PULMONARY HYPERTENSION ASSOCIATION OF CANADA

L'ASSOCIATION D'HYPERTENSION PULMONAIRE DU CANADA

September 11, 2024

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

Via Online Submission Form

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

Dear Members of the PMPRB,

Since 2020, the Pulmonary Hypertension Association of Canada (PHA Canada) has submitted to the PMPRB on behalf of Canadians affected by pulmonary hypertension (PH), a serious, progressive, and life-threatening condition characterized by high blood pressure in the lungs.

Fewer than 4,000 Canadians have pulmonary arterial hypertension (PAH), a rare, clinically distinct subtype of PH. Patients with PAH experience barriers at every stage of their journey: an accurate diagnosis often takes months or years; PH expert centres are located in urban centres and require regular visits for optimal care, often necessitating significant travel; and, despite 11 approved PAH-specific medicines so far, median survival after a PAH diagnosis has plateaued at only approximately five years[1]. For PAH patients and many other patients with rare diseases, access to new, innovative medicines and enhanced access to existing medicines is essential for improving their health outcomes and quality of life.

The following comments on the Discussion Guide focus on our primary objective as a patient organization: improving PH patient outcomes through access to high-quality care and support. We generally favour transparent guidelines that reduce the risk of new medicines not coming to Canada. Importantly, we also value a process that provides patients with regular opportunities to collaborate with the PMPRB, especially when major changes are considered.

Topic 1: Price level within the PMPRB11 to be used in the initial and post-initial price review

⇒ We prefer *Option 2: HIP*

The countries in the PMPRB11 were chosen to be suitable comparators for Canada. Thus, the highest price paid by any of the PMBRB11 countries should still be considered acceptable in Canada. The fact that the PMPRB's example to demonstrate the case against HIP dates back to 2008 further suggests that HIP is a reasonable comparator.

The MIP is highly unpredictable, and the midpoint between the MIP and HIP is unpredictable and arbitrary.

[1] Weatherald et al. The evolving landscape of pulmonary arterial hypertension clinical trials. Lancet 2022; 400; 1884-98

Topic 2: The length of time Staff should wait, following the implementation of the Guidelines, to determine whether the IPC identification criterion for an Existing medicine is met

⇒ We prefer *Option 2: two years*

We do not offer a strong recommendation on this point other than to support a reasonable timeframe for Rights Holders to adapt to the grandfathering of existing medicines. We believe Option 2 provides Rights Holders a reasonable, but not overlong, adjustment period.

Topic 3: In-depth review based on CPI increase criteria

⇒ We prefer *Option 1: if the list price increase is above one-year CPI*

We do not offer a strong recommendation on this point; however, we believe Option 1 likely ensures greater predictability and transparency.

Topic 4: The individuals/groups permitted to submit a complaint

⇒ We prefer *Option 2B: limit complaints to option 1 above plus private and public payors*

We do not offer a strong recommendation on this point other than to support a balanced process. We believe Option 2B is neither too broad (leading to excessive complaints and causing a time-consuming bottleneck) nor too narrow (leading to an inability to present valid complaints).

Topic 5: Expanding the list of products that would only be subject to an in-depth review following a complaint to include biosimilars and/or vaccines.

⇒ We prefer *Option 2: The PMPRB will only open an in-depth review for biosimilars and/or vaccines when a complaint is received.*

We believe this practice serves the Board's efficiency goals.

Topic 6: Use of clinical evidence to contextualize the degree of similarity of comparators identified for the TCC

⇒ We prefer *Option 2: each comparator will be assigned a level of similarity.*

Each comparator should be assessed independently. PH is a complex condition in which patients may respond differently to various treatment options. Older (often generic) medicines may skew an assessment of a comparator group without accounting for the added value of newer, more expensive medications.

We also strongly suggest that Rights Holders be engaged earlier in the review process, whichever option is chosen. For example, Rights Holders could be requested to identify (and justify) potential comparators as part of their initial submission instead of as optional input after the Scientific Review. This would increase transparency and might also improve process efficiency.

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Topic 7: Future role of Human Drug Advisory Panel (HDAP)

⇒ We prefer *Option 1: HDAP will be used only on an ad hoc basis when deemed necessary by Staff.*

Despite the need for efficiency, we believe that PMPRB staff should be able to seek additional clinical expertise on medicines, especially for rare diseases.

If the PMPRB's scientific and pricing teams have always operated independently, what was the original purpose of the HDAP? More importantly, what has changed about the PMPRB's operations such that the HDAP might no longer be necessary? We feel these are important questions to be answered if the PMPRB is going to move away entirely from seeking clinical expertise when necessary.

Conclusion

PHA Canada encourages the PMPRB to consider the draft guidelines through the lens of PAH patients and other rare disease patients, for whom timely access to the most appropriate, most effective medicines—whether new or existing—is critical. To do this, the PMPRB must continue to engage in meaningful consultations with patient organizations/advocates. As such, we strongly believe that embedding patient engagement into the PMPRB should be mandatory. At a minimum, this should include biannual meetings with patient groups to share updates and receive concerns (as previously committed to), but could also include options such as adding at least one patient representative to the PMPRB Board.

Thank you for the opportunity to comment on this Discussion Guide. We hope the PMPRB's new guidelines will offer patients hope for a brighter and healthier future.

Sincerely,

Joan Paulin
Chair, Board of Directors

Jamie Myrah
Executive Director

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Background

Pulmonary arterial hypertension (PAH) is a rare and very complex lung disease which is progressive and potentially fatal. Like all types of PH, PAH is defined by high blood pressure in the lungs, which leads to enlargement and weakness of the right side of the heart—a serious type of heart failure. PAH can strike anyone regardless of age, sex, or social/ethnic background. While there is currently no cure for PAH, many patients are living longer, healthier lives thanks to available treatments.

PHA Canada is a federally registered charity whose mission is to empower the Canadian pulmonary hypertension community through support, education, advocacy, awareness, and research. Since 2008, PHA Canada has brought together pulmonary hypertension patients, caregivers, and healthcare professionals to better the lives of Canadians affected by PH and represent a united national PH community.