Vertex Pharmaceuticals (Canada) Incorporated
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Patented Medicine Prices Review Board
Box L40
Standard Life Centre
333 Laurier Avenue West, Suite 1400
Ottawa, Ontario K1P 1C1

Feedback Regarding the Discussion Guide for PMPRB Phase 2 Consultations on New 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) Discussion Guide posted for consultation on June 26, 2024. As with previous consultations, this written submission should be viewed as complementary and supportive of the other submissions we have contributed to, 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, Vertex has approved medicines that treat the underlying causes of cystic fibrosis, and we have a new drug submission (NDS) for the treatment of sickle cell disease and transfusion-dependent beta thalassemia that is currently undergoing Priority Review by Health Canada. 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, type 1 diabetes, APOL1-mediated kidney disease, IgA nephropathy, autosomal dominant polycystic kidney disease, myotonic dystrophy type 1 and alpha-1 antitrypsin deficiency. 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 proposed price review framework and supports the Board's objectives of developing Guidelines that refrain from price setting or control, enhance administrative efficiency and provide transparency and predictability to all relevant stakeholders including the pharmaceutical industry. However, we remain concerned that the ongoing development of the Guidelines is contributing to uncertainty for individuals seeking new medications and for rights holders. Like any business, we require a pragmatic, reliable, and transparent regulatory environment that does not create barriers to entry, incentivizes investments in research, and facilitates business planning. The proposed approach lacks clarity on price ceilings that would be considered "within guidelines", which may impact launch decisions.

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For example, complaints and currency exchange fluctuations may trigger in-depth investigations, potentially setting unpredictable lower price ceilings which cannot be planned for.

For the Board's consideration, we have outlined several recommendations regarding key aspects of the price review framework covered in the Discussion Guide:

Initial Price Review

- Vertex supports the PMPRB's proposal to conduct initial price reviews based on an International Price Comparison (IPC) within the PMPRB11 reference countries. However, we recommend adopting the Highest International Price (HIP) level for the IPC because it is the threshold most aligned with the PMPRB's mandate, which is to monitor for price excessiveness as a function of patent abuse and not to control or set prices. Using the HIP would also help reduce barriers to market entry and enhance administrative efficiency.

Post-Initial Price Review

- Instead of transitional provisions for existing medicines, Vertex recommends that medicines launched before July 2022 under the previous Guidelines not be subject to additional price tests through application of the IPC based on the PMPRB11. In most cases, these medicines already have an established Non-Excessive Average Price or NEAP and were brought to market based on the understanding of the regulatory environment at that time. If the NEAP is maintained with permitted consumer price index (CPI) adjustments, the medicine should remain compliant and not trigger additional price tests according to new Guidelines.

Special Provisions – Complaints

- To modernize and simplify the PMPRB's administrative framework, the Board should reconsider the need for a complaint process for medicines successfully negotiated and delivered through established public procurement processes. These medicines, which include innovative cell and gene therapies and blood products, are delivered in hospital settings, evaluated by health technology assessment bodies, and negotiated through pan-Canadian, provincial and hospital procurement processes or the pan-Canadian Pharmaceutical Alliance. These established evaluation and reimbursement processes already provide substantive protections against the risks of excessive pricing.
- The mechanism and the discretion in which complaint-based processes may be used undermines market stability and predictability, creating confusion and diminishing trust in the system for rights holders. Therefore, if incorporated, we suggest limiting complaints to the Federal Minister of Health or any of their Provincial or Territorial counterparts due to their responsibilities in leading health systems and their understanding of the PMPRB's role and mandate.

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- To establish a more stable and predictable pricing framework, we recommend increasing transparency during the complaint process and implementing a clearly defined appeal process. Rights holders should receive details of the complaint with the reasons underlying it and be allowed to make an appeal before an in-depth review is initiated. This would give rights holders the opportunity to clarify or resolve any concerns, potentially eliminating the need for an in-depth review and easing the administrative burden on all parties.

In-Depth Price Review

- Vertex recommends that the application of international or domestic therapeutic class comparisons during investigations be determined by a new panel of independent, arms-length clinicians with an expertise in the relevant therapeutic area and experience in providing care for patients affected by the condition. Both the Human Drug Advisory Panel (HDAP) and PMPRB staff lack the necessary expertise and practical care experience to provide recommendations across all therapeutic areas and modalities.
- As well, it is important that therapeutic class comparisons exclude medicines that are unapproved, generic or have become genericized. Including these medicines would undermine the policy intent of the *Patent Act*, which is to grant time-limited market exclusivity to incentivize the development and marketing of new innovations.
- Lastly, we encourage more meaningful dialogue between the PMPRB and the industry regarding in-depth investigations and comparisons used in these evaluations to ensure the proposed approach is effective in practice.

We thank the PMPRB for considering our feedback and look forward to participating in the next phase of consultations. Should any questions arise, we would welcome the opportunity to meet to discuss our recommendations in greater detail.

Sincerely,

Michael Siauw
General Manager
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