Vertex Pharmaceuticals (Canada) Incorporated
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March 19, 2025

Patented Medicine Prices Review Board (PMPRB)

Feedback Regarding Draft PMPRB Guidelines - Vertex Pharmaceuticals (Canada) Incorporated

Dear Sirs/Mesdames,

On behalf of Vertex Pharmaceuticals (Canada) Incorporated ("Vertex"), thank you for the opportunity to provide feedback on the Patented Medicines Prices Review Board (PMPRB) Draft Guidelines published on December 19, 2024. As with our previous submissions, our input here is intended to complement and support our other contributions, particularly those from the Canadian Forum for Rare Disease Innovators (RAREi) and BIOTECanada.

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. In Canada, we have approved therapies for cystic fibrosis, sickle cell disease, and transfusion-dependent beta thalassemia. Our company continues to advance our research into these diseases and has a robust clinical pipeline of investigational therapies across a range of modalities, including acute and neuropathic pain, APOL1-mediated kidney disease, IgA nephropathy, primary membranous nephropathy, autosomal dominant polycystic kidney disease, type 1 diabetes and myotonic dystrophy type 1. We invest most of our resources in research and development (R&D), as we believe that the true value of our industry lies in scientific innovation. This commitment is reflected in our company's structure, where 3 out of 5 employees at Vertex are dedicated to R&D.

Overall, Vertex is encouraged by the PMPRB's Draft Guidelines and welcomes the adoption of a highest international price (HIP) approach for initial and annual reviews. We also strongly support the PMPRB's guidance that patented medicines will be considered *reviewed* when an International Price Comparison (IPC) doesn't exist in any of the PMPRB11 Schedule Countries. Since more than half of new patented medicines are first launched in the United States,[1] this approach eliminates an additional barrier for companies considering the Canadian market as a Tier 1 launch destination.

While the Draft Guidelines are an improvement over previous versions, we are concerned that ambiguity in the screening and review processes could discourage local R&D investment and delay the entry of patented medicines.

[1] U.S. Department of Health & Human Services. (2024, February). Comparing New Prescription Drug Availability and Launch Timing in the United States and Other OECD Countries. https://aspe.hhs.gov/sites/default/files/documents/430a3e61c234f06270b04414e797ad3a/new-drug-availability-launch-timing.pdf

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For the Board's consideration, we have outlined several recommendations aimed at fostering a transparent and predictable regulatory environment, which should help build a more sustainable Canadian healthcare system and thriving life sciences ecosystem.

Special Provisions - Complaints

- Due to the broad scope and lack of parameters surrounding the proposed complaint mechanism, it could be misused, undermining market stability, creating confusion and diminishing trust for rights holders. Therefore, we suggest limiting complaints to Federal and Provincial Ministers of Health due to their responsibilities in leading health systems and their understanding of the PMPRB's role and mandate. We strongly discourage the inclusion of private insurers as parties permitted to file complaints. This represents a large conflict of interest that could spur anti-competitive behavior. These entities are not beholden to, or responsible for public health and most of them have a primary fiduciary duty to shareholders which the PMPRB is not mandated to protect.
- The Board should reconsider the need for a complaint induced in-depth review process for medicines exclusively funded through public processes, including hospital products. Established evaluation, negotiation, and reimbursement processes for these medicines already provide substantive protections against the risks of excessive pricing. We recognize the difficulty this may pose for PMPRB staff who cannot differentiate sale price submission data for medicines publicly covered. Therefore, we recommend the implementation of a clearly defined appeal process that allows rights holders the opportunity to provide evidence of a medicine's public coverage to resolve any concerns.

In-Depth Reviews

- Vertex is concerned that the subjectivity and lack of transparency in the in-depth review process creates significant uncertainty for rights holders evaluating Canada as a launch destination.
- We recommend that the application of international or domestic therapeutic class comparisons for all reviews be determined in consultation with the Human Drug Advisory Panel (HDAP) and a cohort of independent, arms-length clinicians with an expertise in the relevant therapeutic area and experience in providing care for patients affected by the condition. Obtaining this input is essential, as PMPRB staff lack the necessary expertise and practical care experience to provide recommendations across all therapeutic areas and modalities.
- It is also essential that therapeutic class comparison tests exclude medicines that are generic or have become genericized because their inclusion:
    - Undermines the policy intent of the Patent Act, which is to grant time-limited market exclusivity to incentivize the development and marketing of new innovations.

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- Distorts market dynamics and Canada's competitiveness. Price tests using generics may artificially lower price ceilings, reducing incentives for companies to launch new medicines, harm trade relationships, and deter foreign investment.
- May limit patient access to novel treatments by artificially devaluing the patented medicine.

Flexible Pricing Considerations

- In exceptional circumstances, such as public health emergencies and cross-border trade disputes, the PMPRB should adopt a broader economic perspective when assessing prices. Rigid pricing frameworks that fail to account for sudden supply chain disruptions and increased production costs can lead to shortages or delays in access to critical medicines. In times of crisis, manufacturers may encounter higher raw materials' costs and distribution challenges due to unforeseen tariffs, and exchange rate shifts, underscoring the need for pricing flexibility to sustain innovation and access to important medicines.

Thank you for considering our feedback. Should any questions arise, we would welcome the opportunity to meet with the PMPRB to discuss our recommendations in greater detail.

Sincerely,

Michael Siauw
General Manager
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