RAREi
THE CANADIAN FORUM FOR
RARE DISEASE INNOVATORS

VIA SUBMISSION PORTAL

February 15, 2021

Patented Medicine Prices Review Board
333 Laurier Avenue West Suite 1400
Ottawa, Ontario K1P 1C1

Subject: **January 2021 Guidelines Amendments Consultation: gap medicines and compliance timelines**

Members of the Patented Medicine Prices Review Board,

Thank you for the opportunity to provide feedback on the above-noted draft amendments to the Patented Medicine Prices Review Board's (PMPRB) guidelines.

The Canadian Forum for Rare Disease Innovators (RAREi) has provided feedback in response to each consultation opportunity related to the current reforms, and it intends to engage consistently at every future opportunity in order to help improve Canada's regulatory and reimbursement systems for Canadians with rare disorders.

In terms of the proposed amendments, there are three areas of input that the PMPRB should consider: a) the definition of gap medicines; b) the reduced timelines for compliance for on-market medicines; and c) the urgent need for more fundamental changes to the guidelines that should have been considered in the context of this consultation.

(a) Definition of gap medicines

The expansion of scope for the definition of gap medicines effectively implements the *Patented Medicines Regulations* in the context of the updated coming-into-force date. This provides some additional certainty for on-market rare disease medicines that are launched in Canada with sales before that date. Our understanding is that gap medicines will not, as a regular course, face the additional, uncertain and punitive price controls imposed by the economic factors.

(b) Reduced timelines for compliance for on-market medicines

Regarding the other proposed change – the reduction in transition to compliance from twelve to six months – RAREi believes this undermines the entire purpose of delaying the regulations as a whole, which was to provide more time for patentees to avoid having to adapt their reporting requirements in the context of the COVID-19 pandemic pressures. The reversion to a shorter compliance timeframe will create challenges for all patentees, but particularly for smaller businesses that are focused on developing orphan medicines. Manufacturers are still faced with many unanswered questions in terms of how to assess compliance and the impact of the reforms on the supply of medicines and business operations. The guidelines are extremely complex, and

detailed clarifications are still outstanding. For example, clarity is required regarding appropriate price sources for the new basket of reference countries and further details are necessary to enable patentees to assess updated Non Excessive Average Prices (NEAPs) for on-market medicines. With less than a year from the PMPRB's proposed implementation date, the proposed shorter timeframe is unfeasible for patentees and for the PMPRB alike.

(c) Need for more fundamental changes to the guidelines

Beyond the specific changes under review, the re-opening of the guidelines for amendments is a missed opportunity to address the implications of relevant court decisions as well as the extensive stakeholder input addressing other aspects of the regime.

For example, the amendments make no mention of changes to patentees' reporting requirements regarding third-party rebates or the new price tests that determine the maximum rebated price (MRP) despite the fact that related reporting obligations were deemed invalid by the Federal Court of Canada and the Quebec Superior Court in two separate court actions. Since the enforcement of the PMPRB's proposed MRP framework is dependent on the board's ability to collect and assess such information from patentees, it stands to reason that the regulations and guidelines must be modified substantially.

RAREi recommends that the guidelines amendments be expanded to remove any reference to the economic factors and MRP, which would go some way to providing certainty related to the forecasting associated with future rare disease medicines, at least in the short term. It would also be more aligned with the PMPRB's price review mandate which is limited to examining the ex-factory prices of patented medicines.

In addition, RAREi believes that the guidelines should be amended to reflect the government's intentions in the context of its current consultations regarding the development of a national rare disease strategy and its Budget 2019 promise to invest up to $1 billion for two years starting in 2022-23 and up to $500 million per year thereafter to support rare disease treatment access in Canada. If the current guidelines are not altered in a manner that better encourages the introduction of new rare disease innovations, those government pledges will be thwarted.

As detailed in previous submissions, the regulations significantly exacerbate the existing challenges facing rare disease patients, innovators and health systems. Innovators contemplating bringing new medicines to Canada are faced with inappropriate use of economic factors to set market prices, substantial price uncertainty, and little to no rewards to encourage innovation. These are significant hurdles that reduce Canada's attractiveness as a market in which to launch new innovative treatments. For rare disease patients, this can mean lives lost, or significant reduction in quality and length of life.

In sum, RAREi has two specific recommendations:

1. **The PMPRB should retain or expand the 12-month transition period for patentee compliance from the date that the regulations come into force.**

2. **Further amendments need to be made to the guidelines as well as other regulatory instruments to address the uncertainties and key issues mentioned above. Otherwise, the PMPRB will continue to impede access for Canadians who need innovative rare disease treatments.**

Thank you for the opportunity to provide input on this consultation, and we would be pleased to meet with you in the coming weeks if you would like to discuss our submission further.

Yours sincerely,

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About RAREi

RAREi is an informal network of research-based bio pharmaceutical innovators committed to monitoring, responding and shaping policy issues in the Canadian rare disease environment. The members of RAREi are Akcea Therapeutics Canada, 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, a division of sanofi-aventis Canada inc., Sobi Canada Inc., Ultragenyx Pharmaceutical and Vertex Pharmaceuticals (Canada) Inc. For more information, see www.rarei.ca.