September 11, 2024

Response to PMPRB Phase 2 consultation on new Guidelines

Thank you on behalf of the participating patient organizations, Save your Skin Foundation, All.Can Canada, and ACTION for the opportunity to provide a written submission regarding the proposed changes to the PMPRB Guidelines develop pursuant to the *Patent Act* and the Patented Medicines Regulations. Below, we have set out our analysis of pertinent sections.

Section 2.2 Role of Guidelines

We support the two objectives set out on page 5, but would add that there should be a reference to patients requiring transparency and predictability of the process that PMPRB typically engages in, to identify patented medicines that may be at a greater risk for excessive pricing through its in depth review. Patients are ultimately the group directly implicated in any procedure pathways put in place that may well impact the time to accessing safe and effective badly needed treatments. Oncology patients are among the concerned patient groups, particularly due to its high incidence and prevalence as well as the need for expeditious access to provide the best chance for improved outcomes. With the advent of precision medicine and companion diagnostics ready for market and in development, this is more important than ever.

To be clear, the participating organizations to this submission are by no means supporting excessive drug prices. An efficient and effective mechanism to ensure that this does not happen is foundational. We would request that patient groups impacted by the review be advised of the review.

We are not suggesting that patients be involved in conducting the review, but that they be involved in the process by being given an opportunity to explain their views about the drug under consideration. These patients have likely accessed the drug through clinical trials or through special access programs. CDA has begun to provide this opportunity and it seems to be a very successful process. This is particularly important given **5.4 Section D: In-Depth Review**, which appears to describe a process where the issue of excessive pricing will be decided on a case by case basis, balancing all price comparisons based on Section 85(1) factors. Patient may well have information relevant to the manner in which the Board should weigh therapeutic comparators under section 85(1) compared to other factors.

While it is true that the provincial and federal courts provided guidance to PMPRB about the scope its mandate and authority, it is still in the PMPRB's mandate to create the basket of countries to consider when determining whether drug prices is excessive. It is unfortunate that PMPRB has kept the 11 countries it has used previously rather than using the new basket of companies in earlier Guideline proposals. Taking the U.S. out of the basket alone will generally create an opportunity for lower drug prices for drugs approved for sale in Canada by Health Canada.

Section 4. Valued rights: Addressing Important Topics Beyond PMPRB's Decision making Authority

We completely agree that while PMPRB should certainly be aware of other government initiatives, the law does not permit PMPRB to factor them into the Guidelines

We appreciate the PMPRB's recognition for the need for meaningful stakeholder engagement. We often lack the expertise to understand policies and procedures without the support of specific expertise including in the areas of health economics. Of course, patient organizations are generally not requesting or expecting direct involvement in Board decision making.

We welcome the offer by the Chairperson to hold a twice-yearly consultative discussion group with patient groups. We also appreciate the offer of the Director General of the Board to make themselves available to answer specific questions of patient groups on any question about the Board or the Guidelines.

Patient groups: Access to medicines

We strongly urge the Board to solicit patient input and consult with them. We especially would appreciate access to information and resources that will assist us in understanding proposals made by PMPRB for policy changes that require specific expertise including health economics experts.

Section 6.1.3 Contextualization of potential TTC

Patient groups are supportive of the consideration of price comparisons including prices of therapeutic class comparators when focusing on excessive pricing, bearing in mind that in doing so, there may be variations in the degree of comparability between drugs in the same class. We agree that a mechanism must be in place to recognize these variations. Part of that mechanism should be included as one of the factors that patient groups can address under Recommendation #2 below.

Patient groups support Option 2 *i.e.* each comparator will be assigned a level of similarity. Even within classes of drugs, the list of factors defining a level of similarity may well be different between drugs in that class. The list of factors on page 28 – Option 1 are all relevant factors to be considered on a case by case basis.

The Board must also recognize that Phase III randomized trials are not always feasible or appropriate due to the number of participants available for the trial *e.g*. rare diseases, precision medicine trials, trials where companion diagnostics including genomic testing can predict outcomes. Trials are often designed to recognize these limitations such as basket trials. These factors must be taken into account by the Board in appropriate cases.

Where the Board needs external expertise in specific cases it should have the authority to do so.

Recommendations

Recommendation #1 – Patient groups impacted by a review of a drug under in-depth review be advised of this review.

Recommendation #2 – Permit relevant patients an opportunity to explain their views about the drug under consideration as part of the government's goal of being patient centric in its work, following the practice CDA has begun to permit this opportunity.

Recommendation #3 – Replace the new basket of 11 countries with the 7 countries proposed in previous draft Guideline Regulation changes.

Recommendation #4 – Provide access to information and resources that will assist patient groups to understand policies and procedures proposed or implemented by PMPRB that require specific expertise including health economics experts in order to ensure meaningful patient engagement, as requested.

Recommendation #5 – Implement a formal policy of holding a twice-yearly consultative discussion group with patient groups and as proposed on page 10 and the offer of the Director General of the Board to make themselves available to answer specific questions of patient groups on any question about the Board or the Guidelines.

Recommendation #6 – Adopt Option 2 to compare similarity and include the list of factors set out under Option 1 to evaluate clinical evidence.

Recommendation #7 – Consider Phase II clinical trials in deliberations and designs other than randomized control head to head trials, such as basket trials, where appropriate.

Option Selection

The patient groups only specifically chose Option 2 for topic 5 but have the above additional recommendations to make.

Yours truly,

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