

Date: February 11, 2025

To: Patented Medicine Prices Review Board (PMPRB)

Subject: Feedback on Draft Guidelines

Dear Members of the Patented Medicine Prices Review Board,

As the President of the CML Society of Canada, I appreciate the opportunity to provide feedback on the proposed PMPRB guidelines. Our organization represents patients living with Chronic Myelogenous Leukemia (CML), and we are deeply committed to advocating for their access to life-saving therapies and improved health outcomes.

After reviewing the draft guidelines, we would like to highlight several concerns and recommendations to ensure these policies balance cost control with the critical need for patient access to innovative treatments, particularly in the case of rare and life-threatening conditions such as CML.

### **1. The Unique Nature of CML Drugs**

CML treatments, such as Gleevec, Tasigna, and Sprycel, Bosulif, iClusig, Scemblix, have transformed the prognosis of this condition, turning it from a fatal condition to a manageable chronic illness. These therapies are often groundbreaking, with limited or no true therapeutic comparators due to their targeted mechanisms of action.

**Concern:** The reliance on Therapeutic Class Comparison (TCC) could unfairly penalize CML drugs if compared to less innovative or generic options that do not deliver equivalent outcomes.

**Recommendation:** We urge the PMPRB to adopt an approach that accounts for the unique therapeutic and societal value of drugs with limited comparators, ensuring their pricing reflects their transformative impact on patient lives.

### **2. Ensuring Predictability and Transparency**

While we understand the need for case-by-case hearings, the absence of clear pricing benchmarks creates uncertainty for manufacturers and stakeholders.

**Concern:** Unpredictability in pricing reviews may introduce delays and administrative challenges, directly impacting timely patient access to critical CML therapies.

**Recommendation:** The PMPRB should establish clearer criteria or thresholds to guide pricing expectations, especially for drugs treating rare conditions like CML. Increased predictability ensures that decisions are evidence-based and transparent, reducing delays.

### **3. Balancing Costs with Long-Term Outcomes**

CML treatments significantly reduce healthcare costs over time by preventing hospitalizations, complications, and condition progression. For instance, studies have demonstrated that early and sustained access to targeted therapies can reduce hospitalization rates by significant margins, resulting in substantial system-wide savings.

**Concern:** Focusing solely on initial pricing without considering long-term system savings and improved patient outcomes risks undervaluing these therapies.

**Recommendation:** Incorporate pharmacoeconomic value (as conducted by the Canada Drug Agency, so as not to duplicate important work) into pricing considerations, recognizing the health system and societal benefits of innovative drugs. This includes factoring in cost savings achieved through reduced complications and improved quality of life for patients. This is further addressed in point 7 below.

### **4. Addressing Inflationary Price Caps**

The draft guidelines' emphasis on Consumer Price Index (CPI)-based price caps may inadvertently penalize manufacturers for necessary adjustments related to inflation and production costs.

**Concern:** Small price increases to account for inflation could trigger unnecessary reviews, leading to administrative delays and potential disruptions in patient access.

**Recommendation:** Allow reasonable inflationary adjustments, especially for treatments that already demonstrate value in long-term health outcomes. Additionally, mechanisms should be put in place to distinguish between minor inflationary adjustments and genuine pricing concerns.

### **5. The Role of Patient Advocacy in the Review Process**

Patient advocacy groups like the CML Society of Canada bring invaluable perspectives to the discussion on drug pricing and access. However, the guidelines currently do not provide a formal role for patient input during reviews or hearings.

**Concern:** Without patient representation, the broader impact of pricing decisions on patient health and quality of life may be overlooked.

**Recommendation:** Create a structured mechanism for patient advocacy groups to provide input during hearings and reviews. This could follow models used by international agencies such as NICE in the UK, where patient organizations contribute directly to decision-making panels, ensuring patient-centric decisions.

### **6. Protecting Access to Innovative Therapies**

CML patients rely on continuous innovation to maintain and improve their quality of life. Any pricing policies that hinder access to next-generation therapies would be detrimental to patient health.

**Concern:** Automatic reviews triggered by international price comparisons (IPC) may not fully account for the unique circumstances of drugs designed for rare conditions.

**Recommendation:** Exempt or modify IPC criteria for drugs treating rare conditions with no viable alternatives, recognizing the unique market dynamics of these therapies. This would ensure that CML

patients continue to benefit from access to innovative treatments without unnecessary pricing-related delays.

### **7. Coordination with the Canada Drug Agency and Health Technology Assessment (HTA)**

The Canada Drug Agency (CDA) plays a critical role in conducting Health Technology Assessments (HTA) to determine the value and effectiveness of new treatments. Effective collaboration between the PMPRB and CDA is essential for consistent and informed decision-making on drug pricing and access.

**Concern:** The current draft guidelines do not specify mechanisms for sharing pricing-related information with the Canada Drug Agency, which could lead to misalignment between pricing decisions and HTA evaluations.

**Recommendation:** Establish formal channels for collaboration and information-sharing between the PMPRB and CDA to ensure that HTA insights are incorporated into pricing evaluations. This collaboration would provide a more holistic view of a drug's value, supporting decisions that optimize patient outcomes and affordability.

### **7. Alignment with the National Pharmacare Program**

The Pharmacare Act, passed in October 2024, aims to provide affordable and accessible medications to all Canadians. It is essential that the proposed guidelines complement and enhance the objectives of this program.

**Concern:** Misalignment between the PMPRB guidelines and the Pharmacare Act could create unintended barriers to affordable medication access for CML patients.

**Recommendation:** Ensure that the PMPRB's pricing policies are designed to support the objectives of the national Pharmacare program. This includes aligning affordability measures with mechanisms that maintain equitable access to essential treatments.

### **8. Emphasizing Value-Based Healthcare (VBHC)**

Value-Based Healthcare (VBHC) focuses on optimizing patient outcomes relative to the cost of care, making it a crucial consideration in discussions about drug pricing and accessibility.

**Concern:** The current guidelines do not fully incorporate VBHC principles, potentially overlooking the value of treatments that deliver long-term patient benefits and cost savings to the healthcare system.

**Recommendation:** Integrate VBHC principles into the PMPRB's pricing framework by evaluating treatments based on their outcomes, such as survival rates, reduced complications, and improved quality of life for CML patients. This would ensure that pricing decisions reflect the holistic value of innovative therapies and align with health system goals.

### **Conclusion**

The PMPRB's mandate to ensure non-excessive pricing is critical, but this goal must be balanced with protecting patient access to innovative and life-saving treatments. By refining the proposed guidelines to account for the unique characteristics of drugs like those used to treat CML, the PMPRB can better serve the needs of patients and the broader healthcare system.

We look forward to continuing to work with the PMPRB to ensure fair and equitable access to therapies for all Canadians. Please do not hesitate to contact me should you wish to discuss these recommendations further.

Sincerely,

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Founder, Board Member

**The Chronic Myelogenous Leukemia (CML) Society of Canada**

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