December 5, 2022

Patented Medicine Prices Review Board (PMPRB)
Box L40 – Standard Life Centre
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Ottawa, ON
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Submitted via online consultation portal: https://www.canada.ca/en/patented-medicine-prices-review/services/consultations/2022-proposed-updates-guidelines.html

RE: Consultation on 2022 Proposed Updates to the PMPRB Guidelines

On behalf of Ultragenyx Canada, I would like to thank you for the opportunity to provide written feedback on the 2022 Draft Guidelines for the Patented Medicine Prices Review Board (PMPRB).

Alignment with Industry Associations

As a member company of both BIOTECanada and RAREi (the Canadian Forum for Rare Disease Innovators), Ultragenyx fully supports and echoes the feedback provided within their respective written submissions to this consultation process. This includes:

- **Major concerns around pricing uncertainty,** including the decision to abandon the PMPRB's traditional price review process in favour of a general criteria-based investigation model that places extraordinary discretion with Board staff and leads to significant uncertainty about what a compliant price may be over time.
    - This also includes concerns around the floating median price which would cause significant uncertainty and could prevent companies from having an established acceptable price over time, which could jeopardize both launches in Canada and future commercial agreements both in Canada and around the world.

- **A lack of recognition from the PMPRB about the value of innovation,** which is not aligned with the Government of Canada's wider plan to support biopharmaceutical and life sciences sector growth. The PMPRB's approach may also undermine the federal government's upcoming Rare Disease Strategy, of which a critical element should be ensuring that Canadians with rare diseases can receive access to the treatments they need.
    - This is especially important with the emergence of gene therapies, some of which could have a transformative effect on Canadian health systems.

The Ultragenyx Perspective

We would like to take the opportunity to provide further input from Ultragenyx as a standalone organization. As a new entrant into Canada's biopharmaceutical sector specifically focused on treatments for rare and ultra-rare diseases, we believe that we can provide an important and unique perspective on the potential impact of the proposed guidelines.

The proposed guidelines will jeopardize the entry of new companies into Canada

Ultragenyx made the decision to enter the Canadian market in 2018 and have made substantial investments to build a Canadian organization to serve Canadian rare disease patients. Had the proposed guidelines been in place at the time, it would have been far more challenging to justify a Canadian affiliate as a priority among other countries. Simply put, the new pricing system and the significant uncertainty that comes with it would have caused serious pause in our decision to establish a presence in Canada.

Delaying access to treatment for Canadian patients

Since our inception, we have always been a champion of Canada, working to ensure that Canada is considered one of the first countries considered for new products. This work has allowed us to launch CRYSVITA® and DOJOLVI® in quick succession following U.S. approval. The benefit of this is clear: faster access to treatment for patients in need.

However, our status as a "first-wave" launch country is at risk with the proposed guidelines, given the uncertainties associated with them and the risk they pose to global pricing. Should the current proposed guidelines be put into place, it will be more difficult to justify Canada as an early launch country, delaying access for patients in need of treatments.

The proposed guidelines threaten access to future innovative products

These proposed guidelines are also weighing heavily on our future plans for Canada. We believe that the future of medicines – especially in the area of rare diseases – lies in next-generation precision medicines, most notably gene therapies. These therapies will undoubtedly require complex reimbursement discussions and innovative commercial agreements, including value-based contracting. While HTA bodies and payers are working towards the goal of finding appropriate vehicles to assess and recognize value for these new innovations, the proposed guidelines are moving away from this objective. They appear to offer no vehicle to recognize the level of innovation these products are likely to deliver, potentially using dated generic molecules and/or OTC medications as pricing benchmarks for new and life-saving technologies. In short, if these guidelines are accepted as proposed, it may be difficult to bring these medicines to the Canadian market in a timely manner – if at all.

As an organization, we sincerely urge the PMPRB to reconsider its proposed draft guidelines and approach, and instead work collaboratively with stakeholders across all areas to build a new price monitoring system from the ground up. This would allow for the creation of guidelines that can provide a level of predictability to industry while ensuring that the PMPRB can continue to serve Canadians and ensure that our health system remains sustainable for decades to come.

On behalf of Ultragenyx, thank you again for the opportunity to provide feedback. Our team remains available at your convenience should you have any questions or concerns about our feedback.

Regards,

Monty Keast
Vice-President and General Manager
Ultragenyx Canada Inc.