VIA EMAIL: pmprb.consultations.cepmb@pmprb-cepmb.gc.ca

August 31, 2021

Dr. Mitchell Levine
Chairperson, Patented Medicine Prices Review Board (PMPRB)
333 Laurier Avenue West, Suite 1400
Ottawa, Ontario K1P 1C1

Subject: Proposed Guidelines changes to the definition of Gap medicines, the references to the comparator countries and the international price tests for Grandfathered medicines and their line extensions

Dear Dr. Levine,

We appreciated the opportunity to participate in this consultation.

Ultragenyx is a biopharmaceutical company focused on developing new treatments for rare and ultrarare genetic diseases for which there are typically no approved therapies.

As a member of the Canadian Forum for Rare Disease Innovators (RAREi), Ultragenyx has contributed to and endorsed RAREi's submission, which is attached to this letter. In this cover note, we wanted to emphasize some of our most important concerns with the proposed changes to the price test and the compliance timeline for Grandfathered medicines.

First, we want to start by stating how pleased we were with the federal government's June decision to delay the implementation of the new pricing regulations. We were hopeful that this additional time would allow the government to work with the regulated sector and stakeholders to come up with a better approach, especially given the significant shift in context that has occurred since the regulatory amendments were adopted in 2019. Specifically, the pandemic has shown us the importance of having a timely system for accessing medicines and vaccines as well as a strong life sciences sector. The federal government clearly signalled a shift in policy toward building a competitive life sciences industry though the Biomanufacturing and Life Sciences Strategy launched in August.

Given this context, we were extremely surprised and disappointed to see the PMPRB propose unexpected and substantive changes to its Guidelines. This proposal runs directly counter to the government's decision to delay the regulations to January 1, 2022, which was to allow the life sciences sector time to focus on responding to the COVID-19 pandemic. The changes also undermine federal pandemic preparedness and life sciences strategy goals. Hastily pushing through major Guidelines changes that are at odds with national priorities, as we head into

the fourth COVID-19 wave and on the eve of a federal election seems misaligned from the broader pandemic, political and policy environment.

As well, we want to highlight that since opening our Canadian office in 2018, our goal has been to help grow the Canadian market for research and development of rare disease treatments and reach the largest number of Canadians who could benefit from our therapies. Ultragenyx is also committed to fair and reasonable pricing that recognizes the need of payers, patients and our health system. However, continued and unexpected changes to the regulatory pricing environment, such as those proposed in the latest proposed Guidelines changes, will make it increasingly difficult for companies like ours to commercialize our rare disease treatments and make further investments in Canada.

Over the past two years, our company has been operating with one set of assumptions regarding the pricing regime. We are now faced with an entirely new proposal that is unprecedented, with seemingly limited rationale. In particular, the PMPRB is proposing to change the key criterion for the maximum list price (MLP) for grandfathered and line extension medicines from the *highest international price* (HIP) among the PMPRB11 to the *median international price* (MIP) among the PMPRB7, as of the June 31, 2021 reporting period. This represents a significant departure from any of the previous proposals put forward by the PMPRB and undermines the extensive analyses and planning that patentees have undertaken to prepare for the impact of the impending changes on their currently marketed products. The impact of this change to patentees should not be underestimated and represents yet another obstacle interfering with our fundamental objective – making our therapies available to the Canadian patients who need them.

When companies make business decisions, they rely on clear, transparent, and predictable rules to guide their planning. However, if the rules can be changed unilaterally at any time and with no reasonable rationale, it destabilizes the entire market for pharmaceuticals and disincentivizes further investments in research, development, and deployment of new medicines in Canada.

We therefore encourage the PMPRB to discard the proposed changes to Grandfathered medicines to avoid tangible and negative impact on health systems, the economic recovery post-pandemic, and broader federal and provincial life sciences sector goals.

We thank you for considering our input, and please do not hesitate to contact me if you have any questions regarding our perspectives on the proposed changes.

Sincerely,

Monty Keast

Monty Keast, VP and General Manager
Ultragenyx Canada Inc.

Encl.: RAREi's submission to the PMPRB

RAREi
THE CANADIAN FORUM FOR
RARE DISEASE INNOVATORS

RAREi's submission on the proposed changes to the PMPRB Guidelines

August 27, 2021

This submission relates to important proposed changes to the Patented Medicine Prices Review Board (PMPRB) guidelines published on July 15, 2021.

The Canadian Forum for Rare Disease Innovators (RAREi) input begins with a reminder about the stated rationale behind the federal government's recent decision to delay the implementation of the *Patented Medicines Regulations* and its priorities in doing so, including the COVID-19 pandemic response, its new Biomanufacturing and Life Sciences Strategy and the emerging national rare disease treatment strategy. In that context, RAREi takes issues with the PMPRB's general approach in introducing new proposed guidelines changes and specifically is concerned with the broad and significant effects of the resulting lower list prices for currently-marketed medicines and earlier compliance timelines.

RAREi wishes to stress that these proposed changes, which were unexpected and not the subject of substantive consultation beforehand, add significant uncertainty for the Canadian pharmaceutical market and make it even harder than before to attract research and medicine launches that would benefit Canadian patients, clinicians, researchers, health system and the economy.

Regulations delay as part of the COVID effort and other key federal priorities

RAREi was pleased to see the federal government recognize the need to delay its new pharmaceutical pricing regulations. As RAREi outlined in its communications and submissions related to this issue to date, if implemented, the new pricing regime will have long-standing negative effects on patients, especially those suffering from rare disorders, and put Canada at a competitive disadvantage in terms of medication launches and clinical research.

The changes to the pricing regime also need to be further considered to ensure they do not undermine the federal government's and our collective efforts to develop a national rare disease strategy and to implement the recently announced Biomanufacturing and Life Sciences Strategy. Ultimately, federal pharmaceutical policies should improve and not curtail access to rare disease therapies and should help grow the life sciences industry to protect Canadians against pandemics and other important health challenges.

During the first year of the COVID-19 pandemic, all Canadians learned what many Canadians with rare diseases, unfortunately, have known all too well for many years – the agony of not having an effective prevention or treatment available for a potentially life-threatening disease. New therapeutics developed by RAREi members and others are already saving and improving Canadian lives, and their research programs provide early access to clinicians and patients with rare diseases. Several RAREi companies are also developing COVID therapeutics.

However, as explained further in this submission, with the new proposed changes to the pricing of grandfathered medicines and their line extensions, the PMPRB will create a new obstacle for the Canadian life sciences sector and the rare disease community.

Overall approach of PMPRB to the proposed changes

RAREi is extremely concerned with the PMPRB's approach in proposing substantive changes to the guidelines. RAREi members believe it is highly inappropriate for an independent quasi-judicial government agency to develop policies contrary to government's direction and which demonstrate a strong bias against those it is mandated to regulate fairly. The PMPRB should be focused instead on impartially executing government policy in a fair and neutral manner.

These latest changes have been sprung on all stakeholders using a false pretense that they are in response to the government's latest delay in implementing the new *Patented Medicines Regulations*. However, there is no justifiable reason why any such changes are needed at this point. They appear to be a means for the PMPRB to create new medication pricing policies with no regard to stakeholder input at the same time as the federal government has deemed it prudent to allow the industry to focus on addressing the COVID-19 pandemic.

Further, these changes go beyond the PMPRB's mandate under the *Patent Act*. Based on the Federal Court of Appeal's decision in the Alexion case,[1] the PMPRB's regulatory mandate is to ensure that prices are not excessive as a function of abuse of patent monopoly. The PMPRB offers no rationale regarding how the proposed guidelines changes will achieve this mandate or, more fundamentally, how on-market public (list) prices currently above the median of the comparator countries are excessively priced as a function of patent abuse. The fact that above-median list price medicines are marketed in Canada, and in almost every case subject to value-enhancing product listing agreements and patient support programs, demonstrates that these prices are not excessive. Affordability-enhancing mechanisms also include, among others, private sector risk pooling initiatives and the pan-Canadian Pharmaceutical Alliance's negotiation process.

Regarding the timing, the proposed changes have been presented without warning in a mid-summer consultation period that overlaps with the federal election campaign, during which government agencies should refrain from engaging on policy changes.

Finally, section 96(5) of the *Patent Act* requires the PMPRB to consult with stakeholders on guidelines changes, including the pharmaceutical industry. Presenting new substantive changes with virtually no explanation, justification or impact assessment does not amount to appropriate consultation. No information session or webinars were held by the PMPRB to address the proposed changes. Stakeholder engagement in the limited time before the federal election was triggered was limited to a inadequate consultation document mainly comprised of the changes to the text in the guidelines along with a short "Frequently Asked Questions" document. Stakeholders are left to decipher the meaning of the changes and their implications.

[1] Alexion Pharmaceuticals v. Canada (Attorney General), 2021 FCA 157: https://decisions.fca-caf.gc.ca/fca-caf/decisions/en/item/500849/index.do

It is particularly troubling that this approach was taken so soon following the publication of the PMPRB's February 2021 communications plan,[2] revealing the agency's strong negative biases against the regulated sector and other stakeholders.

Based on the above, there are very strong grounds for the government to initiate a comprehensive review of the operations of the PMPRB – including its leadership, governance, engagement approach and decision-making processes – as it is not acting in accordance with its mandate and the standards required of government agencies, the stated policy directions of the government, or in the best interests of all its stakeholders.

Impact of lowering maximum list price for grandfathered and line extension medicines

The PMPRB is proposing to change the key criterion for the maximum list price (MLP) for grandfathered and line extension medicines from the *highest international price* (HIP) among the PMPRB11 to the *median international price* (MIP) among the PMPRB7, as of the June 30, 2021 reporting period. This represents a significant departure from any of the previous proposals put forward by the PMPRB and undermines the extensive analyses and planning that patentees have undertaken to prepare for the impact of the impending changes on their currently marketed products.

The PMPRB's "Frequently Asked Question" document forecasts that this change will reduce the list prices of grandfathered medicines *on average* by 10% and those for rare diseases more specifically by 3.5%, but the basis for these estimates is completely unsubstantiated. More importantly, the impact will be more drastic than this for many rare disease medicines, depending on the comparator countries used to set the maximum prices and where they are in their lifecycle. While the MIP test may have a lesser impact at the time of introduction, the further along a product is in its lifecycle the greater the potential for disparity among International prices. For this reason, the current PMPRB guidelines rely on the HIP to confirm ongoing compliance and non-excessive pricing for "existing patented drugs", not the MIP. An analysis that considers specific classes and medicines would have been appropriate for the PMPRB to share with stakeholders.

In certain cases, reducing the maximum permitted list price could result in companies having to withdraw medicines from the Canadian market if the price is no longer viable and/or affect pricing in other markets. This would be to the great detriment of Canadian patients, particularly those with rare diseases for whom treatment options are often limited to one potential medication.

Given the above, the proposed PMPRB changes run counter to the government's stated policy of – and strong financial commitment towards – developing a national rare disease treatment strategy for Canada. The goal of that plan is to ensure greater, more timely and more equitable access to rare disease treatments by Canadians.[3]

[2] https://www.dropbox.com/s/eusxuabcq26uqt9/PMPRB%20ATIP%20Disclosure.pdf?dl=0

[3] See Health Canada's July 26, 2021, *Building a National Strategy for Drugs for Rare Diseases in Canada, What We Heard from Canadians*: https://www.canada.ca/en/health-canada/programs/consultation-national-strategy-high-cost-drugs-rare-diseases-online-engagement/what-we-heard.html

Earlier compliance date

When the proposed changes to the *Patented Medicines Regulations* were adopted in 2019 and new guidelines subsequently issued, patentees were given one year to comply with the changes to the pricing of grandfathered medicines. When the regulations were previously delayed, the PMPRB proposed cutting the compliance period to six months. In the face of significant pushback given the COVID pandemic context, this was subsequently changed back to one year.

With the new guidelines changes, the PMPRB is once again proposing to reduce the compliance period from one year to six months following the coming into force of the new regulations on January 1, 2022. This means that companies will have to comply with the changes by July 1, 2022.

Given the additional proposed changes to the guidelines and the ongoing pandemic, which was the primary reason for the regulations delay, it would be more appropriate to give companies more rather than less time to be compliant.

Increased uncertainty and destabilization of the Canadian market for rare disease developers

Overall, the proposed guidelines changes add to the climate of uncertainty and instability that has affected the whole life sciences sector – and the rare diseases medicines community as a whole – since pricing reforms were first proposed in 2016.

These changes and the unwillingness of the PMPRB to consult constructively or respond to the valid concerns of stakeholders have led to a serious destabilization of the Canadian market for pharmaceuticals, which are a crucial part of the Canadian health care system.

Concluding remarks

The PMPRB risks disrupting the provision of much-needed treatments for Canadians by bringing forward unpredictable, uncertain and subjective new rules and conducting insufficient consultations. The proposal also directly contradicts the federal government's goal of ensuring the life sciences ecosystem focuses on managing the greatest health crisis to face Canada in a century, the COVID-19 pandemic, for which the key solution has been the rapid development and provision of vaccines and therapeutics developed by the global life sciences industry, including RAREi members.

In this context, RAREi recommends that the changes to the *Patented Medicines Regulations* and the guidelines be paused permanently to enable the federal government, the life sciences industry and other stakeholders to work constructively to develop better policy approaches that enhance the research and access environment for rare disease medicines.

Ultimately, RAREi believes that issues of accessibility and affordability can be resolved through innovative negotiated solutions and as part of the federal government's national rare disease treatment strategy and in collaboration with the provinces.

RAREi
THE CANADIAN FORUM FOR
RARE DISEASE INNOVATORS

About RAREi

The Canadian Forum for Rare Disease Innovators (RAREi) is a group of the Canadian operations of global biopharmaceutical companies with a specific focus on researching, developing and commercializing treatments for rare diseases.

Its members are Alexion Pharma Canada Corp., Amicus Therapeutics, Inc., Biogen Canada Inc., Biomarin Pharmaceutical Inc., Horizon Therapeutics Canada, Ipsen Biopharmaceuticals Canada Inc., Mitsubishi Tanabe Pharma Canada Inc., Recordati Rare Diseases Canada Inc., Sanofi Genzyme, Sobi Canada Inc., Ultragenyx Pharmaceutical and Vertex Pharmaceuticals (Canada) Inc.

RAREi's vision is to shape the Canadian health system and policy environment to facilitate patient access to diagnostics and medicines that improve the lives of Canadians with rare disorders. Its objective is to affect positive policy change in the rare disease environment within the Canadian health care system to improve the health of Canadians with rare disorders and improve patient access to orphan medicines.