



PULMONARY HYPERTENSION
ASSOCIATION OF CANADA

L'ASSOCIATION D'HYPERTENSION
PULMONAIRE DU CANADA

December 20, 2023

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

Via Online Submission Form

Re: 2023 Scoping Paper on Themes for New Guidelines

Dear Members of the PMPRB,

The Pulmonary Hypertension Association of Canada (PHA Canada) represents a complex patient population that faces significant [physical, social, and economic burdens of illness](#). Pulmonary hypertension (PH) is a serious and life-threatening condition characterized by high blood pressure in the pulmonary arteries. People living with PH experience symptoms such as breathlessness, fatigue, swelling of the extremities, chest pains, dizziness, and fainting. PH often strains the right side of the heart, specifically the right ventricle (RV). If left untreated, PH can lead to RV failure, a serious type of heart failure. PH affects an estimated 1% of the global population and up to 10% of the cohort over 65 years¹, yet there are currently no targeted therapies available for most patients.

Pulmonary arterial hypertension (PAH) is a rare but clinically significant subtype of PH that affects less than 10,000 Canadians. These patients, like millions of other rare disease patients, experience significant delays in receiving an accurate diagnosis and a profound lack of access to appropriate therapies to manage their condition optimally. Despite there being 11 PAH-specific medicines approved in Canada, patients with PAH have a poor prognosis, with median survival after diagnosis of approximately five years². Access to new and innovative treatment options is necessary to advance health outcomes and quality of life for people living with PAH.

Since 2020, PHA Canada has submitted to the PMPRB on behalf of all Canadians affected by PH, urging the Board to consider the potential negative impacts of its new regulations/guidelines on patients, especially those with rare diseases. In our last submission, we urged the Board to refrain from implementing the proposed Guidelines and to work through a multistakeholder process to develop a policy that balances the need to protect consumers with the need to alleviate the suffering of patients. Therefore, we are pleased by the Board's decision to reconsider the Guidelines and continue consultations with all stakeholders, including those with lived experiences. The following comments reflect PHA Canada's principal interest in improving the health outcomes of PH patients and the limitations of our expertise and capacity as a patient organization.

Theme 1: Efficient Monitoring of Prices without Price Setting

PHA Canada lacks the technical expertise to answer the questions about this theme adequately. As such, we endorse the calls made during the policy roundtable held on December 5, 2023, for a multistakeholder "technical working group" to address these questions in the context of s. 85 of the Patent Act and assist in the design of the new Guidelines. Such a group should include patient representatives, and the PMPRB

¹ Hoepfer et al. *A global view of pulmonary hypertension*. *Lancet Respir Med*. 2016; Apr;4(4):306-22.

² Weatherald et al. *The evolving landscape of pulmonary arterial hypertension clinical trials*. *Lancet* 2022; 400; 1884-98



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should provide those patient representatives with plain language information/briefings to support their meaningful participation in the working group. Patient representatives should also be allowed to consult with other patient organizations and coalitions (e.g., COD, Best Medicines Coalition, Health Charities Coalition of Canada) during their work to ensure continuous feedback from people with lived experience.

The PMPRB should study the impacts of the new regulations/guidelines on patients to monitor for adverse effects on the availability of new therapies and clinical trials in Canada. PHA Canada continues to endorse the position of the Best Medicines Coalition that "the federal government ensures a truly independent evaluation of the impact of its Patent Act regulations by engaging a third-party entity to undertake fulsome consultations with all stakeholders to develop and ultimately implement a monitoring and evaluation regime for the PMPRB-related regulations." Implementation of a monitoring and evaluation regime should also be overseen by a multi-stakeholder advisory group that includes patient representatives.

Theme 2: Transition to PMPRB11 – New versus Existing Medicines

PHA Canada supports distinguishing between medicines that existed as of July 2022 and those introduced afterwards and "grandfathering" the existing medicines under the old regime. The assessment of all new and existing medicines under the new PMPRB11 would introduce significant uncertainty into the landscape, potentially threatening patient access to treatment or services through patient support programs funded by drug manufacturers.

Theme 3: Price Reviews During Product Life Cycle

PHA Canada supports aligning pricing reviews with the ongoing work of CADTH and INESSS. The approval of new indications, availability of new evidence, and departures from identified pricing thresholds are all reasonable circumstances to trigger a pricing review. However, the PMPRB must coordinate/streamline its processes to reduce delays in patients receiving appropriate treatment.

Theme 4: Investigations and Referral to Hearing

PHA Canada supports the continued use of Voluntary Undertakings as an investigative closure mechanism. The PMPRB must be fully transparent about the criteria for commencing investigations and the steps taken during an investigation, including providing specific examples or test cases to demonstrate the functional application of the requirements and the investigation process.

Theme 5: Relation to pan-Canadian Health Partners, Insurers (Private and Public); and Alignment with Broader Government Initiatives

PHA Canada fully supports all efforts to coordinate decisions and timelines of the PMPRB with other pan-Canadian partners and insurers. The PMPRB should reduce duplication of efforts and leverage the assessments of Canada's health technology agencies, including those that incorporate real-world evidence and time-limited decision-making.



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Theme 6: Engaging with Patients, Health Practitioners, Pharmacy, and other Stakeholders

The PAH community has direct experience with the life-changing impacts of innovative medicines. Until 1997, there was no treatment available for PAH, and mean survival after diagnosis was only 2-3 years for patients with idiopathic PAH. While survival after a PAH diagnosis has improved since 1997 (increasing to a medium of five years), long-term survival rates have not improved despite the increased use of combination therapy in Canada since 2015³. This high mortality risk demonstrates the urgent need for innovative medicines that target alternative pathways and provide significant improvements for PAH patients.

People with lived experience must play a central role in determining what constitutes "significant improvement" or therapeutic benefit. Patient priorities and preferences should inform the problems research seeks to address and be fundamental in determining whether the innovation meets the needs of patients. Value-based decision-making requires that patients be engaged at every decision-making stage, including by the PMPRB. It is why PHA Canada urges the PMPRB to engage a technical working group, including patient representatives, to lead the development of the new Guidelines and ensure that the implementation of the Guidelines remains focused on improving patient health outcomes.

Access to innovative medicines is essential for improving the health outcomes of rare disease patients and others with significant unmet needs. That is why PHA Canada will continue to urge the PMPRB to adopt Guidelines that will preserve access to new and existing medicines in Canada.

Sincerely,

Jamie Myrah
Executive Director

Background

Pulmonary hypertension (PH) is a rare and very complex lung disease which is progressive and potentially fatal. PH 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. PH can strike anyone regardless of age, sex, or social/ethnic background. While there is currently no cure for PH, some 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.

³ Zelt et al. *Mortality trends in pulmonary arterial hypertension in Canada: a temporal analysis of survival per ESC/ERS guideline era.* Eur Respir J 2022; 59: 2101552