The Chronic Myelogenous Leukemia Society of Canada
Originators of CML AWARENESS DAY® – September 22 (9/22)
La Société de la Leucémie Myéloïde Chronique de Canada
L'origine de "CML AWARENESS DAY®" – le 22 septembre (9/22)

PMPRB Scoping Questions – December 2023

To Whom it may concern,

We are pleased to provide you with our comments and feedback regarding the scoping paper for the PMPRB guidelines.

As a patient advocacy organization, it is difficult for us to answer many of the questions, however, we gave serious consideration as to what elements we would like to see used to guide your decision-making process.

Above all, we would expect that the PMPRB conduct themselves in a transparent manner throughout the process. We believe that the process becomes rather lengthy because there is a gap in communication of expectations.

We look forward to a future where we can all work together collaboratively to improve Canadian patients access to innovative medicines in a timely manner that encourages innovation and stimulates this important are of healthcare.

Best Regards,

Cheryl-Anne Simoneau
President,
The CML Society of Canada
Beaconsfield, Quebec

Question 1.1: What elements of the 2010 Guidelines should be retained? Which ones and why?

From the perspective of a patient advocacy group, it is difficult to provide thorough input on this question. However, we would suggest that the PMPRB build KPI's that would allow them to establish how well their guidelines achieve the objectives of the mandate.

Specific KPIs would take into consideration:

1. **Consider the Effectiveness of Current Elements:**
    - Evaluate the effectiveness of each element in achieving the intended goals of the PMPRB.
    - If certain elements have proven successful in promoting fair pricing and access to medicines, we would advocate for their retention.
2. **Align with Public Health Goals:**
    - We would Argue for the retention of elements that align with public health objectives and contribute to affordable and accessible medicines.
3. **International Comparisons:**
    - Consider international best practices and align with elements that are consistent with successful pricing models in other countries.
4. **Stakeholder Input:**
    - Reference feedback from relevant stakeholders, such as healthcare professionals, patient groups, and the pharmaceutical industry, to support the retention of specific elements.

Question 1.2: Should new Guidelines continue to categorize medicines by therapeutic class comparator characteristics such as the Level of Therapeutic Improvement?

This question is best answered by considering the following:

1. **Consider Fairness and Consistency:**
    - Evaluate whether categorizing medicines by therapeutic class is fair and consistent in assessing their value and pricing.
2. **Clinical Relevance:**
    - Assess the clinical relevance of categorizing medicines based on therapeutic class and the Level of Therapeutic Improvement.
3. **Adaptability:**
    - Consider whether this approach allows for adaptability to advancements in medical science and emerging treatment modalities.
4. **Simplicity and Transparency:**
    - Consider whether the approach contributes to simplicity and transparency in the pricing evaluation process.

Question 1.3: Should the Board accord more weight to one or more of the factors set out in s. 85 of the Act in designing the Guidelines?

We agree that is important to adhere to the legal framework defined in section 95 of the act. We would advocate for according more weight to factors that directly serve the public interest such as, affordability, accessibility, and health outcomes. We would also need to consider the economic impact of pharmaceutical pricing on the healthcare system, and we would argue to giving more weight to factors that address cost effectiveness. It is important to work transparently with all stakeholders in the decision-making process and according weight to factors that reflect diverse perspectives.

Question 1.4: If international prices are used as the initial triage measure for commencing investigations, what price levels within the PMPRB11 should be used as the triage measure? (e.g. HIP or MIP?)

As a patient advocacy group, it is difficult to answer this question, but we would like to see the following be taken into consideration in order for the PMPRB to make the best decision for Canadian Patients:

- **Consideration of Price Levels:**
    - The PMPRB should Evaluate the merits of using different price levels within the PMPRB11, such as the Highest International Price (HIP) or the Median International Price (MIP).
    - The PMPRB should review the advantages and disadvantages of each measure, considering factors like accuracy, fairness, and relevance to Canadian healthcare.
- **Alignment with Objectives:**
    - The PMPRB should use a triage measure that best aligns with the PMPRB's objectives, which may include ensuring affordability, promoting access, and fostering innovation.
- **International Benchmarking:**
    - The PMPRB should place importance of international benchmarking to determine a fair and reasonable price for patented medicines in Canada.
- **Consideration of Health Outcomes:**
    - The PMPRB should evaluate how the chosen triage measure reflects or influences health outcomes for Canadians, ensuring that it supports positive patient outcomes.

Question 1.5: How should the PMPRB conduct an initial review and monitor the prices of patented medicines that have few or no international prices?

We would suggest that the PMPRB should consider **Adaptive Pricing Models:**
We are thinking about a Risk Sharing strategy which is designed to share the financial risks

between pharmaceutical manufacturers and payers, such as health insurance providers or government agencies. The risk sharing strategy would contain some of the following elements: **performance metrics, Outcome-Based Pricing, Financial Risk Sharing, Flexibility in Pricing.** It is crucial that these agreements be transparent and must be negotiated between the pharmaceutical company and the payers in presenting a risk sharing strategy to the PMPRB.

This entire process requires collaboration with all stakeholders.

Question 1.6: Would an expedited price review (e.g., within 90 days after initial Form 2 submission) of a new medicine based solely on international prices being below the MIP accelerate the introduction of innovative medicines?

As a patient advocacy group, we would encourage expedited reviews as a way to encourage innovation and faster market entry of new medicines. We would suggest that that there needs to be a balance for a speedy review with the necessity of maintain a rigorous evaluation process to ensure patient safety and effectiveness. There needs to be a flexible review process that allows expedited reviews when appropriate without compromising through evaluation.

There would need to be a post-expedited review timeline established for a full price review to ensure ongoing scrutiny and to make any necessary adjustments. During the entire process there needs to be a mechanism in place to collect, analyze and consider additional data over time, especially for medicines that initially underwent and expedited review.

During this entire process there needs to be transparency and accountability in the review process and accountability to stakeholders, ensuring that any adjustments to prices are well justified.

Question 2.1: Should the Guidelines distinguish between medicines that existed as of July 2022 (existing medicines) and medicines introduced afterwards (new medicines)?

As a Patient advocacy group, we would favor the consideration of Grandfathering as it offers benefits to all stakeholders while the PMPRB still achieves the objectives of their mandate. This offers predictability for the pharmaceutical company. This stability allows companies to plan and manage their pricing strategies with greater certainty. Grandfathering helps prevent retroactive adjustments to drug prices that were established under previous regulations. Retroactive changes could have significant financial implications for pharmaceutical companies. Pharmaceutical companies often make significant investments in research and development for drug discovery and approval. Grandfathering allows consideration of the pricing environment that was in place when investment decisions were made.

We would hope that the PMPRB would consider a transition period to allow for a smoother adjustment to the new guidelines without causing disruption in the pharmaceutical market. The PMPRB should consider how distinguishing between existing and new medicines might

impact incentives for innovations, ensuring that the guidelines support ongoing research and development.

The PMPRB should evaluate whether this approach aligns with international best practices in pharmaceutical pricing regulation.

Question 2.2: What approach should the Board take with respect to existing medicines with prices above the HIP of the PMPRB11? Should the Board review these prices, and if so, how soon?

We would suggest that the PMPRB allow for a Gradual review process for existing medicines with prices above the highest international price (HIP) to avoid sudden market disruptions. Some factors should be considered such as, therapeutic importance, patient impact, and availability of alternative treatments. There would need to be a timely review of existing medicines with prices above HIP. Throughout this process the PMPRB needs to be in consultation with relevant stakeholders, including healthcare professionals and patient groups to gather input on the urgency of reviewing specific medicines. There needs to be transparency in communicating the timeline and all stakeholders must be informed and engaged throughout the process. There needs to be flexibility built into the guidelines to allow for exceptional cases.

Throughout this entire process we must all be mindful of potential impact on patient access, industry innovation, and healthcare system sustainability.

Question 3.1: How often should price reviews be conducted? (1-5 years). Should they be different for small molecules (average 10-year exclusivity period) versus biologics (average 20+ year exclusivity period)? Should they be different for medicines for rare diseases?

The PMPRB needs to consider average product life cycle and determine a reasonable frequency for price reviews. Acknowledge the differences in exclusivity periods for small molecules and biologics and we suggest a tailored review intervals that align with their respective life cycles. We also Recognize the unique challenges of medicines for rare diseases, considering their limited patient populations, we would suggest a review frequency that accounts for these circumstances.

Question 3.2: What criteria besides time should be used to trigger a price review?

Some of the criteria could be:

1. **Approval of Significant New Indication:**
    - a price review triggered by the approval of a significant new indication, recognizing that expanded indications may warrant a re-evaluation of pricing.

2. **Therapeutic Class Comparator Changes:**
    - when there is a significant change in therapeutic class comparators, ensuring that the pricing remains relevant and reflective of the market.
3. **Availability of New Evidence:**
    - the availability of new and stronger evidence related to the benefit of a medicine compared to therapeutic class comparators as a trigger for price review.
4. **Departure from Pricing Thresholds:**
    - a review when there is a departure from identified pricing thresholds, signaling the need for closer scrutiny.

Question 3.3: Should the relative weighting given to different section 85 (Patent Act) factors change over the lifecycle of a medicine?

We would hope that the PMPRB would consider dynamic weighting of section 85 factors over the lifecycle of a medicine, considering factors such as market competition, patent status, and therapeutic importance. We would Suggest flexibility in weighting factors to adapt to changing circumstances, ensuring that the assessment remains relevant and responsive to the medicine's lifecycle stage.

Question 3.4: How should the PMPRB treat the allowable Consumer Price Index increase in the context where international list prices are decreasing?

We would suggest adjustment mechanisms to adjust the allowable Consumer Price Index increase in response to decreasing international list prices, ensuring that the framework remains adaptive to market dynamics. The PMPRB should also show Consideration of External Factors influencing international list prices and adjusting the allowable increase accordingly to maintain alignment with international benchmarks.

Question 3.5: What is the ideal timing for scientific review and therapeutic comparator identification? At what price review stage(s) should scientific review be applied?

We would suggest that the PMPRB should consider, Early Scientific Review to identify therapeutic comparators and assess the medicine's clinical value, ensuring that pricing considerations align with the product's scientific merit. We would consider that PMPRB implements Continuous Evaluation of ongoing scientific reviews at key milestones, such as new indications or significant updates, to reflect evolving scientific understanding and changing treatment landscapes. Integration with price reviews at relevant stages to facilitate a comprehensive evaluation of a medicine's value proposition.

Question 4.1: Are the criteria published in the 2010 Guidelines for commencing an investigation still appropriate (assuming adjustment to PMPRB11)?

The PMPRB would need to evaluate the effectiveness of the criteria outlined in the 2010 Guidelines in achieving the goals of the PMPRB. Do these criteria align with the objectives of

the updated PMPRB11 and whether adjustments are needed to reflect changes in the pharmaceutical landscape. Consider feedback from stakeholders, including healthcare professionals, patient groups, and the pharmaceutical industry, regarding the appropriateness of the criteria. Compare the criteria to international best practices in pharmaceutical pricing regulation and adjust them accordingly.

Question 4.2: How much detail should the Guidelines set out regarding what happens once an investigation is opened?

We would hope that the PMPRB would consider the following in deciding how much detail should be included in the guidelines:

1. **Clarity and Transparency:** detailed guidelines that provide clarity and transparency regarding the process once an investigation is opened.
2. **Stakeholder Communication: i**nclude provisions for communicating with stakeholders throughout the investigation, ensuring transparency and accountability.
3. **Timelines and Milestones:** Specify timelines and milestones for different stages of the investigation, providing clear expectations for all parties involved.
4. **Flexibility in Procedure:** incorporate flexibility to adapt the investigation process to the unique circumstances of each case while maintaining a structured approach.

Question 4.3: Should the PMPRB continue to use Undertakings as an investigation closure mechanism?

In Answering this question, the PMPRB needs to consider the following:

1. **Effectiveness of Undertakings:**
    - Evaluate the effectiveness of Undertakings as a mechanism for closing investigations, considering whether they have achieved the intended outcomes.
2. **Stakeholder Input:**
    - Consider stakeholder input on the use of Undertakings, including feedback from the pharmaceutical industry, patient groups, and healthcare professionals.
3. **Alignment with PMPRB11 Objectives:**
    - Assess whether the use of Undertakings aligns with the objectives of the updated PMPRB11, adjusting the mechanism if necessary.
4. **Flexibility in Closure Mechanisms:**
    - Consider whether other closure mechanisms might be more appropriate in certain cases and propose a flexible approach that considers different circumstances.

Question 5.1: What efficiencies could be gained by coordinating decisions and timelines with CADTH, INESSS, and pCPA or insurers (public and private)?

Primarily, the PMPRB needs to consider whether given their mandate and jurisdiction are there any limitations to the PMPRB collaborating with these agencies. If they can, we would hope they consider some of the following ideas.

The coordination of decisions and timelines among PMPRB, CADTH, INESSS, and pCPA, as well as insurers, can yield significant efficiencies in the evaluation and access to patented medicines. By aligning review processes and timelines, the following efficiencies can be achieved:

1. Streamlined Processes: Coordinated efforts would avoid duplication of efforts, resulting in streamlined processes for drug evaluation, pricing negotiations, and market access.
2. Reduced Redundancies: Shared information and collaboration would minimize redundant data requests and assessments, reducing the burden on both industry stakeholders and regulatory bodies.
3. Faster Market Access: Aligning decision timelines ensures that the transition from regulatory approval to market access is more efficient, benefitting patients by accelerating access to innovative treatments.
4. Harmonized Recommendations: Coordinated efforts can lead to harmonized recommendations, providing a consistent approach to decision-making across different healthcare agencies and jurisdictions.
5. Optimized Resource Allocation: By avoiding duplication and optimizing resource allocation, regulatory agencies and insurers can use their resources more effectively, potentially leading to cost savings.

Question 5.2: How can the PMPRB optimize its presence within the Canadian bio/pharmaceutical ecosystem to support a whole-of-government approach to issues relating to patented medicines?

To optimize its presence within the Canadian bio/pharmaceutical ecosystem and contribute to a whole-of-government approach, the PMPRB can consider the following strategies:

1. Collaborative Partnerships: Actively engage in collaborative partnerships with key stakeholders, including CADTH, INESSS, pCPA, insurers, and other relevant entities. Regular meetings and joint initiatives can enhance communication and coordination.
2. Information Sharing: Establish mechanisms for seamless information sharing among regulatory bodies and health agencies. This could involve shared databases, regular reports, and collaborative platforms to ensure that all stakeholders have access to relevant and up-to-date information.
3. Integrated Policy Development: Participate in the development of integrated policies that consider both regulatory and access aspects. Collaborate on the design of policies that strike a balance between encouraging innovation and ensuring affordable access to medicines.
4. Unified Communication: Ensure unified communication strategies that convey a consistent message to industry stakeholders and the public. This helps create a transparent and predictable environment, fostering trust and cooperation.

5. Proactive Engagement: Proactively engage with industry stakeholders to understand evolving challenges and opportunities. This engagement can inform policy development and regulatory decisions, aligning them with the needs of the bio/pharmaceutical sector.
6. Whole-of-Government Forums: Participate in whole-of-government forums or task forces dedicated to addressing issues related to patented medicines. This ensures that the PMPRB's perspective is integrated into broader government strategies for healthcare and innovation."

Question 6.1: What is your experience with innovative medicines and their list prices in Canada?

As a patient advocate organization, we are aware of the need for Canada to be able to guarantee Canadian Citizens that they are not overpaying for their medicines.

Question 6.2: What role do the PMPRB Guidelines play in your decision-making process in Canada and globally (if applicable)?

This is not applicable to our organization.

Question 6.3: Should the PMPRB view the question of whether the prices of these medicines are "excessive" through a different lens than other types of medicines? What quality of evidence should the Board consider when conducting its scientific review of these medicines?

We would hope that the PMPRB would consider these suggestions:

1. **Special Considerations for Rare Diseases:**
    - We would advocate for a nuanced approach for pricing rare disease medicines, considering the unique challenges and limited patient populations involved.
2. **Balancing Innovation and Access:**
    - It is important to balance the need for fair pricing with the imperative to incentivize innovation, especially in the context of rare diseases.
3. **Quality of Evidence:**
    - The PMPRB should consider robust and comprehensive evidence in the scientific review process, including data on the medicine's efficacy, safety, and real-world impact on patients with rare diseases.

Question 6.4: How can the PMPRB better engage with you?

1. **Communication Channels:**

- The PMPRB should consider using all methods of communicating to their stakeholders using social media and any other methods of engagement, such as webinars and virtual town hall meetings.

2. **Stakeholder Consultation:**
    - It is important to have regular stakeholder consultations and seek opportunities for increased collaboration in policy development and decision-making. We propose the establishment of industry & Patient working groups or advisory committees that can actively contribute to policy development. This collaborative approach would ensure that diverse perspectives are considered, leading to more effective and well-rounded outcomes.

3. **Transparency and Clarity:**
    - We would Advocate for increased transparency in the PMPRB's processes and decisions, providing clearer guidance on expectations and requirements. Clear and comprehensive guidelines, published in accessible formats, would provide industry stakeholders with a better understanding of expectations. Additionally, transparent communication regarding the reasoning behind specific decisions would contribute to a more predictable regulatory environment.
4. **Feedback Mechanisms:**
    - There needs to be an establishment of effective feedback mechanisms to ensure that industry perspectives are considered, and concerns are addressed in a timely manner. We propose the creation of an online portal or dedicated email address for submitting feedback and inquiries. Timely responses to industry concerns would demonstrate a commitment to addressing issues promptly and maintaining an open dialogue.