Health Charities Coalition of Canada
Coalition canadienne des organismes de bienfaisance en santé

Brief submitted to:

House of Commons Standing Committee on Health

Consultation on:

Patented Medicine Prices Review Board Guidelines

Submitted by:

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Ottawa

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Executive Summary

Health charities have a mission to improve the health of people affected by disease. We represent Canadians with a wide range of medical conditions, many of whom depend on prescription drugs to live longer, better and healthier lives. The aim of providing accessible, affordable and appropriate drug therapies to Canadians is laudable. Our members support the modernization of pricing regulations affecting patented medicines when the goal is to enhance access for patients at lower cost.

Throughout the consultation process our members were pleased to provide significant feedback into the consultation process. We are disappointed that patient voices were marginalized during the long consultation process and that, more significantly, concerns raised in the initial consultations remain unaddressed. We believe that our system can be better than this and we strongly recommend that PMPRB make an immediate and profound commitment to ensure meaningful and continuous patient engagement in their mandate.

We agree with securing lower drug prices in Canada by implementing the new basket of comparator countries. We strongly recommend delaying the use of the proposed economic factors to determine a maximum drug price in Canada until the effect of using the new comparator countries has been evaluated.

The uncertainty that these regulations create cannot be ignored. We are concerned that the new pricing tests will result in drastically lower prices for new treatments, resulting in an unattractive market for manufacturers, thus many medications may not be available in Canada for years after other developed countries, or perhaps at all. This will have a serious negative effect on Canadian patients who need new medicines to improve their quality of life, and for many to extend or save their lives.

Canada takes great pride in being a world leader in health research and discovery. We are concerned that the new changes may severely reduce new investments in pharmaceutical research in Canada, which will erode our world-leading research infrastructure. It is crucial to understand the impact of these changes on the health research environment and on the impacts to the health of Canadians before they are fully implemented.

Accordingly, HCCC makes the following four recommendations vital to Canadian patients:

Recommendation #1:
That the PMPRB undertake a stepwise approach to its proposed changes by initially enacting only the changes to the comparator countries. Once the impact of this change is fully understood and if the objective of lowering Canadian prices sufficiently has not been met, then other new elements could be considered.

Recommendation #2:
That a multi-stakeholder dialogue be established to evaluate the impact of the changes on availability of medicines and specifically to inform any decision on whether and how to implement the use of the new economic criteria.

Recommendation #3

That the Federal Government require PMPRB to hire an independent third party to conduct a formal assessment of the potential and real-time impacts of the reforms on access to therapies and research investment (including clinical trials) in Canada.

Recommendation #4:

That the Federal Government require that PMPRB, along with other appropriate agencies, immediately establish a formal mechanism for meaningfully and continuously engaging patient representatives in its decision-making and processes to ensure patient voice, choice and representation.

Detailed Brief

The Health Charities Coalition of Canada (HCCC) is pleased to provide input to the Standing Committee on Health PMPRB study. Information contained herein is a summary of recent recommendations that were supported by various HCCC members as acknowledged in previous submissions. As there is diversity in how diseases are treated, the size of the patient population and the availability of treatments for various diseases, it is anticipated that many HCCC members will also provide a disease specific submission to the Committee.

Access to medicines is an important issue for our members and to the Canadians that they serve. Prescription drugs can manage conditions, cure disease(s), improve quality of life, shorten or prevent time spent in hospitals and reduce the demand for health care services, potentially leading to positive health outcomes and decreased costs to the healthcare system. An effective and sustainable drug approval process is key in being able to provide timely access to medicines for Canadians.

While there are several organizations that play pivotal roles in the drug approval process in Canada, the Patented Medicine Prices Review Board (PMPRB) is uniquely positioned to regulate the ceiling price of patented medicines and act as a protector for Canadians in ensuring that excessive drug prices are not being charged to Canadians. In this context, *excessive drug pricing* refers to Canadian prices for patented medicines in comparison to the pricing for the same drug in the identified comparator countries as set out in the Guidelines.[1]

Under the Patent Act, the PMPRB was established to 1) regulate the price of patented medicines sold in Canada to ensure that they are not excessive based on the criteria outlined in Section 85 of the Patent Act and 2) to report to Parliament through the Minister of Health.

Within the drug approval process, the PMPRB fulfills a role that is distinct from other agencies. Under the current process, Health Canada is responsible for determining market approval and has oversight for product safety, effectiveness and quality. Health technology assessments are conducted by the Canadian Agency for Drugs and Technologies in Health (CADTH) and the Institut national d'excellence en santé et en services sociaux (INESSS) (in the province of Québec) to determine the clinical and cost effectiveness of a drug and make recommendations for its future usage. Once drugs are recommended for use, the pan-Canadian Pharmaceutical Alliance (pCPA) is responsible for negotiating the cost of the drug and finally individual drug plans make the determination on whether or not to list the drug on their formulary. Each agency represented in the drug approval process fulfils a key function. **Health charities support the unique role that the PMPRB plays in setting the ceiling price for the sale of patented medicines in Canada.**

While the focus of the current PMPRB consultations is on the modernization of the guidelines, it is important to also examine the key function that PMPRB plays in protecting consumers from excessive patent drug costs as well as to understand the impact that any changes to the guidelines may have in establishing access to necessary and innovative medicines for Canadians.

From the perspective of the patients that we serve, excessive pricing is not defined within the limitations of the current guidelines but speaks to the ability of Canadians to afford the medications that

[1]PMPRB Compendium of Policies, Guidelines and Procedures http://www.pmprb-cepmb.gc.ca/CMFiles/Compendium_Feb_2017_EN.pdf

they need to manage/cure their respective disease with the ultimate goal of improved health outcomes. With this in mind, we ask that the PMPRB consider how it will continue to protect consumers from the rising cost of new drugs and emerging therapies based on the criteria set out in Section 85 while ensuring access.

Additionally, it was our hope that the PMPRB consultation would stimulate a discussion on the changes that have taken place in the PMPRB's operating environment since its inception. As this was the first time in twenty years that the Regulations and related guidelines were to be updated, we wanted to ensure that they would be revised in a manner that allows for a flexible framework that enables the assessment of further evolution of therapies.

A major change in the healthcare environment has been the move to integrate patient partnerships as a key component of healthcare reforms. Patients bring a "lived experience" to the table and are able to provide input and solutions from the perspective of the end-user. Increasingly, patient partnerships are being developed and applied at the individual, organizational and system levels. For example, in the current drug approval process, patients bring valued perspectives to the Health Technology Assessment conducted by CADTH. **Throughout the consultations we have repeatedly recommended that the PMPRB seek opportunities to meaningfully and continuously engage patient representatives in their decision making and regulatory processes. Meaningful engagement goes beyond holding briefings and calls for submissions. It is the conscious decision to include patients in identifying gaps and problems, setting priorities and working together to identify and implement solutions**. Engaging and including patients/consumers is an important aspect of maintaining the vital role of a consumer protection agency in today's environment.

The issue of pricing of drugs in Canada – particularly patented medicines which offer the most innovation and hope to those facing serious medical challenges – is of vital importance to the millions of Canadian patients represented by HCCC members. These drugs must be affordable for both the health system and for individual patients. However, affordability is only one element that requires consideration. Timeliness to access and availability of medications through Canada's public and private drug plans are also important considerations. Throughout the PMPRB consultation process, the Health Charities Coalition of Canada has provided several recommendations that address the impact on: affordability, availability of medicines, research and meaningful consultation with patient groups. A complete summary of recommendations made to the various calls for feedback since 2016 can be found in Attachment A.

Impact on Affordability

Canadian patients share governments' concerns about the affordability of medications, and they support policy efforts intended to lower prescription drug prices. However, such efforts must be balanced in such a way as to encourage continual innovation and the launch and uptick of new medicines into the Canadian market, as discussed further in the sections on availability and research below. HCCC expressed these concerns in its brief to the consultations on the initial draft of the new regulations in 2017 and is distressed that these concerns have neither been addressed in the final regulations nor potentially ameliorated by the guidelines currently under discussion.

On several occasions, our members expressed concern around the use of the Quality Adjusted Life Year (QALY) in determining pharmacoeconomic value as many Canadians, especially those living with rare diseases, would be unduly harmed by this form of assessment. Use of the QALY would mean that all

therapies are reviewed equally irrespective of important considerations such as disease severity, rarity, treatment options, appropriate success criteria and other factors. The greatest impact being that a policy that is intended to support Canadians and help them to gain better access to medicines will have a significant negative impact for individuals living with rare diseases because of the methodology that is used to assess their medications. QALY measurements also adversely impact end of life treatments, where there is less time to accrue a benefit. It is still unclear to us what measures are being put in place to respond to this notable inequity.

As previously noted, the QALY-based evaluation does not account for some important metrics to patients, such as the frequency of taking the medication and/or the delivery mechanism (oral/injectable etc.), and side effects of taking the medication. The subjectivity of QALYs is often debated as well. This measure does not always capture all benefits of a healthcare intervention. Often, it is assumed that all QALYs are representative of the same societal value of quality of life. This metric ignores the equity concerns of all patients, irrespective of the disease or condition that they are living with. We fear this change in the Regulations only widens the gaps of equity and access to medicines for Canadians.

Changing the basket of comparator countries used by the PMPRB will have, as the PMPRB has explained, the goal of dropping Canada's prices to or below the median of countries in the Organization for Economic Cooperation and Development (OECD). This will represent a price drop of at least 20%, which is substantial, both for the benefits that will accrue to patients and the health system[2]. Given this, HCCC does not understand the rationale for implementing additional new and untried measures to reduce prices further through reliance on factors such as pharmacoeconomics and market size until such time that the impact of the initial change is fully understood.

Recommendation #1:

That the PMPRB undertake a step-wise approach to its proposed changes by initially enacting only the changes to the comparator countries. Once the impact of this change is fully understood and if the objective of lowering Canadian prices sufficiently has not been met, then other new elements could be considered.

Impact on Availability

Access to new medicines and choice of treatment options are key considerations for patients. As previously submitted, HCCC remains concerned that the application of new economic factors as proposed in the draft guidelines will restrain both considerably.

Under the new schematic, an interim maximum list price is initially set based on the maximum list price of the available PMPRB11 prices. Medicines are then classified into Category 1 or 2 based on whether they have an annual cost and/or estimated market size above the designated threshold. Drugs that fall into Category 1 are then subjected to the new Section 85 factors (pharmacoeconomic, market size and GDP). HCCC remains concerned about the impact of the addition of the economic factors on the assessment process for specific patient populations, such as those living with rare diseases or accessing precision medicines therapies, as these medicines are typically found to be "cost-ineffective" according

[2] Life Sciences Ontario | IQVIA | New Medicine Launches: Canada in a Global Context | June 2020 | Copyright © 2020 IQVIA or its affiliates. All rights reserved

to the methodology used and will be subject to greater price reductions. These apparent inequities will create further barriers to availability for these patients.

Under the previous maximum price assessment regime, medicines were categorized as being breakthrough, showing substantial improvement, moderate improvement or slight/no improvement over current therapy and were allowed maximum prices accordingly. In the new schema, medicines are evaluated under one of two categories. The reintroduction of the Therapeutic Criteria Level scale provides some allowances for therapeutic innovation and is a step in the right direction. However, there remain concerns that the currently proposed model falls short in taking into consideration the impacts that will be experienced by specific populations, especially for those requiring precision drugs, drugs for rare disorders or other high-cost specialized therapies.

Some patients are concerned that such significant decreases in price will result in delays in manufacturers launching their product in Canada and this will have a negative impact on the overall length of time that it takes for Canadians to have access to new medicines in Canada, if at all. These fears are borne out by the results of a survey announced on February 3, 2020, by Life Sciences Ontario. In a survey of senior executives from 36 Canadian biopharmaceutical companies, 97% said the changes would have a negative impact on their company's ability to launch or supply medicines in Canada, with 74% saying the negative effect would be "significant." The survey also revealed these negative decisions are already being taken.[3]

Currently, many Canadians access specialty medicines through special access programs that operate across several jurisdictions in Canada. It is unknown what the impact of the proposed changes will be to the special access programs and to the Canadians who rely on these programs to improve their health. However, the same Life Sciences Ontario survey noted above revealed that 70% of executives believe the changes will have a negative effect on their ability to provide compassionate access programs (55% significantly negative) and 73% believe they will have a negative effect on patient support programs (35% significantly negative).[4]

In order to best understand the full impact of the how the implementation of the new guidelines will impact the availability of drugs to Canadians, it is recommended that the Government of Canada instruct PMPRB to convene an on-going multi-stakeholder dialogue to evaluate the impact of the changes on availability of medicines and specifically to inform on any decision on how to implement the use of the new economic criteria.

Recommendation #2:
That a multi-stakeholder dialogue be established to evaluate the impact of the changes on availability of medicines and specifically to inform any decision on whether and how to implement the use of the new economic criteria.

[3] Life Sciences Ontario, New federal drug pricing rules are already delaying medicine launches and costing jobs in Canada, survey reveals, press release, Feb. 3, 2020, at: https://lifesciencesontario.ca/news/new-federal-drug-pricing-rules-are-already-delaying-medicine-launches-and-costing-jobs-in-canada-survey-reveals/
[4] Ibid.

Impact on Research

Members of HCCC are co-funders, with governments and other investors, of some of the most important leading health research in Canada. Together with their many partners, HCCC members translate knowledge gathered through research to advocate for better public policy and better health outcomes for Canadians. Members of HCCC invest more than $155 million annually in health research, including funding ground-breaking new scientific approaches that contribute to the discovery of new and better medicines.

In addition to compromising patient access to new therapies, HCCC is deeply concerned about the impact the pricing changes will have on the health research infrastructure of Canada that has been built to world-class standards over the past 30 years. HCCC members count on the availability of this infrastructure to allow its own investments in research to be as efficient and cost-effective as possible.

HCCC has concerns that the new pricing regulations and guidelines will result in pharmaceutical companies drastically curtailing research investment in Canada. This will not only deprive Canadian patients of an important means of access to new innovative medicines through clinical studies, it will lead to the dissolution of much of the research infrastructure that has been established with so much effort and care. This will not only cost Canada in terms of jobs and expertise, but it will make other health research, such as that financed by HCCC members, less efficient and more expensive – and in some cases impossible if the required infrastructure for it ceases to exist.

These fears about the impact on clinical research are also borne out by the Life Sciences Ontario survey cited above. In that survey, 91% of pharmaceutical executives said the changes would have a negative effect on clinical research in Canada, with 44% saying the negative effect would be "significant."[5] It is imperative that an analysis of the impacts of the changes to the guidelines to the health innovation ecosystem is understood and taken into consideration.

Recommendation #3:
That the Federal Government require PMPRB to hire an independent third party to conduct a formal assessment of the potential and real-time impacts of the reforms on access to therapies and research investment (including clinical trials) in Canada.

Impact on Meaningful Consultation with Patient Groups

HCCC was profoundly disappointed that the detailed and thorough input and recommendations it and other patient groups provided on the draft regulations were not reflected in the final approved version. As a result, many of those same concerns remain, as do very serious doubts about the commitment of the PMPRB to meaningful dialogue with patients. In fact, in the latest guideline consultations patient input was marginalized into a category now known as civil society input.

It is our understanding that the PMPRB is building a Guidelines Modernization and Evaluation Process (GMEP) that will, in part, track the impact of the guideline changes on patients, healthcare providers and other stakeholders. A specific area of focus will measure Impact on Medicine Access. As organizations that work directly with patients, we are well positioned to provide valuable input to PMPRB on both the qualitative indicators that are relevant to patients as well as contribute by providing valuable

[5] Ibid.

quantitative data (such as information gathered through our registries). Unfortunately, opportunities to participate in this level of engagement and multi-stakeholder dialogue to determine how best to collectively monitor and evaluate progress going forward have not been extended to the patient community.

The time to act on this is now, yet patients are not being included in this impact process at the front end. Patients must be at the table contributing to the design and ongoing operation of such an evaluation process to ensure that it is capable of tracking such things as the timeliness of medication access, real world application of the new framework, the viability of Canada's research and development industry, and the market for innovative medicines in this country. We are extremely disappointed that a path forward is being built and that the patient voice is not being considered nor integrated into the GMEP process.

It is vital that patients play an active and meaningful role in the review recommended above that must take place before all the elements of proposed pricing regulations are implemented. Following that vital step, patients must play an ongoing formal and meaningful role to ensure their voice is heard and respected into the future.

While our members originally called for the above action in our submission on February 14, 2020 and again in our submission on August 4, 2020 opportunities for patients to engage in this level of discourse as a true and valued partner is still outstanding.

The PMPRB website notes that "The Patented Medicine Prices Review Board (PMPRB) is committed to listening to the voices and views of Canadians, and to including them in decision making. Effective and meaningful stakeholder involvement is essential to enable the PMPRB to fulfil its mandate, deliver programs, launch new initiatives, and build public trust".[6] We are calling on the PMPRB to make good on their statement and to listen to our voices and take the appropriate steps to include patients in their decision making.

Recommendation #4:
That the Federal Government require that PMPRB, along with other appropriate agencies, immediately establish a formal mechanism for meaningfully and continuously engaging patient representatives in its decision-making and processes to ensure patient voice, choice and representation.

Conclusion

The PMPRB states its vision as "a sustainable pharmaceutical system where payers have access to information they need to make smart reimbursement choices and Canadians have access to patented medicines at affordable prices." It is our sincere hope that the modernization of the guidelines will help the PMPRB attain the vision that they have set, and that Canadians will have access to patented medicines at affordable prices.

We remain committed to work with the federal government to ensure that Canadians have access to high-quality therapies and services that are appropriate for patient needs, respect an individual's choice and are delivered in a manner that is timely, safe and effective according to the most current evidence available. We strongly encourage the federal government to postpone the implementation of the

[6] https://www.canada.ca/en/patented-medicine-prices-review/services/consultations.html, accessed 2020 -11 -04.

Regulations until such time that the inter-relationship between changes made to the Regulations and the and the full impact of how these changes will impact drug access and Canada's health research and innovation ecosystem is clearly identified.

Organizational Information

The Health Charities Coalition of Canada (HCCC) is a member-based organization comprised of national health charities which represent the voice of patients at all levels of the health continuum. The health charities that HCCC represents strengthen the voice of Canadians, patients and caregivers, and work with others to enhance health policy and increase investment in health research. HCCC strives to ensure that the federal government and policy makers look to the Coalition and its members for timely advice and leadership on major health issues of concern to Canadians; and that they recognize the expertise, commitment, and contributions of health charities in improving the health and well-being of Canadians.

Overview of HCCC Recommendations to the PMPRB Consultations

HCCC Recommendation	How was the recommendation addressed/implemented?
Input into the PMPRB Guidelines Modernization Discussion paper: October 31, 2016	
HCCC recommends that the PMPRB seek opportunities to meaningfully and continuously engage patient representatives in their decision making and regulatory processes	
Submission to Patented Medicines Regulations Consultation on Proposed Amendments: June 28, 2017	
HCCC recommends that the Regulations be revised in a manner that would allow for the continued introduction of innovative medicines in Canada and be responsive to: • Allow for modifications to account for real world evidence • Allow for shifts in treatment patterns such as the emergence of precision medicine • Meet the health care needs of individual patients	The final guidelines will utilize the additional factors that are based on QALYs and market size and it is uncertain how the needs of individuals will be taken into consideration.
HCCC recommends that no duplication be accepted regarding the respective actions and evaluations followed by each of the various organizations involved in the Canadian medication review and regulation process.	PMPRB will utilize the CADTH/INESSS evaluations
HCCC recommends that the PMPRB establish a formal mechanism for meaningfully and continuously engaging patient representatives in its decision making and regulatory processes to ensure patient voice, choice, and representation.	
HCCC recommends that the Government of Canada provide patients with relevant knowledge to help them make informed decisions regarding input into future submissions before any changes are made. Also that updates to the Regulations must be undertaken in a fully transparent manner.	
HCCC recommends that any current or proposed factors used to regulate excessive medication pricing in Canada should be complementary to the existing regulatory mechanisms, such as HTA processes. The important role and relevance of HTA reviews should not be duplicated by the PMPRB.	PMPRB is utilizing the CADTH/INESSS evaluations

HCCC recommends that any changes made to the Regulations should not have a negative effect on the overall length of time for medications to reach Canadian patients.	
HCCC recommends that any analysis of the value of a medication for pricing purposes should reflect the full value of the treatment to individual patients and the healthcare system.	
HCCC recommends that the Regulations include provisions to ensure that Canadians with rare diseases are not further disadvantaged.	
HCCC recommends that: • In addition to market size considerations, the needs of patients should also be taken into account. • Any analysis of the market by PMPRB must consider that one medication may not meet the needs of all patients living with that respective disease or disorder.	
HCCC recommends that: • Any amendments to the Regulations must be evaluated in advance to ensure that their inclusion will not delay market launches in Canada. • The PMPRB should ensure that any changes to the schedule of comparator countries does not affect Canada's launch standing internationally. • Selected comparator countries should have comparable health systems overall to Canada's.	
Submission to PMPRB Draft Guidelines Consultation: February 12, 2020	
HCCC recommends that the PMPRB undertake a stepwise approach to its proposed changes by initially enacting only the changes to the comparator countries. Once the impact of this change is fully understood and if the objective of lowering Canadian prices sufficiently has not been met, then other new elements could be considered.	All changes will be implemented simultaneously.
HCCC recommends that a multi-stakeholder dialogue be established to evaluate the impact of the changes on availability of medicines and specifically to inform any decision on whether and how to implement the use of the new economic criteria.	

HCCC recommends that the Federal Government require PMPRB to hire a third party to conduct a formal assessment of the potential and real-time impacts of the reforms on research investment and activity in Canada (including clinical trials).	
HCCC recommends that the Federal Government require that PMPRB, along with other appropriate agencies, immediately establish a formal mechanism for meaningfully and continuously engaging patient representatives in its decision-making and processes to ensure patient voice, choice and representation.	
Submission to PMPRB Guidelines Consultation: August 4, 2020.	
HCCC recommends that the PMPRB undertake a stepwise approach to its proposed changes by initially enacting only the changes to the comparator countries. Once the impact of this change is fully understood and if the objective of lowering Canadian prices sufficiently has not been met, then other new elements could be considered.	All changes will be implemented simultaneously
HCCC recommends that a multi-stakeholder dialogue be established to evaluate the impact of the changes on availability of medicines and specifically to inform any decision on whether and how to implement the use of the new economic criteria.	
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