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Patented Medicine Prices Review Board
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Input Regarding PMPRB Draft Guidelines

Introduction

The Best Medicines Coalition (BMC) is a national alliance of 27 patient organizations, together representing millions of Canadian patients, with a shared goal of equitable, consistent and timely access for all Canadians to safe and effective medicines that improve patient outcomes. Areas of interest include drug approval, assessment and reimbursement along with patient safety and supply concerns. The Coalition strives to ensure that Canadian patients have a voice and are meaningful participants in health policy development, specifically related to pharmaceutical care.

As part of our efforts on behalf of patients, we welcome this opportunity to comment on the Patented Medicine Prices Review Board's (PMPRB) Draft Guidelines to implement the Patented Medicines Regulations, as amended. This follows previous input regarding proposed reforms, provided February 2018 and June 2017, and October 2016 in response to Health Canada's *PMPRB Guidelines Modernization Discussion Paper*.

In addition, a BMC representative participated on the PMPRB Steering Committee, and formal input was provided regarding committee deliberations and process issues. Most recently, several representatives of BMC member organizations attended the PMPRB's briefing and consultative session for civil society organizations in December 2019.

As with previous submissions, this consensus document has been developed with the participation of BMC member organizations, each of whom has had the opportunity to review content and provide input. Statements and positions expressed within this submission to the PMPRB reflect areas of agreement among BMC member organizations.

Pricing Regulation: Core Positions

As the Draft Guidelines are reviewed, we ask that the BMC's previously provided core positions regarding reform and modernization of Canada's pharmaceutical pricing regulatory framework be considered carefully. These principle-based positions remain focused on those aspects directly related to patient interests and needs, as follows:

- ***Balanced Oversight.*** The BMC supports a strong, balanced and fair regulatory framework for pharmaceutical pricing aimed at sustaining the life, health and wellbeing of patients. Serving both patients and payers, this framework should support sustainability of the health care system while also contributing to an environment which facilitates access to new medications to meet unmet patient needs.

- ***Availability.*** A primary goal must be to enable the introduction and availability of a comprehensive range of medicines, including newly developed advancements to address unmet needs, and to clinical trials which provide patients with early access to promising new treatments.

- ***Timely Access.*** The ability to access necessary medicines in a timely manner is critical to protecting and optimizing the health and wellbeing of Canadians. The pricing framework and Guidelines must respect this premise and facilitate, rather than deter, early introductions. Furthermore, the review process must be timely, efficient and free of duplication and overlapping administration. Patients must be spared extended wait times to access medicines that will restore or improve their health.

PMPRB Draft Guidelines: Issue Discussion

We recognize the complexity of pharmaceutical pricing and its diverse implications, including industry profitability and downstream impact on the healthcare system, patient care and outcomes, and therefore warrant full and careful consideration.

While supportive of protection from excessive pricing – especially for out-of-pocket costs for patients -- we have concerns related to how the reforms outlined in the Draft Guidelines will impact future patient access to needed medications. As patient organizations, our primary position is that, at a minimum, the quality of care that is currently available to patients must be first preserved and then enhanced.

We understand that drug developers/patentees have fundamental concerns that the changes may inhibit early introduction of new advances. Specific elements identified as problematic include the application of new economic factors, criteria for reassessments, thresholds for assessments, grandparenting provisions, and restrictive price setting impacting patients with rare diseases. Drug developers/patentees are the ultimate decision makers on whether and when drugs will be introduced in Canada, and the location of clinical trials. Therefore, we urge the PMPRB to consider their input during these consultations, on level with positions from other stakeholders, and to work towards ameliorating issues and developing the best possible workable solutions for optimal patient care and access. If not possible within the set timelines, these dates must be revised, and implementation of regulations delayed.

Moving Forward: Monitoring and Evaluation

The BMC urges comprehensive post-implementation surveillance, including ongoing monitoring and evaluation. We understand that a strategy will be outlined in a revised *Guidelines Modernization and Evaluation Process,* and look forward to learning more.

We support an evaluation process which is broad in scope and rigorous. Building on the areas outlined in the Draft Guidelines background document provided, we request the incorporation of metrics specifically focussed on patient care outcomes.

Monitoring and evaluation processes must examine the impact on what drugs are and are not made available to Canadian patients, including timing, and in comparison to other countries and previous drug launch rates in Canada prior to the application of the new regulations. Evaluation must include analysis of real savings and subsequent investments, including the health system costs if access to new breakthrough drugs is delayed or prevented. Importantly, there must be mechanisms in place to incorporate adjustments if patient care has been negatively impacted within the new framework and Guidelines.

The monitoring and evaluation processes must address these fundamental questions:

- What has been the impact on the range of drugs made available, compared to previous levels of Canadian new drug introductions and other countries, the timing of introductions, and the number and types of clinical trials conducted in Canada?
- Do the new regulatory framework and Guidelines reduce duplication, improve efficiency, and contribute health care system sustainability?
- Is the new regulatory framework flexible enough to ensure new medications to meet unmet needs are expedited?
- Does the new framework contribute to improved patient care and outcomes and, if so, to what extent?

These monitoring and evaluation processes must encompass high standards of transparency and accountability, with thorough reporting. All stakeholders, including patient communities, should be consulted on their design and be involved in their application.

Moving Forward: Engagement and Decision-Making Participation

Patients have an important role in health policy development, and all public bodies must implement processes for meaningful participation and integration of patient values and perspectives. The PMPRB is no exception and, therefore, it is essential that improving engagement must be taken on as a core priority with the objective of ensuring the PMPRB's work is accountable and aimed at improving patient care and outcomes.

We urge the PMPRB to take a holistic, collaborative and values-based approach to patient engagement, committing to and establishing processes for communication, meaningful consultation and decision-making participation. Regarding governance, we support both patient representation on the Board and the establishment of a formal Board advisory body to incorporate a range of patient voices. We believe that patients have a role in strategic planning, policy development and prioritization. Processes for patients and/or patient organization input to specific pharmaceutical reviews should be developed and implemented. Importantly, patients should be involved in establishing and participating in monitoring and evaluation processes, starting with the *Guidelines Modernization and Evaluation Process*. As working groups are established, appropriate patient involvement must be facilitated with appropriate support provided.

We urge the PMPRB to institute ongoing information sharing and education. This can take several forms (such as webinars or events, for example the December 2019 civil society briefing), providing an opportunity for the PMPRB to share and receive perspectives.

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Summary: Working Together

To patients who rely on medications to maintain health and life, the availability of current drugs and the hope that future medications to treat unmet needs will be introduced in Canada are paramount. We ask you to consider our recommendations, as summarized below:

- Fully integrate fundamental patient-driven principles regarding balanced oversight, availability and timely access to current and new medications as you seek to balance consumer protection and affordability with maintaining an environment which is commercially viable.
- Carefully consider and integrate positions and recommendations from patients and work closely with manufacturers/patentees, on level with all stakeholders, to develop workable solutions that factor in varied considerations such as cost containment and patient outcomes.
- Develop and implement rigorous monitoring and evaluation processes with specific metrics focussed on patient care outcomes, while incorporating high standards of transparency and accountability.
- Adopt a holistic approach to patient engagement encompassing governance, strategic planning, policy development, prioritization, education and outreach, with processes to facilitate input to specific pharmaceutical reviews.

The patient community, including the BMC and its member organizations, welcomes any opportunity to consult and assist as the Government of Canada, through Health Canada and the PMPRB, pursues development of a pharmaceutical pricing regulatory framework which serves the needs of all.

BEST MEDICINES COALITION

About the Best Medicines Coalition

The Best Medicines Coalition is a national alliance of patient organizations, together representing millions of Canadian patients, with a shared mission of equitable and consistent access for all Canadians to safe and effective medicines that improve patient outcomes. Areas of interest include drug approval, assessment and reimbursement issues, as well as patient safety and supply concerns. The BMC strives to ensure that Canadian patients have a voice and are meaningful participants in health policy development, specifically related to pharmaceutical care. The BMC's standing goals are as follows:

- Drug programs which deliver high standards of equitable, consistent and timely access to medications for all Canadians.
- Drug review and post-marketing surveillance systems to address patient safety; knowledge of risks and benefits throughout drug lifecycle.
- Effective models for meaningful and equitable patient participation in drug reviews and policy development.

Through issue education and consensus development, patient-driven positions are communicated to decision makers and stakeholders. Formed in 2002 as a grassroots alliance, the BMC was registered under the Not-for-profit Corporations Act in 2012 and is governed by a Board of Directors elected from member organizations.

Best Medicines Coalition Members