RAREi
THE CANADIAN FORUM FOR
RARE DISEASE INNOVATORS

Written Submission for the Pre-Budget Consultations in Advance of the 2020 Budget

Government of Canada
House of Commons Standing Committee on Finance

By RAREi – The Canadian Forum for Rare Disease Innovators

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Recommendations from RAREi – The Canadian Forum for Rare Disease Innovators

Recommendation 1. That the federal government move forward with dedicated funding for rare disease treatments.

Recommendation 2. That the federal government implement a holistic national rare disease strategy to improve access to diagnosis, care and treatments for all Canadians with rare diseases.

Recommendation 3. That the federal government expand efforts to grow Canada's life sciences sector, including investments and incentives for rare disease research, biotechnology companies and clinical excellence in rare diseases.

About RAREi

RAREi – Canada's Forum for Rare Disease Innovators is a network of Canadian biopharmaceutical companies that are committed to improving the lives of patients around the world living with rare disorders by researching, developing and commercializing rare disease treatments. RAREi includes Alexion Pharma Corp., Amicus Therapeutics Canada Inc., Biogen Canada Inc., BioMarin Pharmaceutical (Canada) Inc., Horizon Therapeutics Canada, Ipsen Biopharmaceuticals Canada Inc., Mitsubishi Tanabe Pharma Canada, Inc., Recordati Rare Diseases Canada Inc., Sobi Canada, Inc., and Vertex Pharmaceuticals (Canada) Inc.

Recommendations in Context

Recommendation 1. That the federal government move forward with dedicated funding for rare disease treatments.

Incredible scientific and technological advances in recent years have improved our collective understanding of many rare diseases and created a very exciting time for the rare disease community. However, too many Canadians cannot access the benefits of these technologies because they are not funded by provincial and federal drug programs and individuals and their families cannot afford the medicines on their own.

In this context, RAREi is very encouraged by the attention policymakers have given to issues facing the rare disease community in Canada in recent years. The federal Budget 2019's allocation of specific funding to improve patient access to rare disease therapies is a welcome step in the right direction. When supported by the two-year $1 billion funding commitment that will begin in 2022-23 (and provide up to $500 million annually thereafter), patients could begin to see a real improvement in their access to rare disease therapies.

The new funding responds to a critical gap and need in Canada. Of the approximately 7,000 rare diseases, there are only effective treatments for 5% of these conditions. Many of these conditions are severely debilitating or life-threatening and the affected patients and families face many challenges living with these conditions. More than 79% of Canadians believe there should be equal access to rare disease therapies across the country.[1]

Some commentators have raised concerns about the financial impact of extending public coverage for rare disease treatments due to their high sticker prices. However, there is data that indicate it is a very small percentage of the public drug plan and broader health care budgets. Most recently, data presented recently by the federal Patented Medicine Prices Review Board estimated that Canada spent only 1.8% of its total pharmaceutical bill on non-oncology medications for rare diseases in 2017-18.[2]

In this context, providing funding for rare disease medicines of approximately $500 million per year will go a long way to extending access to thousands of Canadians who currently are unable to benefit from treatments that they need to survive and have better quality lives. Patients face critical gaps in accessing these treatments today and RAREi urges the federal government to implement its commitment before its intended timeline of 2022-23.

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[1] Polisena, J. et al, Engaging the Canadian public on reimbursement decision-making for drugs for rare diseases: a national online survey, *BMC Health Services Researc*h, May 26, 2017: https://doi.org/10.1186/s12913-017-2310-4.

[2] Lungu, E., What is the "Expense" for Expensive Drugs for Rare Diseases? *2019 CADTH Symposium*, April 2019: http://www.pmprb-cepmb.gc.ca/CMFiles/News%20and%20Events/Speeches/edrd-april2019-en.pdf.

Recommendation 2. **That the federal government implement a holistic national rare disease strategy to improve access to diagnosis, care and treatments for all Canadians with rare diseases.**

Budget 2019 also announced that the federal government will work with stakeholders and other governments to develop of a "national strategy for high-cost drugs for rare diseases to help Canadians get better access to the effective treatments they need."[3] This is extremely important given that Canadians with rare diseases face disproportionately low access to new therapies.[4]

RAREi therefore supports the government's commitment to expand access to effective treatments and is pleased to take this opportunity to highlight how Canada's system for evaluation and funding should be enhanced in this context.

Specifically, the strategy should begin by reviewing all elements of the Canadian process, including regulatory review, pricing oversight, health technology assessment, product negotiations and funding. Each stage needs to be streamlined and customized in a manner that addresses the needs of patients with specific rare diseases rather than the current broad population-based approach used in assessing medications with large populations.

Distinct approaches to the review and reimbursement process for rare disease therapies should include:

- **A regulatory framework that incents the development and commercialization of rare disease therapies.** This would include a definition of rare disease, an orphan product designation process, additional market exclusivity, research promotion funds, tax incentives and regulatory submission fee reductions.
- **Pricing tests and tools that do not rely on cost-effectiveness assessments or have a disproportionate impact on rare disease treatments.** The currently proposed reforms to patented medicine pricing would introduce a huge barrier to access by establishing a restrictive and uncertain new price assessment process, which will result in regulated price reductions of 70-90% for rare disease medicines.[5] In addition, the proposed reforms would have a disproportionate effect on rare disease treatments, as demonstrated in a study that explored the impact of proposed reforms on hypothetical rare disease treatments,[6] and should be reconsidered.
- **Health technology assessments (HTAs) conducted by the Canadian Agency for Drugs and Technologies in Health should include processes, criteria and standards that are appropriately suited for rare diseases.** HTA reviews can be enhanced by formally incorporating specialized clinician expertise, patient values, smaller study sizes, more frequent reliance on observational studies and real-world evidence beyond randomized clinical trials, and the requirement for higher prices for these treatments per patient. The pan-Canadian Oncology Drug Review process, which involves patients, clinicians and manufacturers throughout, can be drawn upon as a best practice.

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[3] Government of Canada, Budget 2019: Investing in the Middle Class, p. 58, March 19, 2019: https://budget.gc.ca/2019/docs/plan/budget-2019-en.pdf.

[4] Rawson, N., *Regulatory, Reimbursement and Pricing Barriers to Accessing Rare Disorder Drugs in Canada*, Written brief to the House of Commons Standing Committee on Health, November 16, 2018: https://www.ourcommons.ca/Content/Committee/421/HESA/Brief/BR10186527/br-external/RawsonNigel-e.pdf.

[5] 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.

[6] See also Rawson N., *New Patented Medicine Regulations in Canada: Case Study of a Manufacturer's Decision-Making about Regulatory Submission for a Rare Disorder Treatment*, Canadian Health Policy Institute, October 2018: https://www.canadianhealthpolicy.com/products/new-patented-medicine-regulations-in-canada--case-study-of-a-manufacturer---s-decision-making.html.

- **A reimbursement process, including product negotiation and funding decisions, that provides timely and equitable access to rare disease treatments.** The product negotiation and funding decision process for public drug plans should implement measures that draw on international innovations, such as allowing reimbursement at the time of regulatory approval, the increased use of pay-for-performance, managed access programs and real world evidence to address clinical and economic uncertainties without stalling or delaying patient access.

- **A multi-stakeholder approach to inform the provincial and territorial governments' proposed supplemental reimbursement review process for complex and specialty medicines.** Many of the initiatives listed above are being considered by the provincial and territorial working group focused on improving access to rare diseases.[7] The federal government can play a coordinating, supportive and policy role to ensure that the expertise and input of all key players – regulators, evaluators, funders, developers and, most importantly, patients – are at the table designing any updated review process.

- **Investments in real world evidence (RWE) research, infrastructure and policy development in order to generate data and support optimal use of medicines.** RWE can fill critical clinical gaps and increase certainty for public drug plans, payers and manufacturers, especially for rare disease therapies. Health Canada is already taking part in efforts to expand the use of RWE throughout the review and funding process, through participation in the multi-stakeholder efforts coordinated by the Canadian Agency for Drugs and Technologies in Health, the Canadian Association for Population Therapeutics, Health Canada and the Institute for Health Economics. Building on the workshop held in October 2018, the federal government should continue to support the development of a national RWE framework.[8] Specific federal investments can include funding for research, patient registries and centres of clinical expertise, as well as enhanced regulatory reviews that support manufacturer data development submitted in Health Canada regulatory applications.

Please note that several of these initiatives are further elaborated in RAREi's recommendations to the House of Commons Standing Committee on Health,[9] and some are highlighted in the final report of the federal government's Advisory Council on the Implementation of National Pharmacare.[10] Both of these committees recognized the need for a distinct approach to rare disease treatments.

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[7] Expensive Drugs for Rare Diseases Working Group, Stakeholder Consultation: Supplemental Process for Complex/Specialized Drugs Background Document, November 2018: http://www.raredisorders.ca/content/uploads/EDRD-supplemental-process-background_24Oct2018_Final.pdf. *See also* RAREi - The Canadian Forum for Rare Disease Innovators, RAREi Responses to Consultation Questions, December 2018: https://www.linkedin.com/feed/update/urn:li:activity:6559059577416007680, and Expensive Drugs for Rare Diseases Working Group, Stakeholder Engagement Summary: Supplemental Process for Complex and Specialized Drugs, July 2019: https://www.linkedin.com/feed/update/urn:li:activity:6558035111844073472.

[8] Canadian Agency for Drugs and Technologies in Health (CADTH), Canadian Association for Population Therapeutics (CAPT), Health Canada, and Institute for Health Economics (IHE), Defining decision-grade real-world evidence and its role in the Canadian context: A design sprint – Summary report of a workshop, October 21, 2018: https://www.ihe.ca/events/past/conferences/ihe-capt-rwe/ihe-capt-rwe-about.

[9] RAREi – The Canadian Forum for Rare Disease Innovators, Unique approach needed: Addressing barriers to accessing rare disease treatments, October 31, 2018: https://www.linkedin.com/feed/update/urn:li:activity:6556579888877363200. *See also* Standing Committee on Health, Canadians Affected by Rare Diseases and Disorders: Improving Access to Treatment, February 2019: https://www.ourcommons.ca/DocumentViewer/en/42-1/HESA/report-22/.

[10] Advisory Council on the Implementation of National Pharmacare, A Prescription for Canada: Achieving Pharmacare for All, June 12, 2019: https://www.canada.ca/en/health-canada/corporate/about-health-canada/public-engagement/external-advisory-bodies/implementation-national-pharmacare/final-report.html.

Recommendation 3. **That the federal government expand efforts to grow Canada's life sciences sector, including investments and incentives for rare disease research, biotechnology companies and clinical excellence in rare diseases.**

RAREi is very encouraged by the work of the Health/Bio-sciences Economic Strategy Table (HBEST). HBEST has set out an ambitious but achievable objective to double the size of Canada's health and biosciences sector and become a top-three global hub by 2025 through a number of specific strategies, including: leveraging and advancing innovative technologies; attracting and retaining capital, skills and talent; and ensuring a vibrant ecosystem that will unleash the full potential of the sector and lead to improved health outcomes.

HBEST's strategic roadmap includes accelerating innovation adoption, regulatory agility, digital technologies, talent development and attraction and the creation of anchor firms, which would benefit health systems, Canada's innovation economy and, most importantly, Canadian patients.

HBEST also highlights barriers to achieving these goals, including federal regulatory processes along with "layers of provincial/territorial, regional and local regulations, policies and procedures that impact the adoption of innovative products." In addition, the report suggests that the policy reform associated with the Patented Medicines Price Review Board is a potential barrier to growth and that industry concerns should be addressed.[11]

RAREi members stand ready to contribute to the success of HBEST building on our accomplishments to date. In recent years, RAREi members have injected billions of dollars into the Canadian economy via partnerships and investments in Canadian biotechnology companies, research institutes and academic programs across Canada. These investments have helped Canadian innovations leverage global capital and expertise to reach patients in Canada and around the world. Two notable examples include Alexion's acquisition of Enobia Pharma Corp. for over $1 billion and Ipsen's purchase of Clementia for over $1.5 billion.

Our members hope to continue to invest in research partnerships and funding of Canadian innovation in the coming years, and believe that the HBEST roadmap and removal of barriers to the sector growth should be key features of federal government's 2020 Budget.

[11] Canada's Economic Strategy Tables: Health and Biosciences, The Innovation and Competitiveness Imperative: Seizing Opportunities for Growth, p. 9, September 27, 2018: https://www.ic.gc.ca/eic/site/098.nsf/vwapj/ISEDC_HealthBioscience.pdf/$file/ISEDC_HealthBioscience.pdf.