August 4, 2020

Patented Medicine Prices Review Board
333 Laurier Avenue West, Suite 1400
Ottawa, Ontario K1P 1C1

Best Medicines Coalition Input Regarding PMPRB Revised Draft Guidelines

Introduction

The Best Medicines Coalition (BMC), a national alliance of patient organizations together representing millions of patients, welcomes this opportunity to comment on the Patented Medicine Prices Review Board's (PMPRB) revised draft Guidelines to implement the Patented Medicines Regulations, as amended.

This submission follows input provided February 2020 on the previous draft Guidelines, as well as on proposed reforms, provided February 2018 and June 2017, and October 2016 regarding Health Canada's *PMPRB Guidelines Modernization Discussion Paper*. In addition, BMC was represented on the PMPRB Steering Committee. Most recently, BMC member organizations participated in PMPRB's July 8, 2020 stakeholder briefing webinar.

As with previous submissions, this submission was developed with the participation of BMC member organizations, each of which has had the opportunity to review content and provide input. Statements and positions expressed within this submission reflect areas of consensus among BMC member organizations below:

Executive Summary:

Patient perspectives on regulatory goals:
Canada needs regulations which improve affordability of medicines and bring prices in line with appropriate international comparators. Equally important, regulations must facilitate and not deter the introductions of new medicines and not prevent clinical trials from being conducted in Canada. Governance for regulating prices must be accountable, transparent, and inclusive.

Fundamental considerations:
With these core objectives in mind and following careful review, our position is that while there are positive elements significant concerns remain and, therefore, the regulations and the Guidelines should not proceed as presented.

Primary areas of concern are:

1. Initial signs of negative impact
2. Price reductions beyond and below original intent
3. Inequity regarding affordability gains
4. Lacking transparency, accountability, and meaningful patient engagement

Recommendations to move forward:
Evidence-based decisions. The path forward must be informed by current, credible, and comprehensive evidence, including on initial impacts on critical markers, namely introductions of new medicines and initiation of clinical trials sponsored by drug developers (Phase 2, 3 & 4). Work must begin on gathering appropriate data. The framework for data collection and analysis must be developed in cooperation with patient representatives and all stakeholders and conducted independently and impartially.

Appropriate pricing and phased implementation. Price reduction goals must be carefully reviewed and considered to ensure measures are appropriate and necessary and whether the regulatory package is in line with these goals. In addition, the regulations and Guidelines must be evaluated from the lens of all patients, regardless of how they access medicines, with special consideration of whether entrenching negotiated rebates is equitable to all patients. Regulation implementation should be phased in to quickly achieve affordability goals while permitting all of us to understand potential negative impacts on access to new medicines and relevant clinical trials. Specifically, a first phase would apply the new basket of comparator countries to bring down prices as soon as possible for all patients, leaving the economic factors to a second phase after data collection, analysis, and public discussion.

Rigorous evaluation. Measures to ensure transparency and accountability must be strengthened with the goal of ensuring that health outcomes and patient care are not diminished as regulations and Guidelines are applied. Monitoring and evaluation must be transparent and rigorous including analysis of real savings and costs related to possible treatment delays in the short or long term, with mechanisms in place to trigger adjustments. Patients must be involved in determining these factors. Importantly, public reporting must be entrenched, such as in the PMPRB Annual Report, and made public in a more timely manner than current PMPRB practice. In addition, an external audit would be appropriate to provide Canadians with confidence in our federal pricing regulator.

Collaborative engagement. A holistic and value-based approach to patient engagement must be adopted, with opportunities for input and involvement embedded in PMPRB's structure and throughout its processes. Specifically, patients should be represented on the Board and included on the Human Drug Advisory Panel, and a formal patient advisory body should be established.

Best Medicines Coalition Detailed Input On PMPRB Revised Draft Guidelines

Regulatory Goals: Affordability, Access, Transparency and Accountability

Our core position remains that Canada needs effective and balanced pharmaceutical pricing regulations which contribute to sustaining and improving the health and wellbeing of current and future patients. Regulations must achieve the following:

> ***Improved Affordability of Medicines.*** We support the goal of improving the affordability of medicines, both for individual patients, health care systems, and public and private insurance. Patients and their families, and those who pay on their behalf, bear a significant burden of prescription medicine costs, and we support the government's intention to address this, particularly in relation to appropriate international comparators.
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> ***Comprehensive Access to Medicines.*** Of equal importance, patients need timely access to new medicines which address unmet needs. There must be confidence, based on best available evidence, that regulations and Guidelines will facilitate and not discourage rapid introduction of a full range of medicines and vaccines as well as clinical trials which provide willing patients early access to promising new therapies.
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> ***Accountable, Transparent and Inclusive Governance.*** Canadians expect that public health care agencies adopt updated governance which upholds and demonstrates transparency and accountability. Relevant stakeholders, including patients, must be included in the PMPRB's policy discussions and decision making as a best practice.

PMPRB Revised Draft Guidelines: Key Considerations

We reviewed the revised draft Guidelines through the lens of the patient communities we represent, and in this context, we asked fundamental questions: How will patient care be impacted? Will policy goals of affordability, access to medicines, accountability, transparency, and inclusivity be achieved by these regulations and Guidelines?

We are encouraged that aspects of the proposed Guidelines have been revised, indicating the PMPRB heard many of the concerns raised by the patient community and others. Specifically, the ability to establish an interim Maximum List Price (iMLP), the elimination of a Maximum Rebated Price (MRP) for Category 2 medicines, and higher thresholds and price floors for Category 1, among other revisions, are positive developments.

However, our position remains that these regulations and draft Guidelines should not proceed without confidence that there will not be negative implications for patients and with processes in place to identify and address possible unintended repercussions. From our review, there continues to be unease about the downstream impact of the regulatory proposal and Guidelines.

Our primary areas of concern are:

1. Initial signs of negative impact
2. Price reductions beyond and below original intent
3. Inequity regarding affordability gains
4. Lacking transparency, accountability, and meaningful patient engagement

These considerations are reviewed below, followed by a discussion of implementation options and recommendations.

Discussion on Key Considerations:

1. Initial signs of negative impact

A key marker for the patient community is whether medicines – particularly those which address unmet needs – will be available to Canadians affordably and in a timely manner. There are early indications that introductions have declined in comparison to other countries, although we acknowledge that some evidence is contradictory or unclear.

It was encouraging to learn from PMPRB at its July 8, 2020 webinar that countries with lower prices may have greater availability of new medicines, citing its own 2017 Annual Report. Furthermore, PMBRB also cited Health Canada data indicating that medicines approved in Canada in the first quarter of 2020 have not diminished from previous levels. In addition, the number of medicines approved in Canada within a year after being approved in the United States increased in 2019 over the previous year, citing Health Canada and United States (FDA) data.

However, looking at medicine launches, a Life Sciences Ontario report (*New Medicine Launches: Canada in a Global Context*, June 2020) comparing Canada to 24 countries indicates a 40 per cent drop in 2019 here while launches elsewhere increased. Further, of the total number of medicines launched globally less than half are being introduced in Canada with shortfalls in oncology and rare diseases. As patient organizations, we are not in a position to provide analysis, but we note the pharmaceutical industry cites apprehension about the amended regulations and Guidelines, including inherent uncertainty, as precipitating this apparent cooling in the Canadian market. The industry refers to the impact on global markets and regulatory complexity as deterrents, and that this second version of the Guidelines is significantly more complex than the original. Regarding complexity, it is apparent the regulations and proposed Guidelines introduce various additional layers, rather than streamlining the patented medicines review process. Confusion remains regarding overlapping roles of PMPRB, Canadian Agency for Drugs and Technologies in Health, and the pan-Canadian Pharmaceutical Alliance.

Moreover, there are concerns about possible negative impacts on decisions on whether to conduct industry-sponsored clinical trials in Canada, in particular those in Phases 2, 3 and 4. Clinical trials are an important conduit to promising new therapies for many patients. While we understand that there are many factors that are taken into consideration as decisions are made, among the reasons cited by industry for not conducting these trials in specific countries are prospects for reimbursement in these countries and uncertainty regarding price.

These are complicated issues, of course, and the evidence needs to be fully explored. However, findings about timely access are deeply concerning and reflect what some patient organizations see as they are made aware of specific medicines being used in treatment in other countries while Canadian patients wait. Clearer projections of initial impacts are needed to chart the path forward. Decisions on how to proceed must be informed by current, credible, and comprehensive evidence, ideally developed in cooperation with all stakeholders.

2. Price reductions beyond and below original intent

We fully support a modernized regulatory framework that reduces prices to reasonable levels, which was the government's original intent. However, there are indications that the proposed regulations, implemented according to these revised draft Guidelines, will deliver prices well below this original intent with potentially negative implications for patient access to new medicines.

While there has been ambiguity about price reduction goals, in 2017 the OECD median was cited by the federal government as a target, achieving approximately a 20 per cent reduction. As stated by PMPRB at its July 8, 2020 briefing, just changing the basket of comparison countries would likely deliver a 20 per cent reduction. Analysts indicate that the additional application of the economic factors would deliver more dramatic and unpredictable reductions and thus impact the pharmaceutical market and corporate decisions.

We urge careful consideration of price reduction goals, including the goal of access to patented medicines, to ensure measures are appropriate and necessary and to assess whether the impacts of the current regulatory package are in line with these. We call on the Government of Canada to clarify publicly and precisely what are its goal(s) for drug price reductions. Is it, for example, to or below the OECD median and if below, how far below?

3. Inequity regarding affordability gains

We support the overall intent and focus of the regulations and Guidelines on reducing prices for all patients in Canada and especially the changes to the basket of reference countries. However, the regime as proposed by the draft Guidelines entrenches the negotiated price and rebate system and perpetuates patient inequities. Let us explain this point.

The regime as proposed by the draft Guidelines is centered on reducing the maximum rebated price and is designed to reduce patented medicine prices for patients who access medicines through a public or private health benefits plan. This excludes patients who pay out-of-pocket for their medicines and who therefore will not benefit from reductions in the maximum rebated price. Recent statistics from the Canadian Institute for Health Information estimate that patient out-of-pocket payments account for 20 per cent prescription medicine spending (with 43 per cent public plans and 37 per cent private insurance.) Further, patients who provide co-payments through their plans will or could also be paying rates based on the maximum list price, resulting in inflated payments to plans (including governments) that receive the rebates from patented medicine manufacturers.

We encourage the PMPRB to consider the impacts of the regulations on all patients, including those who rely on public and private plans and those who pay directly, to ensure that affordability gains are achieved equitably.

4. Lacking transparency, accountability, and meaningful patient engagement

Throughout the reform consultation process, diverse stakeholders have called for measures to improve the Board's transparency and embed greater accountability through rigorous monitoring and evaluation. In addition, patient organizations have repeatedly called for improved involvement, both through the consultation process and implementation. It has been noted that the consultation process so far has excluded genuine engagement with stakeholders and has been viewed as a one-way dialogue.

It is unfortunate that concerns about lack of patient participation in this process (including the monitoring, reporting and addressing of adverse impacts on patients) have not been reflected in the draft Guidelines, despite consistent requests by patient representatives to be present at the table, as is standard practice at other public bodies involved in the review and assessment of pharmaceuticals.

The updated Guidelines still do not describe points of engagement and input for patients and patient organizations, except in the consultation process regarding proposed changes to the Guidelines. In Section B, item 94, PMPRB staff are given significant freedom to "utilize any of the tests described in the Guidelines and modifications or variations of those tests (e.g., MIP instead of HIP or median as opposed to the top of the dTCC) depending what it believes most appropriate to the factual circumstances surrounding the price of the patented medicine under investigation". PMPRB is out of step with best practices in patient engagement.

We acknowledge that giving administrators the ability to deviate from the Guidelines could be positive if decisions are made which improve patient care. Nonetheless, there is a great deal of uncertainty and lack of transparency regarding staff discretion. This broad ability to carry out modifications and variations at the staff discretion without further guidance or explanation for the use of this discretion goes against the spirit of providing clear and transparent guidelines on actions as well as engaging full consultation with interested parties.

Minimal details have been provided regarding accountability, including ongoing monitoring and evaluation. It is unfortunate that the *Guidelines Modernization and Evaluation Process*, or a plan for its development, was not made available in early 2020 as originally planned but is, instead, postponed until after Guidelines are finalized. We look forward to the opportunity to provide input on the development of this process. This is one of several reasons to phase in the regulations and Guidelines.

Moving Forward: Recommendations for positive implementation

1. Phased implementation to gauge progress

We propose phasing in aspects of the regulations through the Guidelines to meet the goal of increasing affordability without additional measures which may have unintended negative impacts on patient access to new medications. Put simply, these regulations introduce too many changes at the same time, and some of these measures can be considered experimental having never been tried elsewhere or within the unique Canadian system.

Specifically, we support moving ahead with application of the revised basket of comparator companies, given that this will in itself provide price reductions that will satisfy the goal of improving medication affordability. As such, the new economic factors, which are inherently complicated, and lacking in predictable outcomes, and potential to cause adverse impact on our most vulnerable communities, could be moved to a second phase for consideration if desired cost savings are not achieved. This approach is especially appropriate given the recent decision of the Federal Court which confirmed that PMPRB is limited to regulating only the list price. In addition, these policies are potentially being implemented during a time of great upheaval in the economy and social systems, and it is necessary to consider how all these pieces will work together in our "new normal".

Moving forward first with the change in comparator countries would also allow Canadians to better consider the effects and outcomes of this major reduction in prices on its own. As in good medical practice, a prescribing clinician should implement one treatment or change at a time, in most circumstances, to understand the impacts of each new medical intervention on the patient. Moving forward with both major changes (comparator countries and the new economic factors) will make it impossible to assess the impacts of each change, and risks "over-medicating" the already complex and challenged pharmaceutical system in Canada.

We strongly believe that when there is not reasonable certainty that aspects of the new regulations and guidelines will have a positive impact on patient care and health system efficiency, value and sustainability, then these elements must be revisited. Furthermore, before implementation, there must be full assurance that a rigorous and independent process will be implemented to monitor, identify, analyze, publicly report, and address any adverse impact on patients' access to medicines.

2. Comprehensive monitoring, independent evaluation, and adjustments

BMC implores PMPRB and the Government of Canada to provide transparent and comprehensive post-implementation surveillance, including ongoing monitoring and independent evaluation. We request an evaluation process which is broad in scope and rigorous, evaluating the impact on the people of Canada. Building on the areas outlined in the background document provided by PMPRB, we request the incorporation of metrics specifically focussed on patient care outcomes including the availability of new therapeutic options for treating people in Canada in comparison with those in other countries as well as the prices of existing medicines.

Evaluation and monitoring must include both timing and comparisons to other countries and previous medicine launch rates in Canada prior to the application of the new regulations and Guidelines. As an early measure of changes in medicine launches, there must be monitoring of clinical trials, sponsored by drug developers, compared to historical numbers in Canada and other OECD countries. Changes in the number of clinical trials initiated, subjects enrolled, and new medicines researched will be an early sign of the success or shortcomings.

Furthermore, evaluation must include analysis of real savings and subsequent investments, including the health system costs if access to breakthrough medicines is delayed or prevented. Importantly, there must be mechanisms in place to incorporate adjustments within the new framework and Guidelines.

The mechanism and process for monitoring and evaluation, developed in consultation with patient representatives and other stakeholders, should be transparent and conducted regularly with early indicators to trigger early intervention before there is significant harm to Canadians. This must be undertaken in a timely manner with an independent evaluation conducted within 12 to 18 months of implementation and as part of the PMPRB's annual reporting for the first five years following implementation and moving forward. Monitoring and evaluation processes must address these fundamental questions:

- What has been the impact on the range of medicines made available and the timing of introductions, compared to previous levels in Canada and in other countries, on the types of medicines made available and on the number and types of clinical trials conducted in Canada?
- Do the new regulatory framework and Guidelines reduce duplication, improve efficiency, and contribute to health care system sustainability?
- Is the new regulatory framework flexible enough to ensure that new medications to address unmet needs are expedited?
- Do the new regulations ensure that existing and older medicines do not incur price increases that reduce net savings?
- How will patient organizations engage and identify issues and difficulties of accessing breakthrough medicines which may be a direct impact of new regulations?
- Does the new framework contribute to improved patient care and outcomes and, if so, to what extent?
- What is the impact, if any, on drug supplies and shortages?

These monitoring and evaluation processes must encompass high standards of transparency, independence, and accountability, with thorough reporting. Patient communities and other stakeholders should be consulted on design and be involved in implementation and application. Specifically, patients should be part of the team that oversees this process. In addition, an independent audit or independent evaluation would be appropriate to provide Canadians with confidence in our federal pricing regulator.

3. Comprehensive 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 participation in decision-making. In addition to consulting with patients on how to accomplish this, we suggest PMPRB look to best practices both here in Canada and internationally.

Regarding governance, we support patient representation on the Board, inclusion on the Human Drug Advisory Panel (HDAP), and the establishment of a formal patient advisory body. Patients must have a role in strategic planning, policy and prioritization, and processes for patient 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, patient involvement must be facilitated and provided with appropriate support. Discretionary modifications and adjustments to publicly disclosed processes by PMPRB staff should be the subject of broad discussion and consultations incorporating unbiased, diverse perspectives.

Summary: Recommendations to move forward

Canada needs strong and effective pharmaceutical pricing rules to improve affordability of medicines while also allowing for access to a comprehensive range of medicines, including breakthroughs for unmet needs of patients. The regulatory framework must be accountable, transparent, and inclusive of all relevant voices, specifically patients.

There are important questions about whether the regulations and Guidelines will achieve intended goals and, as patient organizations, we remain concerned that effective and necessary medicines will not be made available to patients in Canada. For patients with unmet needs and life-threatening conditions, this is of the utmost importance. Furthermore, the proposed framework, as outlined in the revised Guidelines, does not ensure transparency, accountability, and inclusive involvement.

We offer the following recommendations:

Evidence-based decisions*.* The path forward must be informed by current, credible, and comprehensive evidence, including on initial impacts on critical markers, namely introductions of new medicines and initiation of clinical trials sponsored by drug developers (Phase 2, 3 & 4). Work must begin on gathering appropriate data. The framework for data collection and analysis must be developed in cooperation with patient representatives and all stakeholders and conducted independently and impartially.

Appropriate pricing and phased implementation. Price reduction goals must be carefully reviewed and considered to ensure measures are appropriate and necessary and whether the regulatory package is in line with these goals. In addition, the regulations and Guidelines must be evaluated from the lens of all patients, regardless of how they access medicines, with special consideration of whether entrenching negotiated rebates is equitable to all patients. Regulation implementation should be phased in to quickly achieve affordability goals while permitting all of us to understand potential negative impacts on access to new medicines and relevant clinical trials. Specifically, a first phase would apply the new basket of comparator countries to bring down prices as soon as possible for all patients, leaving the economic factors to a second phase after data collection, analysis, and public discussion.

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About the Best Medicines Coalition

The Best Medicines Coalition is a national alliance of patient organizations, together representing millions of patients, with a shared goal of equitable, timely and consistent access for all Canadians to safe and effective medicines that improve patient outcomes. The BMC's areas of interest include drug approval, assessment and reimbursement, as well as patient safety and supply issues. As an important aspect of its work, the BMC strives to ensure that Canadian patients have a voice and are meaningful participants in health policy development, specifically regarding pharmaceutical care. The BMC's core activities involve issue education, consensus building, planning and advocacy, making certain that patient-driven positions are communicated to decision makers and other stakeholders. The BMC was formed in 2002 as a grassroots alliance of patient advocates. In 2012, the BMC was registered under the federal Not-for-profit Corporations Act.

Alliance for Access to Psychiatric Medications
Asthma Canada
Brain Tumour Foundation of Canada
Canadian Arthritis Patient Alliance
Canadian Association of Psoriasis Patients
Canadian Breast Cancer Network
Canadian Cancer Survivor Network
Canadian Council of the Blind
Canadian Cystic Fibrosis Treatment Society
Canadian Epilepsy Alliance
Canadian Hemophilia Society
Canadian Mental Health Association
Canadian PKU & Allied Disorders
Canadian Psoriasis Network
Canadian Skin Patient Alliance
Canadian Spondylitis Association
Crohn's and Colitis Canada
Cystic Fibrosis Canada
Fighting Blindness Canada
Health Coalition of Alberta
Huntington Society of Canada
Kidney Cancer Canada
Lymphoma Canada
Medicines Access Coalition - BC
Millions Missing Canada
Ovarian Cancer Canada
Parkinson Canada