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THE CANADIAN FORUM FOR RARE DISEASE INNOVATORS

Government of Canada
Department of Finance

Pre-Budget Submission for Budget 2024

February 9, 2024

About RAREi

RAREi is a network of 18 Canadian biopharmaceutical companies committed to improving the lives of Canadians with rare diseases by researching, developing and commercializing rare disease treatments.

The member companies of RAREi are:

Alexion Canada
Amgen Canada
Amicus Therapeutics
argenyx Canada
Astellas Canada
Biogen Canada
Biomarin Pharmaceutical
Boehringer Ingelheim Canada
GlaxoSmithKline Canada
Horizon Therapeutics
Ipsen Canada
Janssen Canada
Mitsubishi Tanabe Pharma Canada
Recordati Rare Diseases
Sanofi Canada
Sobi Canada
Takeda Canada
Ultragenyx Pharmaceutical Canada
Vertex Canada

The impact of rare diseases on Canadians

The Canadian Organization for Rare Disorders (CORD) defines a rare disease as a condition affecting fewer than one person in 2,000 during their lifetime. There are more than 7,000 known rare diseases and dozens more are discovered each year.

While each disorder might be rare, collectively, one in 12 Canadians are affected by a rare disease, or more than 3.2 million Canadians, not including their families.

Families are important to consider, given that two-thirds of rare disorders occur in children, often as a result of inherited genetic conditions. One in four children with a rare disease will not live to see their 10th birthday.[1]

Canadians affected by often severe and debilitating rare disorders struggle to access needed treatments. This is in addition to the multiple other challenges they face, including 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 yet to treat their condition. 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.

All of this makes rare diseases a very significant health issue and burden for Canadians.

[1] Canadian Organization for Rare Disorders, Key Facts, https://www.raredisorders.ca/about-cord/

RAREi recommendations for Budget 2024

1. Ensure that the National Strategy for Drugs for Rare Diseases (DRDs) funding intended for transfer to the provinces and territories flows as soon as possible.

2. Provide policy direction to the Patented Medicine Prices Review Board (PMPRB) that ensures it takes a light touch to price review and that it avoids measures that would discourage medical innovations from being launched in Canada.

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

Details of RAREi recommendations for Budget 2024

RECOMMENDATION #1:

Ensure that the National Strategy for Drugs for Rare Diseases (DRDs) funding intended for transfer to the provinces and territories flows as soon as possible.

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 and in spring 2022, it shared a revised draft framework for stakeholder consideration.

Unfortunately, it took until spring 2023, for the promised strategy to be unveiled. Despite the delays, the Canadian rare disease community welcomed the announcement and the creation of a dedicated directorate within the department to address rare disease policy.

The March 2023 announcement came with a major funding commitment of $1.5 billion during the next three years, of which more than 90% ($1.4 billion) is dedicated to special transfers to the provinces and territories (PTs) to facilitate improved access to new and existing rare disease treatments. The PT funding is to be distributed based on bilateral agreements with each of the participating jurisdictions, but is dependent on first achieving national agreement on a small subset of medicines that would be expected to be reimbursed equally across the country.

It is almost a year later and not one rare disease patient has had their treatment covered as a result of the strategy. Moreover, there is no indication that the barriers to making that happen will be eliminated any time soon.

Based on RAREi's discussions with federal and PT officials, it is clear that no consensus has been achieved regarding the list of medicines and the bilateral negotiations are not making progress, despite the pending end of the first fiscal year of the three-year commitment. The snail's pace at which the

inter-governmental negotiations are proceeding is disappointing, as is the lack of involvement by the rare disease community in framing and facilitating access in a way that meets patients' needs effectively. Canadian rare disease patients deserve better and RAREi has specific suggestions to help unlock these long-awaited funds.

The needs are huge and Canada is behind other developed countries. Numerous academic studies have shown that Canadians with rare diseases have access to far fewer medicines or wait for them much longer than many patients in other jurisdictions, including the United States and Europe.[2,3] A study published in June 2022 showed that between 2014 and 2021 most non-oncology rare disease treatments in Canada ultimately received positive health technology assessment reviews but the process took years (median time 663 days) and patient access is limited by restrictive listing criteria and the lengthy decision-making processes.[4]

It is important to remember that while the spending required to adequately provide rare disease treatments to Canadians is not insubstantial, a recent study showed it is now and is projected to remain a very small percentage of overall public medication spending into 2025 (8%, not including the impact of substantial confidential manufacturer rebates). The study authors conclude: "Projected DRD spending shows robust growth but remains a fraction of total public drug spending. Limiting (rare disease treatment) access because of this growth is not aligned with Canadian patient or societal values."[5]

RAREi has proposed several ways to move the process along.

Regarding the common list of medications, RAREi notes Canada already has a national list of medicines for public reimbursement. The list is organically created and updated monthly. All the major public drug plans in Canada are participating members in the pan-Canadian Pharmaceutical Alliance (pCPA), which negotiates favourable terms with innovators to facilitate public reimbursement. Successful negotiations lead to mutually agreed commercial terms outlined in a letters of intent (LoI) that apply nationally. Many rare disease treatments have already been negotiated successfully through the pCPA and others are under negotiation currently or proceeding through the evaluation process. The list that the government seeks to create is already developed and subject to agreed-upon terms by all Canadian jurisdictions. Federal funding should simply be earmarked to enhance and speed up the implementation of letters of intent by the provinces, effectively cost-sharing with the provinces and – via rebates and other commercial terms – medicine developers.

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[2] Chambers A et al., An international comparative analysis of public reimbursement of orphan drugs in Canadian provinces compared to European countries, *Orphanet Journal of Rare Diseases*, Issue 17, Article number 113. March 2022, https://ojrd.biomedcentral.com/articles/10.1186/s13023-022-02260-6

[3] Rawson N, Availability and Accessibility of Essential Drugs for Rare Disorders in Canada, *Canadian Health Policy Journal*, Oct. 13, 2021, https://www.canadianhealthpolicy.com/product/availability-and-accessibility-of-essential-drugs-for-rare-disorders-in-canada-2.

[4] Rawson N, Health technology assessment and price negotiation alignment for rare disorder drugs in Canada: Who benefits? *Orphanet Journal of Rare Diseases*, Issue 17, Article number 218. June 2022, https://ojrd.biomedcentral.com/articles/10.1186/s13023-022-02390-x

[5] Lech R et al, Historical and projected public spending on drugs for rare diseases in Canada between 2010 and 2025, *Orphanet Journal of Rare Diseases*, Vol. 17, Article number: 371, October 2022, https://ojrd.biomedcentral.com/articles/10.1186/s13023-022-02534-z

In sum, RAREi urges Finance Canada and Health Canada to take immediate action to ensure that the $1.4 billion set aside for the PTs to promote improved patient access to DRDs begins to flow as soon as possible.

RAREi also recommends that, without further delaying treatment access, the strategy should be expanded – it should go beyond treatments and be anchored in a broader approach that would consider diagnosis, care and other elements of managing a rare disease, including support for the Canada's life sciences sector.

In January 2023, the United States marked the 40th anniversary of the passage of its 1983 *Orphan Drug Act* (ODA) which has had a profound positive impact on the development and availability of treatments for rare diseases in that country by introducing tax incentives and regulatory pathways to facilitate approval of such treatments. The ODA has been successful in its objective. In 1983, when the law was passed, only 38 medicines were approved in the US specifically to treat orphan diseases. However, between 1983 and 2019 more than 5,000 medicines were approved and in the last decade in particular the number of medications available to treat rare diseases in children has increased markedly.[6]

Canada urgently needs a broader rare disease strategy to achieve similar positive results for Canadians with rare diseases and to address what CORD calls a "crisis in rare disease in Canada" that is "costly in terms of dollars and lives."[7]

RECOMMENDATION #2:

Provide policy direction to the Patented Medicine Prices Review Board (PMPRB) that ensures it takes a light touch to price review and that it avoids measures that would discourage medical innovations from being launched in Canada.

Since 2017, the Patented Medicine Prices Review Board (PMPRB) has been pursuing a guidelines modernization effort associated with the federal government's pledge to improve Canadians' access to medication coverage and address affordability concerns.

From the rare disease community's perspective, the draft guidelines proposed by the PMPRB in earlier attempts at rewriting the rules did not acknowledge or account for the additional challenges represented by rare diseases and, if implemented, would have significantly exacerbated the difficulties faced by rare disease patients, researchers, clinicians, developers and health systems. To state it plainly, the market uncertainty that has characterized the PMPRB's lengthy price review modernization

[6] Adams J and Rawson N, Opinion: For drug access, Canadians with rare disorders are 40 years behind Americans, *Financial Post*, Jan. 24, 2023, https://financialpost.com/opinion/drug-access-canadians-rare-disorders-40-years-behind-americans

[7] Wong-Rieger D, Open letter to Health Ministers, Canadian Organization for Rare Disorders, Nov. 1, 2022, http://www.raredisorders.ca/content/uploads/CORD-letter-to-health-ministers-and-attachment_01Nov2022.pdf

journey has been damaging to Canada's attractiveness as a location for global investments in research and development.

In addition to being problematic for rare disease patients, the PMPRB's attempts to impose a review process designed to reduce patented medication prices in Canada were the subject of significant stakeholder opposition and were eventually rejected as largely unconstitutional by federal courts.

In response, the board has begun to recalibrate its approach. It held a series of roundtables in December and received 70 stakeholder submissions addressing how it should move forward with the reforms in manner that is consistent with its clarified mandate – to ensure that patentees are not abusing their rights by pricing products excessively during a period of market exclusivity. It is now in the process of reviewing all the input and intends to develop a new set of price review guidelines for further feedback during the remainder of 2024.

Overall, RAREi is encouraging the board to adopt a more minimalist and balanced approach to price regulation that would be consistent with its recently clarified mandate and believes that the PMPRB should confine its role to acting as a backstop to protect Canadians against excessive pricing of patented products while they remain on patent, not engaging in price controls or attempts to lower prices where there is no evidence of abuse. Furthermore, the new guidelines should be designed to reduce uncertainty for rare disease innovators.

In addition, RAREi has encouraged the board to take care to ensure that its price review activities are aligned and consistent with some broader policy platforms being pursued by the federal government such as a multi-year biomanufacturing and life sciences strategy, a genomics strategy, a pediatric medication development initiative, a variety of regulatory modernization initiatives related to health product review and approval and the national rare disease drug strategy. The government should support the national-level goal of encouraging a healthy, prosperous and globally competitive life sciences ecosystem in Canada.

It must be stressed that this is an extremely exciting time for the rare disease community given the incredible scientific and technological advances underway. However, the benefits of many of these technologies will not reach Canadian patients in a timely manner if the PMPRB guidelines are not crafted carefully and in a way that would position Canada as an example to follow internationally in terms of providing timely access to rare diseases treatment and a place to pursue real innovation.

RECOMMENDATION #3:

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

RAREi believes that a modern, wealthy and innovative country like Canada should aspire to be a life sciences leader and an important contributor to the development of innovative rare disease treatments.

We have already seen remarkable progress in this regard, as was demonstrated by the very rapid development of innovative and very effective vaccines against COVID-19 using new genetic knowledge and techniques. The understanding of the human genome and the genetic basis of disease has exploded, reaching a crucial tipping point. The same is true with the tools to put that knowledge to use to create effective treatments, and even outright cures, of previously untreatable conditions.

There is now an opportunity to embrace the dramatic changes in science and health care and harness them for the benefit of both patients and Canada's economic development by building a life sciences sector that is a vital pillar of a prosperous and growing economy.

However, there are vulnerabilities in the current Canadian health and innovation ecosystem. Life sciences is very much a global industry that will locate and invest in the areas that offer the best overall environment and potential. A crucial element of that is having the policies in place that promote and value health innovation and the huge investments that are needed to create it. It is in this area that Canada has often fallen short.

As medical research evolves into an era of increasingly personalized treatments that rely on a genomics-based understanding of disease and illness, rare disease treatment development represents something of a vanguard on which many developed countries are increasingly taking advantage to drive economic growth.

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 build out its capacity in this area and catch up with our counterparts to the south and overseas. Canada has a unique opportunity to capitalize on the momentum currently driven by federal, national, provincial and stakeholder efforts in rare disorders policy and life sciences.

The federal biomanufacturing and life sciences strategy (BLSS) announced in the summer of 2021 contains some elements that can help improve Canada's competitiveness on the global stage, including an emphasis on enabling innovation by ensuring world class regulation. Still the government should go further, by linking the BLSS to the national rare disease drug strategy 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 orphan drug designation* – Based on agreed upon eligibility criteria, Health Canada would designate new medicines as orphan drugs. The designation would guarantee that the product would be reviewed and considered as a priority throughout 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.

- *Potential for 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 should be encouraged to submit products as part of the medication review and approval process by offering them reduced or no fees for any reviews required.
- *Additional intellectual property protection* – When innovators undertake clinical research in orphan diseases, the opportunity for extended patent life and/or additional data protection 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 opportunities that arise from rare disease development. They should be considered as core elements of both the BLSS and the DRD strategies.