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THE CANADIAN FORUM FOR
RARE DISEASE INNOVATORS

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House of Commons Standing Committee on Finance

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RAREi Recommendations

1 – That the federal government halt pharmaceutical price reforms and commission an external review of the Patented Medicine Prices Review Board to prevent important negative effects on access to new medicines and vaccines, health research and the biopharmaceutical ecosystem.

2 – That the federal government implement a national rare disease strategy and the related funding commitment in a way that improves and accelerates access to rare disease treatments.

About RAREi

RAREi is a network of 12 Canadian biopharmaceutical companies committed to improving the lives of rare disease patients by researching, developing and commercializing rare disease treatments.

Recommendations in Context

Recommendation 1 – That the federal government halt pharmaceutical price reforms and commission an external review of the Patented Medicine Prices Review Board to prevent important negative effects on access to new medicines and vaccines, health research and the biopharmaceutical ecosystem.

In August 2019, the federal government adopted regulations to change how the Patented Medicine Prices Review Board (PMPRB) regulates patented medicine prices in Canada. Given the ongoing COVID-19 pandemic, the implementation of these changes was postponed to January 1, 2022.

While these amendments are not yet in effect, they have generated substantial market uncertainty, which has already delayed patient access to new treatments and decreased investments in advanced clinical trials.[1] The main challenge is that the changes offer very little predictability in terms of price compliance for developers of new treatments, particularly those operating in the rare diseases space. The range of mandatory regulatory price decreases, compared to current levels, is very concerning. It makes it almost impossible, in many cases, to develop a business case globally for the deployment of new treatments and research investments in Canada within timelines that are comparable with those in countries with similar health care systems (i.e., the EU and US). This results in fewer medications being made available to Canadian patients and significant delays in access to critical medicines when they are made available.

In addition to the problematic regulation changes, the PMPRB has acted in a manner unbecoming a regulator during the dialogue related to these reforms. As a result, the PMPRB has lost the trust of stakeholders, citizens and the regulated sector. Specific inappropriate actions undertaken by the PMPRB include:

- Lack of meaningful consultations on sweeping changes, including short timelines for input, no forum for real discussion or exchange of perspectives and information and disregard for the legitimate concerns raised by a vast majority of stakeholders
- Use of selective data to support its policy positions
- Refusal to undertake studies to properly consider the projected impact of the proposed changes on access to medicines and investments in research
- Unethical advocacy campaigns against stakeholders who disagree with its approach and positions as demonstrated by the PMPRB's February 9, 2021 communications plan[2]
- Use of biased language by board members and staff when referring to the pharmaceutical industry in various communications, reflecting lack of neutrality of the PMPRB

[1] Life Sciences Ontario, IQVIA, New Medicine Launches: Canada in a Global Context, June 2020: https://lifesciencesontario.ca/wp-content/uploads/2020/06/EN_LSO_Global-Launch-Benchmarking_Webinar-June22-20_Final.pdf and Rawson, Nigel, Canadian Health Policy, Clinical Trials in Canada: Worrying Signs that Uncertainty Regarding PMPRB Changes will Impact Research Investment, February 2021: https://www.canadianhealthpolicy.com/products/clinical-trials-in-canada--worrying-signs-that-pmprb-changes-will-impact-research-investment.html?buy_type=

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

In fact, Donald Savoie, a Canadian public administration and governance scholar, reviewed the PMPRB's February 9, 2021 communications plan in the context of the duty of neutrality. As an independent quasi-judicial body with a regulatory mandate, Professor Savoie stressed that the PMPRB is "expected to go about its work in a detached manner, relying on empirical evidence and not arriving at the table with a bias or predetermined position."[3]

Dr. Savoie is not the only external observer who has concerns about the PMPRB's action. In a July 2021 Federal Court of Appeal decision, the court admonished the PMPRB for exceeding its legislated mandate and acting in an unaccountable fashion. The decision, which was related to a previous board order for a patentee to reduce the price of its medicine, noted that the board should not be trying to regulate prices and does not have a broad consumer protection role. The court's position calls into question the current proposed changes, which if implemented, would effectively be a form of price regulation.[4]

Even so, the PMPRB recently moved ahead with a major new proposal to change its guidelines that runs counter to the intent of the federal government's regulations delay. In late June 2021, the federal government delayed the implementation of the new pricing regulations to January 1, 2022, in order to provide more time to pharmaceutical companies to focus resources and efforts on responding to the ongoing COVID-19 pandemic. Instead of tooling down and respecting the government's policy direction, the PMPRB launched an unexpected consultation in the middle of this summer to propose a new price test that would unexpectedly and significantly reduce the prices of currently-marketed medicines and impose a shorter timeline for patentee compliance with the new rules. The PMPRB provided no rationale or impact assessment to help explain the new test and timeline to the industry and stakeholders.

The PMPRB's new proposal is additive to the other clouds of uncertainty that the regulatory changes entail. Regulating down prices for on-market medicines will destabilize the entire pharmaceutical supply chain, including innovators, generics suppliers, pharmacies, distributors and wholesalers, at a critical time given the ongoing global pandemic.

The changes to the pricing regulations and guidelines are also completely at odds with the goals of the recently launched federal Strategy on Biomanufacturing and Life Sciences Strategy,[5] which aims to grow a strong and competitive sector in Canada to protect Canadians against current and future pandemics as well as other health challenges. In particular, the PMPRB reforms need to be re-assessed in the context of the strategy's 5th pillar which is to "enabl[e] innovation by ensuring world class regulation". The government cannot achieve this pillar while at the same time moving forward on the PMPRB reforms that discourage commercialization of innovation and reduce research investments in Canada.

Based on the above, RAREi requests a comprehensive external review of the PMPRB to evaluate its conduct and policies that appear to run counter to the Government of Canada's commitment to good governance, stakeholder engagement, innovation, industrial, economic development and health care policy and pandemic preparedness.

While this review is ongoing, RAREi also asks that all proposed pricing reforms be put on hold and revisited to ensure they "*do not create barriers for new medicines for Canadians"* as recommended by this committee in

[3] Memo prepared for the Canadian Organization for Rare Disorders by Canadian by Université de Moncton Professor Donald Savoie, June 7, 2021: http://www.raredisorders.ca/content/uploads/Savoie-DJ-re-PMPRB-and-Duty-of-Neutrality-June-7-2021.pdf
[4] https://decisions.fca-caf.gc.ca/fca-caf/decisions/en/item/500849/index.do
[5] https://www.ic.gc.ca/eic/site/151.nsf/eng/00019.htm

Recommendation 12 of its 2021 budget report[6] and that they do not undermine the goal of building a resilient domestic life sciences sector as envisaged by the Strategy on Biopharmaceutical and Life Sciences.

Recommendation 2 – That the federal government implement a national rare disease strategy and the related funding commitment in a way that improves and accelerates access to rare disease treatments.

The 2019 federal budget included a commitment to invest in a national strategy for rare disease treatments. It pledged up to $1 billion for two years starting in 2022-23, with up to $500 million per year afterward. This commitment was reaffirmed in the 2020 Speech from the Throne and the 2021 budget. In January 2021, Health Canada launched a consultation seeking input into the development of that strategy.

RAREi is encouraged by these commitments and measures, as Canada remains one of the only developed nations in the world that has not yet implemented a rare disease strategy. As a result, Canadians with rare disorders face immense challenges in accessing the treatments they need to improve their health and quality of life and survive. More specifically, treatments for rare diseases are usually launched in Canada at a much later time than in the United States and/or Europe and, in some instances, they do not even come to Canada at all.[7]

To address these important gaps, RAREi recommends that the federal government swiftly adopt a national rare disease strategy and implement the related funding commitment. It encourages the government to do so in a way that improves and accelerates access to rare disease treatments rather than adopting additional cost-containment processes and measures. The federal government has too often focused on the prices for some of these therapies without considering the value and health benefits they bring to patients and health systems. As well, because these therapies are intended for a very small number of patients, their overall budgetary impact is low. In fact, a recent analysis found that non-oncology rare disease treatments represented only 1.9% of total public medication expenditure (and a much lower proportion of total health care expenditure) in Canada in 2019.[8] Finally, there are already measures in place, such as negotiations with public and private insurers, to reduce the prices of medicines.

In order to successfully achieve the goal of improving access to rare disease treatments, RAREi recommends that the government include the following key elements as part of its national rare disease strategy:

- A framework approach that ensures more timely and equitable access to rare disease medicines across the provinces, building on models that have worked in other federations, such as the United Kingdom, and that already exist in Canada, such as Canada's Rare Disease Strategy spearheaded by the Canadian Organization for Rare Disorder (CORD)
- Principles, measurable objectives and an external evaluation process
- Clear definitions for rare disease treatments that are aligned with international best practices
- Improvements to Canada's research environment to promote the development and adoption of new rare disease treatments
- A separate specialized regulatory pathway aligned and in cooperation with international comparators, including enhancements to intellectual property protection (e.g., market exclusivity, data protection and/or patent protection)

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[6] https://www.ourcommons.ca/Content/Committee/432/FINA/Reports/RP11058298/finarp01/finarp01-e.pdf

[7] Rawson N., *Regulatory, Reimbursement, and Pricing Barriers to Accessing Drugs for Rare Disorders in Canada,* Fraser Institute, 2018: https://www.fraserinstitute.org/sites/default/files/barriers-to-accessing-drugs-for-rare-disorders-in-canada.pdf

[8] Forte L et al., *The Current and Future Costs of Orphan Drugs in Canada - A Public Payer Budget Impact Analysis*, Patient Access Solutions, 2019 ISPOR Europe Poster, November 2019: https://www.ispor.org/heor-resources/presentations-database/presentation/euro2019-3122/96632.

- Clear and predictable timeframes for funding decisions across all jurisdictions
- A fair and internationally compatible pricing regulation process
- A predictable and efficient health technology assessment and price negotiation process using appropriate parameters for rare disease medicines
- A comprehensive infrastructure to capture and evaluate real-world evidence to drive evidence-based decision-making regarding funding of rare disease medicines

Many of these recommendations were reflected in the "What We Heard" report on *Building a National Strategy for Drugs for Rare Diseases* issued by Health Canada on July 26, 2021.[9]

[9] https://www.canada.ca/en/health-canada/programs/consultation-national-strategy-high-cost-drugs-rare-diseases-online-engagement/what-we-heard.html