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Ms. Anie Perrault
Acting Chairperson, Patented Medicines Price Review Board
333 Laurier Avenue West, Suite 1400
Ottawa, Ontario
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March 19, 2025

Input on: ***Draft Guidelines for PMPRB Staff*** *(December 2024).*

Dear Chairperson Perrault,

On behalf of Myeloma Canada and our patient community, we would like to thank the PMPRB leadership for this opportunity to provide feedback on the Draft Guidelines.

We appreciate the efforts to include patient perspectives in this process, as patients are ultimately the beneficiaries of the PMPRB's overarching goal—monitoring/curtailing excessive drug prices. We fully support this goal and are hopeful that, in practice, the new Guidelines will effectively balance the need for lower drug costs, with the potential impact on patients' access to live-saving treatments.

In view of these efforts, we hope the PMPRB still intends to hold twice-yearly consultative discussion groups with patient organizations to "*share information about the issues and decisions before the Board, particularly on the structure and implementation of the Guidelines, and their expected impact.*" (From 2024 Discussion Guide). These discussions should not be restricted to 'expected impact' but should continue following the implementation of new Guidelines, to ensure that any *actual* impacts can be discussed and addressed. PMPRB should clarify if and how it plans to respond to the comments shared in these meetings and apply them to its work administering the new guidelines.

If there are no specific plans review the impact of the new Guidelines following their implementation, these should be made. The process of developing these Guidelines was a long one, imaginably no more so than for members of the PMPRB and Staff. To avoid similarly extensive processes in the future, while ensuring the Guidelines can respond to contemporary changes in Canada's socioeconomic landscape, the PMPRB should, in collaboration with stakeholders, review the impact of the new Guidelines at set intervals, and make minor adjustments if necessary.

Again, we are grateful to the PMPRB for their consistent inclusion of stakeholders through the Guidelines' development, and we look forward to further engaging with PMPRB in the near future to discuss their real-world impact.

Regards,

Martine Elias
Chief Executive Officer, Myeloma Canada
on behalf of the Myeloma Canada National Advocacy Committee

First Step

We broadly agree with the Guidelines on procedures outlined for the First Step:

1. Basket of Scheduled Countries — The selection of the PMPRB11 basket of comparator countries for drug prices is appropriate as it includes similarly situated countries possessing healthcare systems and pricing practices aligned with Canada's. PMPRB should set in advance a timeline for reviewing the appropriateness of the basket, ex. every 10 years.
2. First Step Price comparison—Using the highest international price (HIP), determined by list prices from these countries official formularies is a suitable benchmark for identifying *potentially* excessive prices at this stage.
3. Annual Review—Conducting an annual review of list prices for new and existing medicines, including adjustments for inflation (CPI), is reasonable.

Second Step

We broadly agree with the Guidelines on procedures outlined for the Second Step:

In-Depth Review

1. Complaints Process (65–71)

   We agree that it should be possible to trigger an in-depth review based on a complaint, even if the list price doesn't exceed the HIP, but we are concerned that these complaints can only come from those with existing political and/or institutional power. This unfairly advantages the interests of groups who already possess the connections and resources to effectively advocate for their concern. We understand the desire to limit the administrative burden on the staff and ensure the integrity of complaints, yet still feel it is crucial to include a pathway for patients (as members of the general public) to submit 'complaints' based on their own experience. Particularly considering the difficulties PMPRB has expressed regarding its limited capacity to involve patients in the review process, this would be the only

formal process by which the PMPRB can hear directly from those personally impacted by the price of a drug before advancing to a price hearing. We propose a compromise wherein the PMPRB still receives 'complaints' from "*other parties who have concerns about the list prices of specific patented medicines".* When any specific patented medicine reaches the stage of in-depth review, any public complaints related to say patented medicine will be reviewed by staff in the context of the in-depth review. For clarity, and to distinguish how PMPRB will treat these 'complaints' differently, they may be named 'public concerns' with a webform for their submission provided on the PMPRB website.

2. Human Drug Advisory Panel (HDAP):

   We fully support the involvement of the HDAP, where subjective decisions are being made on the basis of clinical evidence it is imperative that PMPRB Scientific Review Staff has access to specialist clinical expertise in the field/disease area relevant to the product under review and makes use of this advice to improve their analysis, particularly in more 'complex cases.' To this end, the HDAP should be encouraged to consult external experts in the face of uncertainty. It is also critical to ensure the right experts are being consulted. The HDAP can capitalize on the knowledge of existing organizations and institutions such as patient groups, Rights Holders, CDA/INESS, etc., by a) providing a method (such as a webform) for stakeholders to submit recommendations for clinical experts to be consulted in a particular disease area, and/or b) requesting recommendations for clinical experts from relevant patient groups (without publicizing the drug under review) and rights holders on an *ad hoc* basis.

3. Domestic Therapeutic Class Comparison (dTCC) — In situations where generics and biosimilars may be the most appropriate comparators, there is a lack of clarity regarding how this may be factored into the comparison, and/or how it may impact recommendation decisions made by staff. Similarly, if the most appropriate

comparator is used as part of a combination product, it is unclear how the comparison will proceed.

4. Sources (89): PMPRB should allow Rights Holders to inform relevant patient groups when an in-depth review is initiated and request their input, or request testimonial directly from patients. This would be submitted to Staff by Rights Holders in their 'written input.' It has been confirmed verbally by PMPRB staff in the process of the preceding consultations that Rights Holders would be permitted to do so. If this is still true, we ask that the 'Sources' section of the Guidelines explicitly describes this possibility to Rights holders.

Undertakings

1. In view of improving transparency and assisting Rights Holders in understanding which Undertakings may be accepted, PMPRB should publish an annual report detailing rejected Undertakings (without identifying information attached) or summarizing common features of the rejected Undertakings and how they compare to accepted Undertakings. It should similarly be made public the number of Undertakings accepted and rejected by the Chair per year. Alternatively, after an appropriate waiting period to allow for the accumulation of data, PMPRB could hold an information session for Rights Holders to convey the same information.

Price Hearings

1. The PMPRB's limitations on inclusion of patient input have thus far been attributed to the privacy requirements of the Board's quasi-judicial nature. As such, the public price hearings are not subject to these restrictions, and PMPRB should take steps to invite patient input at this stage. Patients can provide much needed context to help the Board decide if a price is excessive, since the determination of an 'excessive' price is made subjectively and, on a case-by-case basis. Therefore, PMPRB should include formal procedures in the Guidelines requiring Staff to

publicly provide notice of upcoming hearings, so relevant patient groups may submit written input to be considered by the Board in their deliberations. This could be conducted similarly to the stakeholder input processes at CDA/INESSS with a standard window of time to prepare a submission, so patient groups may survey their community and provide relevant information to the Board in advance of the hearing date.

The Draft Guidelines state at paragraph 13, after considering the named s85 factors, if *"the Board is unable to determine if a price is excessive, subsection 85(2) of the Act stipulates that it may consider the costs of making and marketing the medicine, as well as other factors which can be specified by regulations or that the Board considers relevant in the circumstances."* While this language does not bar Board members from referring to patient perspectives if they are considered 'relevant', there is no guarantee they will be considered, and even less assurance that the Board will seek out this critical information. Similarly, it should not be left to the discretion of the Board, complainants or Rights Holders to determine which files merit the inclusion of patient input nor who will provide it.

Patient perspectives, are crucial factors to understanding if a price is *excessive* in the Canadian context and are thus relevant to the circumstances of every 'medicine' intended to treat Canadian patients and subject to the PMPRB's authority.

Importance of Patient Input

Pharmaceutical companies and insurers already have significant influence in pricing discussions, but patients—the end users—often lack representation. Especially considering the complaints mechanism allows public drug plans and insurance companies to trigger an in-depth review, if these cases proceed to hearings, it is crucial to ensure that the patient experience of a medicine's price is also considered by the Board. Including patient input

ensures that determinations of 'excessive' pricing reflect not just industry/regulatory perspectives, but also the experience and needs of those directly affected.

While insurers and public drug plans assess 'excessive' pricing from a budgetary perspective, only patients can speak to the real-world impact of a medicine and its cost on patients' lives. Patients offer insights into how pricing affects treatment adherence, overall healthcare costs and long-term harms or benefits. A drug appearing to be 'excessively' priced might enable a patient to return to work, reduce hospitalizations, or improve quality of life in ways that justify its price. Conversely, if a high-priced drug delivers minimal improvement over existing treatments, or has more serious side effects, patient perspectives can highlight concerns about value for money.

Even if insurers cover part of the cost, patients may still face significant out-of-pocket expenses— information that would not necessarily be captured by the s.85 factors and can have harmful downstream effects on patients and the health system. Patient input can reveal whether pricing creates financial hardship, limits access, or impacts treatment adherence, which are essential in determining whether a price is excessive in the Canadian context.

Particularly in a situation where the Board finds it difficult to decide if a price is excessive, it follows that the real-world impact of that price on Canadians should be a primary consideration, if not the determinative factor. Therefore, incorporating patient input at the hearing stage is essential to making fair and comprehensive determinations about 'excessive' pricing, on a case-by-case basis.