada Aug. 2024
5.0%
50.9%
51.5%
62.5%
60.9%
Total 2020-24 51.5%

Data from U.S. Food and Drug Administration and Health Canada Notice of Compliance database.

The PMPRB can play a significant role toward early submission of new treatments and timely approval by Health Canada relative to the USA and other countries. The PMPRB is key to creating an environment that encourages companies to bring their treatments to Canada as early as possible. To that end, CORD brings to attention of the PMPRB the following considerations in the context of the latest Discussion Guide:

1. Consultations vs Dialogue
2. Lack of environmental context
3. A transparent and predictable process

1. Consultations vs Dialogue

CORD continues to be extremely disappointed that the PMPRB continues to engage in consultations rather than meaningful dialogue with stakeholders.

We note three important elements missing from the PMPRB consultation process that are vital to ensuring effective dialogue:

a) **Case studies:** CORD has regularly asked the PMPRB to produce case studies *with real numbers* to illustrate how its proposed evaluation and decision-making processes will work. This is the only real way for people to see the concrete impact of the complex processes and formulas proposed in the PMPRB's new Guidelines. We fail to understand why such a seemingly simple request has gone unheeded for so long.

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b) **Working groups:** Similarly, we have asked numerous times for working groups of different stakeholders, including patients, be established to work through potential issues and challenges together.

c) **Real interchange via meetings:** Similarly, we urge the PMPRB to have constructive one-on-one or small group meetings with different stakeholders to truly learn their issues and concerns. CORD again requested such a meeting during this round of consultations but was denied. The regular biannual meetings of the Chair of the PMPRB with certain stakeholders, while useful to discuss ongoing issues, are not an adequate replacement for meetings specifically related to the subject under a specific consultation process such as this one.

Without interactive dialogue, the PMPRB does not benefit from fulsome expertise and experience of stakeholders and we do not benefit from the ensuring patient and community needs are met.

2. Lack of environmental context

The second major area lacking in the PMPRB consultation context is in its regulatory processes.

CORD, among many others, took time, effort and input from our stakeholders to provide important comments and proposals in December 2023, yet none of what we provided appears to be reflected in the updated Discussion Paper and the PMPRB seems only to want to focus narrowly on the four factors related to its mandate as stated in Section 85 of *The Patent Act*, with other issues being deemed irrelevant.

The lack of patient engagement within the PMPRB stands in sharp contrast to the inclusion of patients in other entities and initiatives. For example, in the Rare Disease Drug Strategy patient involvement is positioned as an important core component. Similarly, Canada's Drug Agency (CDA) has a long history of patient engagement at many levels throughout its operations and is currently bringing a patient representative onto its board of directors. We appreciate that the process of patient engagement may be gradual for the PMPRB but we are willing to be helpful and "patient."

3. A transparent and predictable process

CORD and our members support ongoing efforts to improve medicine affordability. However, this cannot be at the expense of disincentivizing new drugs from coming to Canada. It is crucial that the PMPRB develops predictable and transparent processes that can help support new medicine launches in Canada. This will help protect consumers and our health system from excessive prices but also, crucially, without inhibiting drugmakers from choosing to bring their new treatments to Canada to benefit Canadian patients – particularly those with rare diseases.

Importantly, it is crucial for measures to be put in place to measure the impact of the new PMPRB Guidelines on access to medicines by Canadians, such as evaluating and reacting to address issues raised by data such as the failure for many new medicines to be submitted for

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approval in Canada, as noted above. In 2021, the PMPRB put forward its proposed Guideline Monitoring and Evaluation Plan but it would appear this effort has been abandoned. It is crucial that both the PMPRB and patients be able to track the impact of PMPRB activity to ensure we are not creating additional barriers to access by Canadians to the medicines they desperately need.

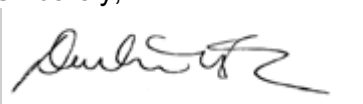
CONCLUSION

I've travelled across the globe – including on a trade mission to Asia – and everywhere I have gone, leaders in the sector were wondering and asking me, "What is happening in Canada?" with specific reference to the PMPRB reforms. We are a G7 country so should be leading the way in helping our patients access the latest medical innovations.

But we are not. Canadian patients are not receiving the treatments they deserve when they deserve them because Canadian policies, including those of the PMPRB, are not working to serve them.

We ask the PMPRB to put first and foremost in everything it does this simple question: "How will this action benefit Canadian patients?"

Sincerely,



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