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December 20, 2023

Patented Medicine Prices Review Board
Box L40
Standard Life Centre
333 Laurier Avenue West, Suite 1400
Ottawa, Ontario K1P 1C1

Feedback Regarding the Scoping Paper for the Consultations on 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) scoping paper posted for consultation on November 10, 2023. This written submission should be viewed as complementary and supportive of the other submissions we have contributed to, particularly those from BIOTECanada and RAREi (the Canadian Forum for Rare Disease Innovators).

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. Our company has multiple approved medicines that treat the underlying cause of cystic fibrosis (CF). Additionally, we have a robust pipeline of investigational small molecule and cell and genetic therapies in other serious diseases where we have deep insight into causal human biology, including sickle cell disease, beta thalassemia, APOL1-mediated kidney disease, pain, type 1 diabetes, alpha-1 antitrypsin deficiency and Duchenne muscular dystrophy. We are dedicated to reinvesting most of our resources back into our research and development (R&D) engine – exemplified by our track record of allocating over 70% of our operating expenses to R&D, which is well above average in the industry.

The ongoing development of the PMPRB Guidelines has continued to create uncertainty for individuals in need of innovative medicines and for the pharmaceutical industry. There is evidence suggesting that this has negatively affected access to medicines, clinical trial launches, and R&D in Canada.[1,2] Like any business, developers of innovative treatments require a

[1] Labrie, Yanick., *Is there evidence that regulating pharmaceutical prices negatively affects R&D and access to new medicines? A systematic literature review,* (Canadian Health Policy Journal, June 2020), https://www.researchgate.net/publication/342783080_Is_there_any_evidence_that_regulating_pharmaceutical_prices_negatively_affects_RD_or_access_to_new_medicines_A_systematic_literature_review, Accessed December 10, 2023

[2] Rawson, Nigel., *Clinical Trials in Canada: Worrying Signs that PMPRB Changes will Impact Research Investment*, (Canadian Health Policy Journal, February 2021), https://www.canadianhealthpolicy.com/product/clinical-trials-in-canada-worrying-signs-that-pmprb-changes-will-impact-research-investment-2/, Accessed December 10, 2023

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consistent and reliable regulatory environment that supports business planning, commercial certainty and an opportunity to forecast revenues.

We recognize the challenge governments are facing to foster a vibrant life science and biomanufacturing environment, while providing the best standard of care for patients and balancing healthcare budgets. These policy goals can be supported by the PMPRB provided that the Board fulfills its legislative and constitutional mandate to refrain from controlling or setting prices, and instead, protect consumers from patent abuse incurred by the excessive pricing of medicines. This principle has been affirmed in several recent court decisions and is essential to the efficient and appropriate functioning of various pan-Canadian public and private organizations within the Canadian health care system that manage the evaluation, negotiation, coverage, prescribing and distribution for innovative medicines.

For the Board's consideration, we have outlined four recommendations aimed at supporting the PMPRB in developing Guidelines that align with everyone's goals and mandates – and most importantly, enable Canadians to access the treatments they need in a timely manner:

1. The Highest International Price (HIP) of the PMPRB11 comparator countries should be the primary trigger for investigation regarding a potentially excessive price. If the PMPRB adopts a therapeutic class comparison test, it should only be done with comparators that are approved and subject to market exclusivity.
    - A price that is within the range of the PMPRB11 countries should not be considered excessive as a function of patent abuse, i.e., the HIP should be the primary trigger for an excessive price investigation.
    - While Canada represents only 2% of the global pharmaceutical market, it can serve as a direct and indirect comparator for other countries employing international reference pricing. Many of these countries have larger markets, which can lead to a negative cascading effect that incentivizes companies to delay or forego launching innovative medicines in Canada. Even before the new PMPRB regulations were adopted, Canadians had far less access to a range of innovative medicines compared to the US and Europe. A notable example is the case of novel antibiotics: research from 2022 shows that between 2010 and 2019, there were 18 new antibiotics launched globally, and only two were marketed in Canada, the worst record among the developed countries reviewed.[3]

[3] Outterson, K., et al, Patient Access in 14 High-Income Countries to New Antibacterials Approved by the US Food and Drug Administration, European Medicines Agency, Japanese Pharmaceuticals and Medical Devices Agency, or Health Canada, 2010–2020, *Clinical Infectious Diseases*, Volume 74, Issue 7, 1 April 2022, Pages 1183–1190, https://doi.org/10.1093/cid/ciab612

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- If the PMPRB applies any international or domestic therapeutic class comparisons, it is essential that these reviews do not consider the prices of generic or unapproved medicines, as this 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.

2. In extraordinary circumstances, price ceiling exceptions should be made for innovative medicines addressing clear unmet need.
    - In recognition of the delay in access pricing reforms may cause to important new life-saving medicines, the Government of Canada exempted COVID-19 treatments from certain aspects of the new PMPRB pricing rules.
    - Similarly, and in extraordinary circumstances (e.g., public health crisis, pandemic), future Guidelines should incorporate special price ceiling provisions to allow for innovative treatments to enter the market at a price higher than the HIP of the PMPRB11.
    - These provisions would both help enable important life-saving medicines to enter the Canadian market quickly and address circumstances in which such treatments do not have comparable listings within the PMPRB11 basket or appropriate therapeutic class comparators.

3. Patented medicines delivered through public procurement processes should be recognized with special consideration.
    - To help modernize and simplify the PMPRB's administrative framework, the Board should consider applying a more specialized and streamlined approach towards medicines negotiated and delivered through established public procurement processes. These medicines, which include innovative cell and gene therapies and blood products, are always 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. Due to these established review and government listing processes, consumers are protected from risks of excessive pricing.

4. The inclusion of a grandfathering clause should be reinstated in the new Guidelines and the PMPRB should avoid re-benching prices after they have been reviewed by Board staff.
    - Medicines launched before July 2022 under the previous Guidelines or "existing medicines" should not be subject to additional price tests through application of the updated basket of countries. "Existing medicines" in most cases have an

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established Non-Excessive Average Price or NEAP and if they maintain that price or take increases based on consumer price index, their prices are not indications of patent abuse that would trigger the regulatory oversight power of the PMPRB. In sum, all prices of existing medicines deemed compliant before the introduction of the new *Patented Medicines Regulations* should be accepted as compliant and not subject to further review according to the new Guidelines.

- Similarly, new medicines in updated Guidelines that have been assessed as compliant under updated Guidelines should not be subject to future price revisions or "re-benching" as this would be a form of price control and therefore outside of the PMPRB's statutory and constitutional mandate. Price control is within the exclusive purview of each province, and the provinces – as well as private payers – have implemented a broad range of tools that lower the effective prices of innovative medicines.

In light of the significant unmet needs across various diseases, prioritizing timely access to transformative treatments, such as innovative precision medicines and advanced therapies, is imperative. This not only plays a crucial role in improving health outcomes and well-being for frequently underserved patients but also contributes to efficiencies in our healthcare system, societal productivity gains, economic growth, and the acceleration of future innovation in the life sciences.

We welcome the opportunity to meet with the PMPRB to discuss our recommendations and look forward to further consultations on the Guidelines in development.

Sincerely,

Michael Siauw
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