RARE i

THE CANADIAN FORUM FOR RARE DISEASE INNOVATORS

Pre-Budget Submission
2022-23

Department of Finance Canada

February 25, 2021

RAREi Recommendations

1 – That the federal government quickly moves ahead with its promised national rare disease treatment strategy and ensures that the related funding is directed in a manner that improves and accelerates access to rare disease treatments for all patients who need them.

2 – That the federal government abandons its proposed pharmaceutical price review reforms in order to facilitate the implementation of policy initiatives that support innovation and improve the lives of Canadians with rare diseases, including the promised national rare disease treatment strategy and the 2021 Biomanufacturing and Life Sciences Strategy.

About RAREi

RAREi is a network of 15 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 quickly moves ahead with its promised national rare disease treatment strategy and ensures that the related funding is directed in a manner that improves and accelerates access to rare disease treatments for all patients who need them

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, and as much as $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 given that 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 to 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.[1]

To address these important gaps, RAREi recommends that the federal government swiftly adopt its promised national rare disease treatment strategy and ensure that the related funding commitment is directed at improving patient access to needed medications across Canada. To that end, RAREi has submitted detailed input to Health Canada to assist its strategy development. RAREi was encouraged by the fact that some of its input was well-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.[2]

However, while RAREi members have been encouraged by Health Canada's open and consultative approach to developing the new strategy, the specific details regarding the strategy and how the funds pledged will be spent have yet to be communicated, even as we are fast approaching the new fiscal year for which funding has been earmarked.

RAREi recognizes that Health Canada has heard many perspectives from a broad range of interested stakeholders, but it remains hopeful that the focus of the new spending will be on improving and accelerating access to rare disease treatments for all who need them. One of the main barriers to access has been an inordinate focus on the prices of these therapies without considering the value and health benefits they offer to patients, the health system and Canada's economic prospects.

There are numerous means in place within Canada to ensure value from reimbursed medicines and manage pharmaceutical budgets effectively. Both public and private health benefit providers have shown that they are

[1] 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

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

able to negotiate very favourable terms with innovators and have proven very resourceful at keeping the share of health care spending represented by medicines stable and growing at moderate rates during the past few decades despite the growing use of treatments for rare diseases.

To be clear, many rare disease medicines have been developed to address complex disorders that may involve far more than just the medicine, including molecular diagnostics and even the use of a patient's own cells to tailor therapeutics. Because these therapies are directed at small numbers of patients, their overall budgetary impact is low and manageable. Moreover, the spending on treatments often leads to avoided costs in other areas of the health care system, such as fewer doctor's visits and other procedures that would be required when the condition persists or flares up. In fact, a recent analysis examining the current and projected public payer costs for non-oncology rare disease treatments determined that they represented just 3.2% of total public expenditure on medications in 2021 and even though medical innovation is expected to generate many more important new treatments in the coming years, they will still only represent 8.3% of total public spending on medicines by 2025.[3] This analysis suggests that concerns regarding unsustainable growth in public spending on rare disease treatments may not be justified.

RAREi believes that a modern, wealthy, and innovative country like Canada should aspire to lead the world in ensuring that vulnerable residents, such as those living with rare disorders, should be able to rely on the predictable and rapid access to the care they require without undue barriers. However, this country also has the capacity and responsibility to be an important contributor to the development of many of those treatments. Embracing innovation and supporting a vibrant and prosperous R&D life sciences sector would help achieve that goal and simultaneously provide much needed economic development and wealth creation that will ensure that we can afford to make needed treatments widely available.

Unfortunately, an important barrier continues to imperil the evolving national rare disease treatment strategy – namely the pharmaceutical price review reforms. In this context, RAREi recommends the following:

Recommendation 2 **– That the federal government abandon its proposed pharmaceutical price review reforms in order to facilitate the implementation of policy initiatives that support innovation and improve the lives of Canadians with rare diseases, including the promised national rare disease treatment strategy and the 2021 Biomanufacturing and Life Sciences Strategy.**

In August 2019, the federal government adopted regulations to change how the Patented Medicine Prices Review Board (PMPRB) regulates patented medicine prices in Canada. In response to the ongoing COVID-19 pandemic, the implementation of these changes has been postponed several times, most recently for the fourth consecutive six-month delay. The new *Patented Medicines Regulations* are now slated to come into effect on July 1, 2022.

While these amendments are not yet in effect, they already have generated substantial market uncertainty, which has delayed patient access to new treatments and reduced investments in advanced clinical trials.[4] They

[3] Poster Presented at the 2021 CAPT AGM: *Historical and projected public spending on drugs for rare diseases in Canada between 2010 and 2025*, Lindy Forte et al., Eversana, 2021.

[4] 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=

have also made it challenging for Canadian pharmaceutical executives to make a compelling business case to their global headquarters for the deployment of new treatments and research investments in Canada.

The main challenge is that the changes offer very little predictability in terms of price compliance for innovators, particularly those operating in the rare diseases space. In addition, the range of mandatory regulatory price decreases compared to current levels demanded by the regulations is very concerning. That market uncertainty has created an impression globally that Canada is not a competitive location to operate in and has already led to some innovators scaling back their commercial activity here. The uncertainty Canada could be compounded further if domestic list prices are increasingly referenced by payers in other major markets, such as the United States.

It must be noted that Canada's complex, slow and expensive medication review and approval process already makes this country a challenging place to do business. Canadian patients already experience significant delays in gaining access to critical medicines when they become available, and that is particularly true for rare disease patients. These proposed price review changes will only exacerbate an already problematic and multi-layered system.

Of even more concern, the PMPRB has pursued the reforms in an aggressive manner, including through an unethical advocacy campaign against stakeholders who disagree with its approach and positions as demonstrated by the PMPRB's February 9, 2021 communications plan.[5]

In fact, when Donald Savoie, a well-respected Canadian public administration and governance scholar, reviewed the PMPRB's February 9, 2021 communications plan in the context of the duty of neutrality, he identified serious concerns. 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."[6]

Dr. Savoie is not the only external observer who has expressed concerns about the PMPRB's actions. 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.[7]

The proposed pricing regulations and guidelines are also completely at odds with the goals of the federal biomanufacturing and life sciences strategy,[8] 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 with the PMPRB regulations that discourage commercialization of innovation and reduce research investments in Canada.

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[5] PMPRB, Communications Plan, February 9, 2022: https://www.dropbox.com/s/eusxuabcq26uqt9/PMPRB%20ATIP%20Disclosure.pdf?dl=0

[6] 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

[7] Federal Court of Appeal, *Alexion Pharmaceuticals Inc. v. Canada (Attorney General)*, July 27, 2021: https://decisions.fca-caf.gc.ca/fca-caf/decisions/en/item/500849/index.do

[8] Innovation, Science and Economic Development Canada, *Canada's Biomanufacturing and Life Sciences Strategy,* July 28, 2021: https://www.ic.gc.ca/eic/site/151.nsf/eng/00019.html

Finally, as delineated above, the proposed reforms pose a significant barrier to the effectiveness of the national rare disease treatment strategy, because if they are implemented as conceived, many innovative medicines for rare diseases will not be launched in Canada. The rare disease community is already in a disadvantageous position in terms of access to new medicines. Most rare diseases do not have a treatment or cure available. Adding another layer of complexity through the proposed regulations, which disincentives companies to launch medicines in Canada, is counterproductive. Negotiations to achieve affordable prices happen every day across the country and are yielding billions of dollars in annual savings. Saving 100% on medicines because they are not available in Canada has a direct and almost incalculable cost in terms of lost lives and poorer health outcomes.

Based on the above, RAREi requests a halt to the pharmaceutical price review reforms in order to support the implementation of policy initiatives that will improve the lives of Canadians with rare diseases. Doing so would demonstrate the Government of Canada's commitment to good governance, stakeholder engagement, innovation, industrial, economic development and health care policy and pandemic preparedness.