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February 13th, 2020

Douglas Clark
Executive Director
Patented Medicine Prices Review Board
Suite 1400 – 333 Laurier Avenue West
Ottawa, Ontario K1P 1C1

Submitted electronically: PMPRB.Consultations.CEPMB@pmprb-cepmb.gc.ca
RE: Horizon Submission – PMPRB Draft Guidelines Consultation

Dear Mr. Clark:

Horizon Therapeutics Canada ("Horizon") would like to provide a number of comments with respect to the PMPRB's current Draft Guidelines consultation.

Horizon is an innovative biopharmaceutical company in Canada focused on developing and commercializing medicines for rare diseases. Horizon aims to improve the lives of Canadians living with rare diseases by bringing innovative therapies to Canada in a safe, timely and sustainable way. Our commitment to our patients – especially in the rare disease space – is grounded in our personal investment in the lives of the people our medicines help, from diagnosis through ongoing care.

Horizon is a member of Innovative Medicines Canada, BIOTECanada and an industry forum for rare disease innovators (RAREi). Horizon fully supports the submissions put forward by these organizations. By adding to their submissions, Horizon is eager to provide our further input on this important consultation in consideration of the implications specifically for rare disease medicines under the proposed guidelines.

Rare diseases are typically severe conditions affecting a very small proportion of the population. Approximately 1 million Canadians are affected by rare diseases. For 94% of these debilitating and chronic conditions, there is no treatment available.[1] These diseases sometimes appear at birth or develop in early childhood. One-third of children with rare diseases die before their fifth birthday.[2]

Canadian rare disease patients face numerous challenges in managing their health. The path to reach diagnosis is often long and complicated due to lack of disease awareness or access to the correct clinical expertise. For those patients who are fortunate enough to

[1] Report of the Standing Committee on Health (Feb. 2019)
[2] Ibid.

have a diagnosed and treatable rare disease, other major challenges persist, particularly with respect to the predictability and timeliness of access to new treatments. Of the approximately 7,000 identified rare diseases, only 5% of these conditions have effective treatments.[3] In the United States there are fewer than 15 new treatments for rare diseases approved per year.[4] Even fewer are approved in Canada.

Due to the unique characteristics of rare diseases and the already uncertain path rare disease patients face in their treatment, Horizon is seriously concerned that, absent major adjustments, the Draft Guidelines would serve to increase the barriers to treatment for Canadian patients. They do so by disincentivizing orphan drug manufacturers, like Horizon, from being able to plan with certainty, for the commercialization of these rare medicines. Canada unlike the United States, Europe and many other countries still does not have any formal legislation or regulations in support of patients with rare diseases.

Rare Disease Drugs in Context

The current discussion related to the cost of rare disease medications fails to consider the reality and context of spending in our broader healthcare system. According to the PMPRB's own figures, the overall cost of non-cancer, patented rare disease medicines represented just 1.8% of total public and private drug spending or less than $450 million in 2017.[5]

The Draft Guidelines fail to recognize or account for the established reality that the majority of those medicines, considered to be "expensive drugs" by the PMPRB, are already subject to multiple price reduction mechanisms at the national and provincial level, including pan-Canadian and other payer negotiations, hospital and national tenders, and other affordability and value-enhancing policies.

Health Technology Assessment (HTA): Inappropriate For Rare Disease

Horizon has serious concerns regarding the application of HTA tools to the price regulation of rare disease drugs. It is widely acknowledged that conventional HTA standards and assumptions are not well-suited for assessing rare disease medicines. With the reality of smaller patient populations and the concomitant paucity of clinical data available, rare disease medicines present methodological challenges for assessments compared to other therapies for larger patient populations where extensive clinical data may exist. Smaller and shorter studies designed to expedite access to treatments in the face of very rare, life-

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[3] The Canadian Forum for Rare Disease Innovators (RAREi) – Submission to the House of Commons Standing Committee on Health (2019)

[4] Ibid.

[5] What is the "Expense" for Expensive Drugs for Rare Diseases? PMPRB Presentation (April 2019)

threatening diseases may result in less clinical trial data for analysis.
Regulators and evaluators need to understand the unique challenges rare drug innovators face when developing new therapies. Sometimes only unpublished proprietary data and real-world experiential data from a limited number of specialists are available at the time of submission. HTA evaluation for rare disease drugs needs to consider the alternative clinical trial design methodologies that enable the study of rare disease drugs and realize the inappropriateness of cost-effectiveness thresholds designed for larger population medicines.

Due to the unique challenges of rare disease drug development, it is our recommendation that the Draft Guidelines not apply to rare drug innovators. It is recommended that a separate system be established that recognizes the need for greater flexibility when assessing rare disease medicines. We would welcome the opportunity to provide greater input on alternative approaches.

Draft Guidelines Represent A Substantially Higher Regulatory Burden

Within the current policy and regulatory context, there are already existing challenges to commercializing new rare disease treatments in Canada. The Draft Guidelines will pose a major new barrier to the availability of new medicines for rare diseases in Canada. Beyond the attempt to apply HTA information, the proposed guidelines include other subjective or impractical elements inconsistent with a modern, agile and predictable regulatory framework.

For example, the proposed filing requirements will place an undue regulatory burden on many innovative companies. Details are critical, for example, related to the timing and ability to provide accurate amounts of third-party rebates. As currently structured, patentees may not know the rebate amounts until the reimbursement agencies submit invoices. This process can often take many months to complete, making it next to impossible to provide the required information at the time that it is required by the PMPRB. This lack of clarity over the reporting of rebate data is compounded by the lack of operational templates for providing required new information.

The cost involved with the additional regulatory burden is not something smaller companies can readily absorb. Combined with the pricing impact the new regulations will impose upon the economic viability of smaller innovative drug companies, like orphan drug manufacturers, this added burden could easily lead to new medicines not being made available in Canada.

The PMPRB's own case studies show the amended regulations will reduce drug ceiling

prices by up to 70 per cent[6]. This will affect Canada's international competitiveness as a country in which to attract world class clinical trials. It will also impact the sustainability for innovative pharmaceutical companies in Canada. Canada will no longer be among the first countries in which to launch new drugs. Simply put, Canada's ability to lead in innovation in the pharmaceutical space will be displaced, relegating it to a position of follower, or worse.

While the regulations have removed the United States from the basket of comparator countries, it has further put Canada at a disadvantage. With the removal of the U.S., it excludes a reference country, with the closest societal ideals to Canada and where the large majority of the world's pharmaceutical innovation comes from. As the primary innovator of many drugs, the U.S. will look at markets that have systems in place that encourage and attract innovation in which to launch new therapies.

Lack of Alignment with Other Government Policies

The proposed guidelines will add new pricing obstacles to the already significant reimbursement barriers for rare disease medications. There is real concern from patentees and stakeholders that Canada will lose its competitiveness as a preferred jurisdiction in which to launch new products. This would be a highly counterproductive policy outcome, as it would be Canadian patients most impacted if product launches are delayed or avoided.

The overall policy direction reflected by the Guidelines is at odds with the Government's expressed interest in economic growth and fostering innovation. For example, it is challenging to reconcile the Guidelines with the specific and ambitious goals to advance the Canadian life sciences sector identified by the Government's own Health and Biosciences Economic Strategy Table (HBEST).

To address these challenges, Horizon would recommend pursuing frameworks and tools to manage the legitimate sources of uncertainty inherent in evaluating and reimbursing rare disease medicines. This would promote timely and sustainable patient access to medically necessary drugs for rare diseases without compromising Canada's ability to attract new launches.

Horizon welcomed the federal government's commitment in the 2019 Budget to develop and fund a national strategy for drugs for rare diseases. In addition to an investment of $1 billion, there is now an opportunity to develop a more balanced and sustainable approach to managing these medicines. A future framework will need to incorporate critical elements including how to gather and evaluate evidence for drugs for rare diseases, how to improve

[6] Innovative Medicines Press Release (Aug. 19)

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the consistency of decision-making and patient access across Canada, and how to arrange for the meaningful engagement of manufacturers, clinical experts and patients. Ultimately, our shared objective should be ensuring that Canadian patients with rare diseases have access to effective, medically necessary therapies at prices that are fair to both the public and those investing in developing therapies for these rare diseases.

Horizon also notes the lack of alignment these Guidelines have with provincial and territorial governments. Work is ongoing to improve publicly funded access to rare diseases through the Provincial Drugs for Rare Diseases Working Group. Quebec is also making rare disease drug access a priority and Ontario has said it prefers to see a potential national pharmacare plan that focuses on rare disease treatments.[7] These jurisdictions understand the uniqueness of rare diseases and why they deserve separate consideration.

Moving forward in this area will depend on taking a revised approach to the PMPRB Guidelines. In light of this opportunity, as well as the existing agencies and tools in place, Horizon recommends that much greater scrutiny be exercised in updating the Guidelines to ensure that rare disease medicines remain accessible for Canadian patients. If the PMPRB is unable to account for the unique aspects of rare disease in policy, we strongly recommend that major elements of the proposed Guidelines be set aside until further notice, in particular the proposed use of HTA factors.

Thank you for the opportunity to provide our unique perspective. Many stakeholders feel that the input from industry has not been reflected in this process. We urge fair consideration of our comments. Should you have any questions, please do not hesitate to contact me.

Sincerely,

John Haslam
General Manager
Horizon Therapeutics Canada

[7] iPolitics – Elliott says Ontario doesn't want full pharmacare overhaul, urges focus on drugs for rare diseases (Nov 2019)