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Patented Medicines Regulations Consultations
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Input Regarding Proposed Amendments to the Patented Medicines Regulations

Introduction:

As part of our efforts on behalf of Canadian patients, the Best Medicines Coalition (BMC) is interested in the work of all organizations which comprise the frameworks through which pharmaceuticals are reviewed, approved and reimbursed, including the Patented Medicines Prices Review Board (PMPRB). We value the opportunity to comment on the Health Canada document *Protecting Canadians from Excessive Drug Prices* released May 16, 2017, outlining proposed amendments to Patented Medicines Regulations. In addition, we participated via conference call in Health Canada's June 9, 2017 consultation meeting held in Ottawa.

The BMC is a national, non-profit alliance of 26 patient organizations with a shared goal of equitable and consistent access for all Canadians to safe and effective medicines that improve patient outcomes: the right drug for the right patient at the right time. Areas of interest include drug access, approval, assessment and reimbursement along with patient safety and supply concerns. As an important aspect of its work, the BMC strives to ensure that Canadian patients have a voice and are meaningful participants in policy development, specifically regarding pharmaceutical care.

Issues and goals: What do patients want?

As a coalition, BMC positions are informed by the values and perspectives of its members, each of which represents distinct patient communities. Perspectives are gathered informally through discussions, such as at coalition meetings, and formally through surveying on issues and goals. For example, in late 2016 and 2017, BMC patient organizations were asked to identify issues and priorities, with the following conclusions:

- Throughout the lifecycle, including through pricing regulation, assessment, negotiations and reimbursement, timely access to medicines is a top priority. Simply put, Canadian patients should not have to endure extended wait times to access and be covered for necessary medications.

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- Canada's convoluted and fragmented processes for delivery of pharmaceutical care, what can be described as a labyrinth, presents significant barriers to improved patient care and outcomes. Processes throughout the life cycle must be streamlined and optimized by eliminating duplication and improving alignment, accountability and transparency. Patient participation in policy development and decision-making should be an integral component of all stages.

Research conducted in 2017 regarding perceptions of BMC's external stakeholders, including policy-makers, shows strong congruency with that of the patient community, including common hopes for a harmonized, lifecycle approach to pharmaceutical care, with more coordination, modernization and efficiency through pan-Canadian efforts.

In addition, fundamental BMC positions are outlined in *Equitable Pharmaceutical Care: Principles and Considerations Regarding Pharmacare for All Canadians*, a 2015 consensus document prepared to inform pharmaceutical reform. These principles include universal and equitable pharmaceutical care, timely access to a comprehensive range of therapeutic options and a collaborative approach to policy and program reform which includes patients.

BMC's input on pricing regulation, including both broader modernization and the current regulation consultation, is informed by these perspectives and principles highlighted above. In summary, these including the following:

- Timely and universal access
- Comprehensive range of options
- System alignment without duplication
- Transparency and accountability
- Collaborative policy reform and patient participation in decision-making

Pricing reform: Outlining core patient positions

As with the BMC's comments on the PMPRB's guideline modernization, our input on the proposed regulations is focused on those issues most relevant to patient perspectives and interests.

As a starting point, please consider the following core, patient-driven positions:

- BMC supports a strong regulatory framework for pharmaceutical pricing aimed at both protecting consumers, primarily patients, and contributing to the value and sustainability of the health care system itself.
- A primary goal of the PMPRB, like other agencies operating in the realm of pharmaceutical care in Canada, should be to contribute to an environment that offers Canadian patients access to and coverage for a comprehensive range of medicines, including newly developed advancements to address unmet needs.

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- It is appropriate that a pharmaceutical pricing framework be implemented by a national body and that it operate in concert with and reflect the realities of other national and regional bodies which play a role in pricing, thereby avoiding duplication.
- All stakeholders, including patients, have a legitimate role in determining the next iteration of a pharmaceutical pricing regulation framework and should be engaged on an ongoing basis.

Fundamentals: Framing reform goals

In reviewing the documentation on pricing regulation reform and the proposed regulations, goals are clearly stated as to protect Canadians from excessive prices for patented drugs. Indeed, this goal reflects the PMPRB's mandate and is appropriate in the context of system sustainability. From a patient perspective, the intention to lower prices is worthwhile but maintained and improved levels of patient care are also important. Each measure to achieve reform goals must be evaluated in terms of its role in influencing the range of treatment options available in Canada now and in the future. In addition, related to international pharmaceutical launch plans, ensuring that Canadians are able to access treatments early must also be a core goal. An appropriate balance must be found so that levels of patient care are improved and not compromised.

In addition, the price reduction goal is limiting and does not reflect broader health care goals. From a patient perspective, the PMPRB is not just a body to protect from excessive pricing, but it also has a role, along with other bodies, of contributing to an improved health care system. Specifically, to be an effective and relevant part of the entire framework, the PMPRB must play a positive role in maintaining and enhancing a high level of quality care and contributing to improved outcomes for all patients.

Pharmaceutical spending is viewed as a cost to the system but there must also be a recognition that it is an investment in improved health for Canadians. The language used to describe goals and objectives should reflect this.

In addition, introducing greater system-wide efficiency, including alignment and avoidance of duplication, must also be considered goals of this specific regulatory initiative and indeed broader reform.

In this context, each element of this reform package must be viewed and evaluated from these perspectives:

- Does it ultimately contribute to improved patient care and outcomes?

- Does it reduce duplication, improve efficiency and contribute to value and sustainability of the health care system?

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Proposed regulations: Applying patient considerations

Economic-based price regulation factors:

While there is fundamental support to the notion that pharmacoeconomic analysis has a place in pharmaceutical review and evaluation, it is challenging to evaluate the concept of incorporating economic factors into pricing regulation without a comprehensive explanation of anticipated process and timing. From a patient perspective, this must be viewed through the lens of avoiding duplication and improving alignment in the system, and, of course, not negatively impacting timeliness of access. Even with the understanding that PMPRB would be using the same evidence and evaluation currently provided to the Canadian Agency for Drugs and Technologies in Health (CADTH) and *Institut national d'excellence en santé et en services sociaux* (INESSS), it is imperative that provisions be in place to ensure that any additional consideration not add to cumulative review times throughout the process, which would essentially create a barrier to timely patient access.

In addition, while patients support the notion of prices reflecting a demonstration of better health outcomes, we would urge greater consideration of broader evaluation of outcomes. Specifically, measures should be included to incorporate patient-reported outcomes, patient engagement in clinical trial design and other forms of evidence beyond randomized control trials, especially real-world evidence.

Regarding the use of Quality-Adjusted Life Years (QALYs), it is stated that this will allow for comparisons between drugs, but there must also be consideration of the cost of not having access to a specific drug even if other similar drugs are available. It is important that the patient-driven criteria of access to a comprehensive range of treatment options be applied. Importantly, from a patient perspective, QALYs and other conventional methodologies of health technology assessment are believed to be problematic when applied in some disease areas and so perhaps not appropriate. The use of QALYs is in essence unfair to patient communities with small numbers affected by rare disorders, for example.

Expanded list of comparator countries:

Expansion of the list of comparator countries is in theory acceptable. However, from a patient perspective, there is a lack of confidence that all the countries in the proposed basket are truly appropriate, given levels of quality pharmaceutical care and types of systems in those countries. For example, some countries have hard caps on drugs allowed per person and may put limits on the range of drugs offered through public programs. There are significant differences in the range of medicines available and funded in each proposed comparator jurisdiction which can be a measure of quality of care. While it is understood that many factors contribute to manufacturer decisions on when to launch, further analysis, detailed data and explanation would be helpful to foster more meaningful understanding of the full impact of this proposed change.

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Regulating and reporting on patented generic drugs:

From a patient perspective, it is difficult to evaluate the appropriateness of the level of regulatory rigour applied to managing prices of newer medications versus generic medications, including those with a patent. It would appear that the move to a complaint-based system for patented generics would facilitate increased efficiency. Again, appropriate regulatory burden and impact on the market must be fully evaluated to ensure that Canada offers an environment that encourages timely introduction of medications and offers a level playing field. If the PMPRB's oversight of these types of drugs moves to a complaints-based process, it is crucial that all stakeholders be able to navigate the process, including those in the patient community. In addition, further consideration could be given to expanding the application of a complaints-based system to other pharmaceuticals which are considered low risk in terms of excessive pricing. Regarding generic drug prices generally, and outside the realm of these proposed regulations, it could be argued that for the system to be more effective there could be a role for tighter, higher level regulation of generic drugs. However, consideration must include a full analysis of other bodies and policy tools currently used and how all elements could be used more effectively including those at the drug program/payer level. Again, duplication and efficiency criteria must be applied on every consideration.

Reporting of rebates and discounts:

In the interests of improved transparency and greater alignment within the entire review system, mandatory reporting of any subsequent rebates and discounts is acceptable as long as it leads to greater value in the system and does not contribute to decreased treatment options. For example, due consideration must be given to whether this will have an impact on depth of discounts offered. Broadly, reform of the regulation framework must acknowledge the realities and complexities how actual prices are currently managed by other bodies including both the pan-Canadian Pharmaceutical Alliance and in the private insurance sector through negotiated agreements with manufacturers. From a patient perspective, it would seem that organizations other than the PMPRB have a strong role in effectively mitigating prices through reimbursement channels. There exists a significant level of duplication in this regard and this must be addressed, if not through this specific regulation reform initiative then through broader, more comprehensive reform. This overlap of mandate and duplication of mechanisms needs to be fully understood and addressed, including longer term consideration of the option of moving from a national wholesale price ceiling model to focus on the price negotiation function of bodies associated with reimbursement.

Monitoring and evaluation

Regardless of how Health Canada moves forward with these proposed regulations, we urge ongoing monitoring of the process and a rigorous evaluation of outcomes. This must include full understanding of impact, including analysis of real savings. Patient values and perspectives must be incorporated throughout monitoring and evaluation phases, including consideration of impact on timely access, availability of a range of treatment options and system efficiencies as well as alignment and reduction of duplication. It will be imperative to analyze and evaluate how resultant savings are invested in improved patient care, and not realized in general revenue to government.

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Moving forward

We trust that Health Canada, in consultation with the PMPRB and other regulatory and pharmaceutical program organizations, will consider all patient input carefully, including submissions from other coalitions, disease-specific patient organizations and individuals. We also urge Health Canada to work cooperatively with entities with a commercial interest in pricing frameworks to ensure the regulatory framework encourages pharmaceutical availability and Canadian investment. We trust that the next phase of the consultation will also include full representation from the patient community, and we respectfully request that the BMC be part of this process.

Again, we thank Health Canada and the PMPRB for its efforts to educate the patient community and seek its input, and would welcome an opportunity to discuss how this can be improved. In addition, again we suggest that the PMPRB and Health Canada, consider establishing a patient advisory body to enable greater collaboration moving forward, and that significant patient representation be included in any multi-stakeholder collaboration or review.