



Written Submission to the PMPRB Draft Guidelines Consultation

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Introduction

Our country's healthcare system is, for many Canadians, a source of national pride. Affordable, equitable and timely access to therapies is something that Canadians expect. Given the PMPRB's mandate to ensure the prices of patented medicines sold in Canada are not excessive, it follows that any efforts undertaken by the PMPRB to make medicines more affordable for Canadians should be supported, at least in principle. We understand that the revised PMPRB guidelines have been developed with this goal in mind, and we appreciate the opportunity to provide feedback as well as the additional effort that has gone into updating them. However, our review of the revised guidelines in order to provide meaningful feedback on them has been a challenging undertaking. The cost of medicines is an issue of great importance and relevance to Canadians. Yet, the revised guidelines document, which could have significant implications for how medicines are priced in the future, is in itself a barrier to meaningful dialogue and feedback because the language used is too technical and complex to be well understood by the Canadians it aims to protect.

At the same time, we are aware of numerous developments that have created a murky environment in which to implement the revised PMPRB guidelines. For example:

- On June 29, 2020 the Federal Court ruled on recent amendments to the PMPRB based on a judicial review requested by Innovative Medicines Canada. The ruling was a split decision, with the court upholding two amendments, while finding another outside of the scope of the *Patent Act*. A second court proceeding on the regulations, before the Quebec Superior Court, is scheduled to be heard in September 2020. Yet the revised PMPRB guidelines are scheduled to come into effect approximately three months later on January 1, 2021.
- As stated by PMPRB staff at least twice during their July 8, 2020 webinar, the guideline reforms are needed because Canada does not have a Pharmacare plan that will pay for drugs accessed outside of a hospital setting. Yet within the 2019 Budget, the federal government proposed a significant investment in national Pharmacare and announced the development of three pillars foundational to its implementation (the Canadian Drug Agency, a national formulary and a national strategy for high-cost drugs for rare disease).

Ultimately, we believe the guidelines implemented by PMPRB must result in fair prices for patented medicines. While we do not have the expertise or mandate to determine whether a drug's price is too high or too low, the model used to determine pricing must balance affordability with access. There are dozens of ALS therapies currently in clinical trials worldwide and every Canadian now living with ALS – and every Canadian diagnosed today, tomorrow and in the future – needs the ability to access these therapies as soon as possible. We do not want to see a future where regulatory bodies in other countries are approving therapies, but pharmaceutical companies elect not to come to Canada. They must see Canada as a viable market to run clinical trials and pursue regulatory approval.

Efforts intended to lower prices must be made in a balanced way that continues to encourage innovation and does not result in the delay of launches and introduction of new medicines in the Canadian market. Any changes to Canada's drug access pathway – including the PMPRB – must be flexible and responsive to the needs of rare diseases, such as ALS, and encourage more proven therapies to be developed, sold, and reimbursed in Canada. It is essential that tomorrow's proven therapies be accessible to all Canadians in a timely, equitable and affordable way.

Recommendations

Recommendation #1: That the Federal Government require PMPRB to engage an independent third party to conduct a formal assessment of the real-time and potential impacts of the reforms on access to therapies and research investment in Canada (including clinical trials), with specific consideration to therapies for rare diseases, before the PMPRB guidelines are implemented.

It is well established that clear and understandable frameworks are essential for manufacturers to bring their therapies to market, especially small companies within the rare disease space. Without this clarity, new drug launches in Canada could be delayed until well after the therapy is available in other countries, or may not be launched here at all.

As such, with great uncertainty around the real-world application of these proposed reforms, we cannot meaningfully comment on the impact the June 2020 Draft Guidelines could have on the availability of therapies in Canada, especially those within the rare disease space. Furthermore, without case study validation of the guidelines, we cannot assess how Canadians, including those living with ALS, will ultimately be affected.

Recommendation #2: That the PMPRB undertake a phased approach to enacting its proposed reforms in order to assess the impact of each change on research investment and access to therapies for both rare and non-rare disease therapies. Only then should additional reforms be considered.

The PMPRB guidelines propose multiple measures to achieve the goal of significantly reducing the price of drugs in Canada. However, the real-time implications of those measures on access to therapies and research investment in Canada remains tremendously uncertain – especially given the inclusion of new and untried measures to reduce prices through reliance on factors such as pharmacoeconomics.

For example, it estimated that changing the basket of comparator countries used by the PMPRB will represent a substantial price drop of at least 20% for new therapies. Yet, how that initial decrease in price will affect whether companies choose to pursue clinical trials or regulatory approval in Canada is not fully understood – at all.

A number of early studies indicate that there is a strong negative correlation between drug price controls and investment in R&D and the availability of new medicines¹. According to Life Sciences Ontario, 21 out of 37 new active substances launched globally in 2018 were not launched in Canada, a number of which were in the rare disease space. Additionally, the data demonstrates that in 2019 there was a significant decrease in the number of new launches in Canada compared to global trends².

We must be able to fully realize the impact of each proposed change before implementing further measures. Taking an incremental approach is crucial to making pharmaceutical policy changes that balance access and affordability, in the best interest of Canadians as both taxpayers and patients.

Recommendation #3: That the PMPRB implements a distinct pathway for medicines for rare diseases.

While a number of the adjustments in the June 2020 Draft Guidelines attempt to address the inherent disadvantages of therapies for diseases that have smaller populations, the guidelines do not go far enough. The high cost and small market size of rare disease therapies means that the majority will exceed the proposed thresholds and be subject to pharmacoeconomic assessments, which could be detrimental for future ALS therapies. In addition, the use of the pharmacoeconomic assessments as

¹ Labrie, Yanick. Evidence that regulating pharmaceutical prices negatively affects R&D and access to new medicines. [Canadian Health Policy Journal. \(2020\).](#)

² Life Sciences Ontario. New Medicine Launches: Canada in a Global Context. (2020) [Slides 15 to 17.](#)

proposed lengthen an already complicated process and do not take into consideration metrics relevant to patients.

A distinct pathway for rare disease therapies must be implemented so that the complexities specific to drugs in this space are considered in a meaningful way.

Recommendation #4: That the Federal Government require that PMPRB decision-making and processes include patient representatives.

The time is long overdue for PMPRB, along with other appropriate agencies, to establish a formal mechanism for meaningfully and continuously engaging patient representatives in its decision-making and processes. It's time to ensure the patient voice, choice and representation at PMPRB.

Conclusion

Canadians are both taxpayers and patients, and therefore have an interest in affordability as well as accessibility of medications. Any efforts made to reform how patented medicines are priced in Canada must balance both. There is room for the 2020 Draft Guidelines as proposed to do a better job of this, particularly for diseases with smaller populations like ALS who are at a natural disadvantage for gaining access to clinical trials and approved therapies.