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THE CANADIAN FORUM FOR RARE DISEASE INNOVATORS

Pre-Budget Submission
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House of Commons Finance Committee

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RAREi Recommendations

1. Make it a priority to finalize the bilateral deals with provinces and territories associated with the National Strategy for Drugs for Rare Diseases in order to enhance Canadian patient access to orphan medicines

2. Ensure that the Patented Medicine Prices Review Board is not a barrier to the availability of innovative rare disease treatments in Canada

3. Expand the focus of the federal Biomanufacturing and Life Sciences Strategy to improve the policy, research and commercial environment for rare disease innovators

Introduction

Rare diseases affect more than three million Canadians, imposing a significant burden on families, health care systems and society. Despite this, Canada lags behind other nations in supporting rare disease patients. In fact, Canada remains among the only developed nations in the world without a comprehensive national rare disease strategy.

Recent developments offer hope. The federal government's 2023 commitment of $1.5 billion to improve access to rare disease treatments presents an opportunity for Canada to enhance its health care system and become a leader in diagnosing and treating these conditions.

RAREi – a network of 17 innovative biopharmaceutical companies – has proposed a three-point vision to enhance Canada's policy environment, improve the lives of Canadians with rare diseases, and bolster our innovative health system and economy. Specifically:

1. **Better access to therapies:** Patients need better and faster access to medicines that can save or improve their lives. A special system for reviewing rare disease medicines needs to be established to expedite patient access post-Health Canada approval. Patients should receive treatments without making them wait for additional value assessments or price negotiations.
2. **Improved care pathways:** Current rare disease care pathways are difficult to understand and navigate, for patients and care providers. The solution is to create specialized rare disease centres across Canada (similar to cancer care centres) to streamline and enhance access to care and treatment.
3. **Focused on results for patients:** Health care systems tend to prioritize bureaucracy and administrative processes over the well-being of patients. Canada needs to transition toward value-based health care that prioritizes patient outcomes over administrative processes.

This committee is well-positioned to help advance these recommendations and contribute to making this vision a reality. For further details, visit www.rarei.ca/vision.

Recommendations in Context

***Recommendation 1* – Finalize the bilateral deals with provinces and territories associated with the *National Strategy for Drugs for Rare Diseases* in order to enhance Canadian patient access to orphan medicines**

More than five years have passed since the federal government first announced a pledge to fund rare disease medicines and 18 months since it launched the *National Strategy for Drugs for Rare Diseases* (DRDs). That strategy pledged more than $1.5 billion, the majority of which ($1.4 billion) was for provinces and territories (P/Ts) through bilateral agreements to help them expand public access to rare disease treatments.

The Canadian rare disease community welcomed the announcement and launch of the strategy, but RAREi notes that this is just the beginning of a comprehensive strategy needed to support rare disease patients. Its hope is this is just a first step towards a broader policy.

In the meantime, the implementation and getting the promised funding flowing nationwide is complicated by the recent introduction of national pharmacare legislation. Bill C-64 calls for yet another set of bilateral negotiations with P/Ts to distribute the five-year, $1.5 billion pledge intended to facilitate expanded public coverage for contraceptives and diabetes medicines and supplies.

Regarding the DRD strategy, time is of the essence, and the promised funds need to start flowing as soon as possible. RAREi was pleased to see the first bilateral agreement signed with British Columbia in July 2024, which will finally start the flow of funding for patients in need. In particular, RAREi was encouraged by the federal government's approach to the agreement which respects jurisdictional roles, recognizes that P/Ts are responsible for publicly funded medication coverage and lines up with the existing pharmaceutical review and approval systems in Canada. This stands in sharp contrast to its proposed national pharmacare approach which could override P/T and private drug plan decision-making and impose a federally dictated system on top of existing processes. RAREi urges Health Canada to finalize similar agreements with remaining jurisdictions promptly.

The reality is that only 60% of innovative treatments for rare disorders are approved by Health Canada. And when authorized, it can take up to six years longer than in the United States and Europe for them to become available for patients.[1] Even when approved and launched, patients face challenges in securing public funding. The time between regulatory approval and listing on public provincial formularies averaged 736 days in 2022, double the average time reported in comparable countries.[2] As well, there is a significant difference in reimbursement timelines between public and private drug plans in Canada. According to one study, private plans take 142 days on average to cover new medicines following Health Canada's approval compared with 449 days for public drug plans.[3] As a result, many people with rare disorders in Canada are missing out on treatments that could save or significantly improve their lives. RAREi is hopeful that the funding in the bilateral agreements will accelerate public funding of rare disease medicines approved by Health Canada.

In this context, RAREi is asking the federal government to ensure that the national pharmacare initiative does not divert from or upend Health Canada's efforts to negotiate funding deals related to the *National Strategy for Drugs for Rare Diseases*. It is vital that the rare disease strategy negotiations be prioritized in light of already lengthy delays and the fact that most Canadians who need access to contraceptives and diabetes medications and supplies are generally well covered nationally.

Recommendation 2 **– Ensure that the Patented Medicine Prices Review Board is not a barrier to the availability of innovative rare disease treatments in Canada**

The Patented Medicine Prices Review Board (PMPRB) is consulting now on new price review guidelines for innovative pharmaceuticals that align with updated regulations and recent court decisions regarding the constitutional mandate of the board.

The regulations passed in 2022, removed the United States and Switzerland from the list of comparator countries and added six lower-priced jurisdictions. That list informs the "international price comparison" that the PMPRB is required to consider – among other factors – when reviewing patented medicine prices to determine if they are "excessive" as a function of abuse of intellectual property rights.

The PMPRB's recent discussion paper on new guidelines proposes a triage method for determining when a new medicine could be subject to an "in-depth review" and potentially recommended for a judicial hearing by the Board. However, there remains significant uncertainty regarding how the PMPRB will apply the criteria it is

[1] Ward L et al, An international comparative analysis of public reimbursement of orphan drugs in Canadian provinces compared to European countries, *Orphanet Journal of Rare Diseases*, March 4, 2022: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC8895096.

[2] Conference Board of Canada, Access and Time to Patient Prescription Drugs in Canada, January 4, 2024: https://www.conferenceboard.ca/wp-content/uploads/2022/10/access-and-time-to-patient_jan2024.pdf.

[3] Canadian Health Policy Institute, Coverage of new medicines in public versus private drug plans in Canada 2008-2017, 2018: https://www.canadianhealthpolicy.com/product/coverage-of-new-medicines-in-public-versus-private-drug-plans-in-canada-2008-2017-2

required to consider when recommending a hearing. Ceiling prices that are reviewed and effectively regulated by the PMPRB have a material effect on the timing and availability of new medicines coming to the Canadian market. The years-long uncertainty that Canada experienced during the PMPRB regulatory reforms had a very significant and negative effect on access to globally launched medicines, including treatments for rare diseases.[4, 5]

The PMPRB is an independent, quasi-judicial body, but the government remains responsible for the legislation and regulations that set out the Board's mandate. With that in mind, in its 2021 recommendations to the government, this committee called on the government to "Ensure that the Patented Medicine Prices Review Board changes do not create barriers for new medicines for Canadians." Given that we are still debating the same issues three years later, RAREi encourages FINA to reiterate its 2021 recommendation this year.

Recommendation 3 **– Expand the focus of the federal Biomanufacturing and Life Sciences Strategy to improve the policy, research and commercial environment for rare disease innovators**

As personalized medicine advances, rare disease treatments are at the forefront of medical innovation. At the same time, the challenges faced by Canadians affected by rare disease are numerous. They include the very long journey to reach a diagnosis, small patient populations, fewer health care resources available to treat the conditions and many unknowns about the disease. For many rare disease patients, there are no treatments available. That is why it is important to ensure that those that are developed can be made available to patients who need them as soon as possible.

Orphan treatment innovators are in turn challenged by a high degree of uncertainty tied to many variables. There is huge variation in the prevalence and incident rates country to country, province to province and even region to region. For genetic conditions, differing mutations can affect the progression and severity of disease. How conditions are managed, who is eligible for treatment, and even by whom they are managed can also vary considerably. Add to that the high upfront cost of doing business in Canada and the fact that Canada represents just 2% the global pharmaceutical market, and one can begin to see why it is vital that Canadian policymakers take care to implement regulatory, policy and funding initiatives that support and encourage innovation.

Other countries have implemented tailored regulatory and reimbursement review processes and provided incentives such as enhanced intellectual property, and, for rare disease treatments, recognizing the high risks and costs involved. These policies have led to a significant increase in new treatments for rare conditions globally. The dramatic increase in the number of new treatments for orphan conditions can be traced directly to ground-breaking policy choices made by governments in the US, Europe and Asia that are designed to encourage research and innovation that targets rare diseases.

Canada has a critical opportunity to take action in this area and catch up internationally. It can also capitalize on the momentum currently driven by federal, national, provincial and stakeholder efforts in rare disorders policy and life sciences.

The 2021 federal Biomanufacturing and Life Sciences Strategy (BLSS) contains some elements that can help improve Canada's competitiveness on the global stage, including an emphasis on enabling innovation by

[4] Life Sciences Ontario Webinar, New Medicine Launches: Canada in a Global Context, June 22, 2020: https://lifesciencesontario.ca/wp-content/uploads/2020/06/EN_LSO_Global-Launch-Benchmarking_Webinar-June22-20_Final.pdf.
[5] Grootendorst P & Spicer O, The effect of patented drug price on the share of new medicines across OECD countries, *Health Policy*, Vol. 126, Issue 8, August 2022, pp. 795-801: https://pubmed.ncbi.nlm.nih.gov/35654617.

ensuring world class regulation. Still the government should go further, by linking the BLSS to the *National Strategy for Drugs for Rare Diseases* in a way that facilitates access and drives innovation.

In this context, RAREi recommends that the federal government consider adding the following rare disease research elements to its BLSS.

- *Formal rare disease treatment designation* – Based on agreed upon eligibility criteria similar to those used in other leading jurisdictions, Health Canada would designate new medicines as rare disease treatments. The designation would guarantee that the product would be reviewed and considered as a priority in the Canadian medication review and approval process. In addition, the sponsor would be encouraged to take advantage of any regulatory measures designed to permit adaptive approaches to review and have access to additional incentives as outlined below.
- *Waiver or reduction in review fees* – In cases when the anticipated number of patients to be served is small and/or the expect budget impact of a product is likely to be minimal, sponsors with an orphan designation should have reduced or waived submission fees from federal regulators and health technology assessment bodies, most notably Canada's Drug Agency and Quebec's INESSS.
- *Additional intellectual property protection* – When innovators undertake clinical research in orphan diseases, the opportunity for extended patent life, additional data protection or market exclusivity should be available.
- *Regulatory sandboxes* – As part of the federal regulatory modernization framework, the government has proposed the use of regulatory sandboxes as a means of road-testing new and innovative approaches to review and assessments. Opportunities should encourage government, researcher and industry innovators to propose and pursue new approaches with regulators, reviewers and payers to assessing and making products available for patients.

Adding any or all of these to the Canadian system would demonstrate that Canada wishes to compete for the economic development and health innovation opportunities that arise from rare disease development. They should be considered as core elements of both the BLSS and the rare disease strategies.