Medicines Access Coalition - BC

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HESA
Standing Committee on Health

Submitted on website

BRIEF SUBMITTED REGARDING PMPRB UPDATED DRAFT GUIDELINES

BACKGROUND

Medicines Access Coalition – BC (formerly The Better Pharmacare Coalition) has been effectively advocating for appropriate and timely access to evidence-based prescription medications through the BC PharmaCare program and federal agencies since 1997. With a renewal of the Coalition in 2020 and a new name which more effectively reflects our mandate, we aim to be the leader in advocating for better access to medicines in BC by providing a unified voice of many patient care organizations. We are now known as MedAccessBC and have expanded our scope and activities to more effectively meet the needs of our coalition members and improve the health of British Columbians which often requires us to take action at a federal level, such as feedback and submissions we have provided to CADTH and its programs and services, PMPRB, and other national organizations.

MedAccessBC's current member organizations represent more than two million BC patients, caregivers and advocates. We achieve our mandate by providing education and awareness, interacting with stakeholders who participate or influence the decisions directly affecting the access to medicines including, policy makers, government, researchers, health practitioners, public and private health payers, benefit managers/consultants, pharmaceutical manufacturers, and others who play a role in the access to medicines.

On behalf of the members of MedAccessBC, we welcome the opportunity to provide a written submission sharing our views on the Patented Medicine Prices Review Board (PMPRB) Updated Draft Guidelines and put forward requests for considerations prior to its implementation. We recognize the importance of maintaining and ensuring fair prices for medicines which are affordable for Canadians. However, we also emphasize the importance of ensuring a healthcare landscape that ensures Canadians have consistent access to new and breakthrough medicines as well as participate and gain benefit from clinical trials involving new drug therapies. Early access to innovative and life-saving medicines in parity with the rest of the world ensures Canadians are able to achieve a high level of quality of life and life expectancy, contributing to the success of Canada as a whole. Patients and patient organizations who focus on the health and well-being of people and Canadians as a first priority have perspectives on these PMPRB Updated Draft Guidelines which may be different from those who are regulators, create policies, plan budgets or are employed by for-profit corporations. We draw your attention to a number of areas which we highlight so you may consider and engage.

VIEWS AND CONSIDERATIONS

As a starting point, we support and endorse the submission and input provided by the Best Medicines Coalition (BMC), who have provided their submission under separate cover.

We acknowledge the updates which have been made to the PMPRB Draft Guidelines where higher values and thresholds are in line with recognizing the benefit drugs bring to Canadians, including the 150% of GDP threshold for Category I drugs. Similarly, the increase in Pharmacoeconomic Value Threshold ("PVT") to higher levels per QALY are more appropriate than originally proposed.

Our core concerns are ensuring that medicines are accessible to the people who need them for the treatment of medical conditions, and that:

- choices of treatments are available to appropriately treat the diversity of individuals in Canada with chronic conditions,
- medicines are fairly priced and affordable for Canadians,
- Canada continues or improves on the number of new and useful medicines launched
- Canadians benefit from research of new medicines,
- new medicines are available in Canada early, and that Canada is one of the first countries to have access in the world,
- the processes of controlling or limiting prices is conducted through a transparent process,
- price control measures are done with accountability and responsibility to Canadians,
- patients and patient organizations are genuinely and meaningfully engaged when it comes to health regulations, processes and policies which impact the health and well-being of Canadians.

PMPRB Updated Draft Guidelines: Key Areas Requiring Review

Although some items have been improved, the main concerns expressed in our previous submission remain unaddressed and concerns raised by patient communities require attention.

1. **Price reductions through complex processes and methods may put Canada in a lower tier for clinical trials and drug launches**
2. **Limitations and unintended consequences of arbitrary market size thresholds**
3. **Patient perspective and patient engagement are lacking**
4. **Transparency and accountability**
5. **Monitoring of the impact of PMPRB changes should be done with built-in early warning signals**

Key Areas Requiring Review – Details

1. **Price reductions through complex processes and methods may put Canada in a lower tier for clinical trials and drug launches**

It has been highlighted in previous consultation forums with the PMPRB that the change in the basket of comparator countries moving from the original PMPRB7 to the proposed PMPRB11 would result in an estimated 20% price reduction in drugs coming to Canada. The reduction of 20% is generally agreed on by most and there is little dispute over the level of reduction expected. This is a reasonable simple approach with more predictable impact which will achieve a significant reduction in drug prices by 20%, compared to adding multiple processes and complexities which have unpredictable direct and indirect effects, will further reduce prices, but may have an unanticipated cost to public healthcare systems and Canadians as a whole. The change in comparator countries and how prices will be assessed involves the Maximum List Price (MLP) and the Maximum Rebated Price (MRP) which are already complicated with the recent court ruling restricting access to information on third-party rebates. Additional processes with pharmacoeconomic analysis and market size thresholds are additional complexities that obscure transparency and result in the lack of predictability of the eventual drug price and its impact on availability of new drugs in Canada. It is recognized that the aim of PMPRB is to arrive at lower prices, however, there have been concerns expressed that excessively low prices and lack of predictability of the price review process may be detrimental to the introduction and availability of innovative and life-extending drugs in Canada.

A phased implementation process may be of benefit to allow better clarity of the cause and effects of these changes. The current updated Guidelines will implement many new changes and processes all at once in addition to the new basket of comparator countries. We urge the PMPRB to consider implementing a phased approach of implementation where Phase 1 may be the move towards the new basket of comparator countries, PMPRB11, with a period of post-implementation review to assess the necessity of price evaluations. It may be possible that the 20% savings or more achieved with the change in the basket of comparator countries may be sufficient and / or consequently then allow more refined changes in the other areas that can then be implemented with more information and knowledge, after gaining some experience and insight.

However, we caution that a potential effect of the PMPRB11 basket of comparator countries is a resulting delay in drugs coming to Canada as manufacturers may wait until other comparator countries in the basket set prices first. As a result, there will likely not be any incentive for manufacturers to select Canada as the first country in which to launch new drugs. These changes have the potential to have Canada de-prioritized to a lower tier in the consideration of which countries are chosen to launch a new drug in the global pharmaceutical market. This is bad news for patients in Canada.

Excessively low drug prices in Canada may have unintended consequences of not only delaying new drug launches, but it may also result in fewer drug launches compared to other developed countries due to no launch decisions for Canada from manufacturers. This contributes to decreasing access to medicines compared to other countries and may also be associated with a decline in the availability and enrolment of clinical trials focusing on innovative and life-saving drugs in Canada. In our previous submission, we described a real-world example where a new drug was not going to be launched due to the anticipated PMPRB Guideline changes, even though it was approved in Canada. Therefore, a

comprehensive and accurate monitoring of new drug approvals, launches, and marketing of these products is needed to ensure that changes have not worsened the access to medicines for Canadians. Leading up to this consultation, we have seen conflicting data presented, ranging from a dramatic decrease in new drug launches and clinical trials, to the opposite extreme where reports are illustrating no change or increase to new drug launches and the number of clinical trials since the announcement of the PMPRB Guidelines. This demonstrates practices of bias and subjective interpretation of complex data, which further emphasizes the need for a robust and reliable method of monitoring developed to accurately capture the impact of PMPRB changes. A monitoring plan should include the participation of individuals with a bias toward patient care and wellbeing, which is discussed in more detail below.

2. Limitations and unintended consequences of arbitrary market size thresholds

As a coalition of patient organizations, we want to emphasize that the arbitrary market size thresholds (high cost or high market size) based on dollar value of sales may not produce the desired impact. Manufacturers who have drugs nearing market size thresholds may make decisions not to provide compassionate drug supply or financial assistance if those drug units (if considered as volume or sales) push them above a threshold. Some patients benefit from compassionate drugs supplied for indications which are off-label and necessary for treatment as prescribed by Specialist physicians. These treatments are used for patients when other alternatives may be exhausted and manufacturers recognize the financial difficulties of paying for these therapies. Similarly, manufacturers have provided financial support to help reduce out-of-pocket drug costs for patients who are financially strapped, and these may also be in jeopardy depending on how the market size thresholds are determined. The COVID-19 pandemic exacerbates the financial burdens for Canadians and can make the PMPRB changes unbearable for patients.

Furthermore, these arbitrary thresholds may also dissuade manufacturers from pursuing research and applications for line extensions (more dosage forms) or applications for more indications for the molecule, particularly if it may cause a detrimental impact due to exceeding a market size threshold. Patients can benefit from the research conducted on seeking new indications for drugs, Health Canada approval of new indications for new medicines is an effective approach to preserving the health of Canadians rather than searching for a novel molecule. A new indication for an existing drug can be better and quicker than research and testing of subjects with a new drug. Due to the complexities associated with calculating market size thresholds and the fact that there has been a Federal Court ruling related to the reporting of rebates as beyond the scope of the PMPRB's regulating authority, it is not possible to fully anticipate the impact of the Guidelines, including;

a) the number of new drugs coming to Canada to treat patients,
b) the number of clinical trials evaluating new drugs in Canadian patients, and
c) the delay in the introduction of new drugs in Canada compared to other countries.

Clinical trials are not only an important opportunity for therapy and evaluation when existing treatments have been tried unsuccessfully, but those who respond to the study drug will often be able to continue the therapy after conclusion of the clinical trial, subsidized by the study sponsor.

These are some important factors for patients when accessing medicines, which are not commonly recognized as benefits of participating in clinical trials in Canada. These market thresholds and how they are calculated raise significant concerns if these changes lead to unintended negative consequences and negatively impact the areas that are of importance to patients.

3. Patient perspective and patient engagement is lacking

As patients and patient organizations, we appreciate the opportunity to provide input, feedback and consultation. However, despite the existence of the reform consultation process, many stakeholders including the MedAccessBC members have asked – without success – for improvements to the PMPRB's transparency and that they demonstrate greater accountability through rigorous monitoring and evaluation. It makes practical sense to include patients and patient organizations to be genuinely involved in the consultation process, but many of those who have been directly involved in any form of consultation have felt and expressed frustration that the dialogue has been mainly one-way. PMPRB has moved forward with its plan without truly considering the input, recommendations, and knowledge that these individuals and groups have provided. Involvement in consultation and preparing submissions are a significant undertaking for patient groups, many of which are registered charities and non-profit organizations run with small budgets and volunteers. Meaningful engagement is the very least these tireless individuals and groups should receive for all the efforts and time they put in. Afterall, these regulations, guidelines and policies are made, or indeed should be made, in the best interest of Canadians who need medicines.

Concerns about the lack of patient participation in this process (including the monitoring, reporting, and addressing of adverse impacts on patients) have not been addressed in the Updated Draft Guidelines, despite consistent requests by patient representatives to be invited to the table. This is standard practice for other public bodies who have involved patient representatives in the review and assessment of pharmaceuticals.

In addition, the Updated Draft Guidelines still do not describe points of engagement and input for patients and patient organizations in the process. The opportunity exists for consultation when changes are made to the Guidelines, however, much of the feedback provided in the 122 submissions do not appear to be addressed in the Guideline updates.

4. Transparency and accountability

There continues to be concern around the lack of transparency and that there is too much discretion and subjectivity given to the PMPRB Staff. For example, with respect to investigations where no board members are involved, staff have a great deal of discretion. As described in Section B, item 94, the 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".* This essentially allows complete modification or

variation of tests at the staff's discretion, essentially allowing interpretation and subjectivity to play a role in how tests are applied and conducted in these investigations, which seems inappropriate and lack transparency.

Discretion may be necessary at times, but it is unclear the motive and objective of the staff carrying out these investigations. At the very least, patient representatives and other objective participants should be involved in making discretionary subjective decisions which will affect other decisions and outcomes. Patient representatives are ideal individuals to be involved in such investigations and reviews, as their main goal is to uphold the rights of and benefits to patients, whereas the mandate of staff for investigations is unclear and undefined.

Details around who is responsible and accountable have been conspicuously limited, as well as the metrics and performance indicators which are to be monitored and measured. The *Guidelines Modernization and Evaluation Process (GMEP)*, or a plan for its development, was not made available in early 2020 as originally planned, but is, instead, postponed until after the Guidelines are finalized. Implementation should not take place prior to the development of a robust and detailed monitoring plan. Afterall, appropriate practice dictates that you should not implement changes when you do not have a monitoring plan. We look forward to the opportunity to provide input on the development of a monitoring mechanism and process as well as determining the metrics and monitoring parameters, which are described in more detail below.

5. Monitoring of the impact of PMPRB changes should be done with built-in early warning signals

A comprehensive, robust, and accurate monitoring plan in combination with a monitoring process and details describing the sources of data and definitions is largely absent in the PMPRB Updated Draft Guidelines. We ask that the PMPRB provide a transparent and comprehensive post-implementation surveillance plan and process, including ongoing monitoring and independent evaluation, with active and respected participation from patient organizations or patient representatives who are focused on the impact to patients. The evaluation process must be broad in scope and rigorous, evaluating the impact on Canadians as it relates to market entry and access to new drugs. Building on the areas outlined in the Updated Draft Guidelines background document provided, we request the incorporation of metrics specifically focused on patient care outcomes, including the availability of new therapeutic options for treating people in Canada in comparison with those in other countries. This consists of the following measures:

a) number of new drugs submitted for approval,
b) number of new drugs approved for marketing (NOC),
c) delay in time to launch in Canada compared to first launch in the world,
d) time to availability on market when it can be used for treating patients,
e) number of new drugs listed on public formularies,
f) number of drugs and patients going through the Special Access Programme,
g) number of patients being sent to USA and other countries for treatments not available in Canada, and
h) other measures which are directly or indirectly impacted by the PMPRB Guideline changes.

The number of clinical trials of new drugs, the respective drug trial phases, the number of subjects enrolled, the number of study sites, and other measures directly or indirectly impacted by the PMPRB changes should be measured and compared to historical numbers in Canada as well as other countries to identify trends and forecast impact to Canadians. Negative effects should be identified early, and corrective measure must take place as soon as possible to avoid further harm to Canadians.

A plan that adopts an early warning mechanism is needed to identify and provoke early action, decisions, and changes should the impact of these PMPRB Guidelines appear to show a negative trend compromising the care or treatment of Canadians compared to historical numbers or compared to similar countries. One of the core measures includes the appropriate monitoring and comparison of the number and time to new medicine launches in Canada and the number of drugs and the time it takes before a Canadian patient can be prescribed and treated with the therapy. During the preparation of this submission, we have reviewed discrepant reports on the number of clinical trials in Canada since the announcement of the PMPRB Draft Guidelines, the number of drug launches in Canada, and the number of new drugs to which patients have received access. The numbers and trends should be the same as these seem to be clearly defined measures, but it appears to be rather complex and open to interpretation since the conclusions from different sources of data are in stark opposition to one another. Involvement of patient organizations and patient representatives would help to resolve discrepancies as they are more likely to seek the true impact on the patients rather than uphold their organizational objectives and goals, which might explain the differences seen in the presentations.

In addition, the evaluation plan must include analysis of the net real savings or expenditures (further investments in the process), including the health system costs, PMPRB Staff budgets, and legal and associated litigation costs that arise from these PMPRB changes. It may be prudent to also monitor the number and costs related to legal conflicts which arise directly and indirectly from these PMPRB changes which diminishes the public funds available to improve the access to medicines and the health and wellbeing of Canadians. There is a human cost to delayed or non-access to breakthrough medicines and a mechanism to fairly identify this as early as possible must be incorporated within the new framework and Guidelines.

To maintain balance and improve the transparency of the mechanisms and processes used for monitoring and evaluating the metrics and performance indicators, these must be developed with the participation of stakeholders including patients and patient organizations. Measurements should be made and reported to the public regularly, with early indicators to provoke quick intervention before there is further and significant harm to Canadians. A monitoring process must be undertaken early and not wait until after years of implementation where harm can continue unnoticed and unaddressed. It is suggested that the evaluation is conducted within 12 months of implementation of the Updated Guidelines and as part of the PMPRB's annual reporting for the first five years following implementation and regularly moving forward. Monitoring and evaluation processes must address these fundamental questions (as also put forth by the Best Medicines Coalition):

- What has been the impact on the range of medicines made available, compared to previous levels of Canadian new medicine introductions and other countries, the timing of introductions, types of medicines, and the number and types of clinical trials conducted in Canada?
- Do the new regulatory framework and Guidelines reduce duplication, improve efficiency, and contribute to healthcare 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 medicines 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?

These monitoring and evaluation processes must encompass high standards of transparency, independence, and accountability, with thorough reporting. All stakeholders, including patient communities, 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.

Summary

The Medicines Access Coalition – BC (MedAccessBC) also supports and endorses the submission of the Best Medicines Coalition (BMC). Furthermore, we request that the PMPRB consider a phased implementation beginning with the application of the new PMPRB11 and measure its impact and savings, gathering more knowledge on impacts before proceeding to other more complex Guideline implementations. Comprehensive monitoring of the impact of the PMPRB Guideline changes must be undertaken to identify detrimental effects early so any harm to Canadians resulting from reduced access to medicines is avoided. Increased transparency and accountability by the PMPRB are needed as additional complexities are introduced to the review process. Patient representatives and patient organizations should be engaged to provide valuable input and insight in an unbiased manner to help steer the process, especially where the decisions may be largely subjective. We would be pleased to be involved in the development and implementation of mechanisms to monitor and evaluate these changes as they are implemented.

We are grateful for the opportunity to provide this submission and are open to further dialogue with you or your staff.

Sincerely,

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See list of coalition members on following page.

Members of Medicines Access Coalition – BC (formerly Better Pharmacare Coalition)

aHUS Canada
BC Coalition of Osteoporosis Physicians
BC Lung Association
BC Schizophrenia Society
Canadian Cancer Survivor Network
Canadian PKU and Allied Disorders
Canadian Psoriasis Network
Canadian Pulmonary Fibrosis Foundation
Canadian Skin Patient Alliance
Canadian Society of Intestinal Research
Canadian Spondylitis Association
Crohn's and Colitis Canada
Diabetes Canada
Gastrointestinal Society
HeartLife Foundation
Hep C BC
Kidney Cancer Canada
Kidney Foundation of Canada
Mood Disorders/Lookout Society
MS Society
Obesity Canada
Osteoporosis Canada
Pacific Hepatitis Network
Pain BC
Parkinson Society British Columbia
Prostate Cancer Foundation BC
Save Your Skin Foundation
Women's Health Initiative Network