RARE i

THE CANADIAN FORUM FOR RARE DISEASE INNOVATORS

2024 Budget Consultations

Ontario Ministry of Finance

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Recommendations:

1) The Ontario government should demand that the federal government release the province's share of funding from the national Drugs for Rare Diseases Strategy so that it can invest in timely access to medicines for rare diseases.
2) The Ontario government should develop and implement a provincial rare disease strategy.
3) The Ontario government should invest in rare disease infrastructure as an economic driver.

About RAREi

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

Context

About 1 in 12 Canadians, roughly two-thirds of whom are children, are living with a rare disease. Fewer than 10% of known rare diseases have a treatment available currently.[1] However, promising new advancements in medical research have led to a growing number of new and emerging therapeutics that are offering, and will offer, incredible benefits to patients.

However, even when treatments are available, many patients experience great challenges in receiving access to them. That is because there is no pathway or criteria that distinguishes these treatments within Health Canada's regulatory framework or any of the public reimbursement review and approval processes in Canada.[2] As a result, treatments for rare diseases are generally launched in Canada much later than in the United States and/or Europe and, in some instances, they do not even come to Canada at all.[3] In fact, only 18% of new rare disease medicines launched globally are publicly available to Canadians, which increases to 44% if private plans are included. And when rare disease treatments do make it to Canada and are approved by Health Canada, there is still a greater than two-year gap before most patients are able to publicly access those treatments. This adds to the lengthy delay patients face, during which they often experience disease progression or even death.[4]

Canada remains one of the only developed countries in the world without a national strategy for rare diseases and Ontario remains without its own rare disease policy or strategy. With the federal government's announcement last year of $1.5 billion for a Canada-wide strategy to fight rare diseases, now is the time to take action and ensure that rare disease patients can benefit from the federal funding that has already been committed.

Recommendation 1: The Ontario government should work with the federal government to prompt release of the province's share of funding from the national Drugs for Rare Diseases Strategy so that it can invest in timely access to medicines for rare diseases.

The vast majority of new funding promised by the federal government as part of the *National Strategy for Drugs for Rare Diseases* - $1.4 billion out of $1.5 billion – is earmarked for provinces and territories. Ontario stands secure more than $500 million of the funding, which would greatly enhance public access to new and emerging orphan treatments and diagnostics. While the funding commitment was

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

[2] Ward, L.M., Chambers, A., Mechichi, E. et al. An international comparative analysis of public reimbursement of orphan drugs in Canadian provinces compared to European countries. Orphanet J Rare Dis **17**, 113, 2022: https://doi.org/10.1186/s13023-022-02260-6

[3] 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

[4] PhRMA analysis of IQVIA MIDAS and U.S. Food and Drug Administration (FDA), European Medicines Agency (EMA) and Japan Pharmaceuticals and Medical Devices Agency (PMDA) data. August 2022. Note: New medicines refer to new active substances approved by FDA, EMA and/or PMDA and first launched in any country between January 1, 2012, and December 31, 2021. Data excludes Chile, Colombia, Costa Rica, Denmark, Estonia, Greece, Iceland, Israel and Luxembourg.

welcomed by Canada's rare disease community, nearly a year has passed and not a single patient has received funded access to new medicines.

Canadian rare disease patients need urgent action from federal/provincial and territorial government leaders to secure that funding and enhance and improve access to rare disease medicines.

One of the apparent sticking points from the federal government is its insistence that a nationally agreed-upon list of new and emerging medicines be created that would be covered in all jurisdictions and cost-shared by the federal funds. This makes little policy sense because there is already a common list of new medicines generated every month with the agreement of all levels of government through the pan-Canadian Pharmaceutical Alliance (pCPA). The new federal funding should simply be directed at supporting the cost-sharing and expansion of faster public access to those existing, new and emerging therapies for rare disease medicines.

<u>Recommendation 2:</u> The Ontario government should develop and implement a provincial rare disease strategy.

Other countries have unique approaches to rare diseases, and it is useful to consider whether and how such initiatives could be adopted or adjusted for use in Canada. One of the key things to bear in mind is that Canada remains a serious laggard globally in this regard with few programs in place to meet the needs of Canadian with rare diseases.

In Ontario, a rare disease implementation plan steering committee was established in December 2017 to implement recommendations from a provincially appointed rare disease working group that had issued its final report in March 2017. However, that work has never been pursued. There is currently a private members' bill in the legislature urging the government to take action in this regard.

RAREi has been encouraged by the provincial government's demonstrated commitment to the life sciences sector through the development of Ontario's Life Sciences Strategy in 2022, the first in more than a decade. It is an excellent step forward for the province. It will ensure a healthier population via accelerated and improved access to health innovations and strengthen the economy. However, it is worth noting that rare disease treatments face challenges that are not directly addressed by the Ontario Life Sciences Strategy.

To illustrate some of these challenges, patients with a rare disease can wait up to 10 years to receive an accurate diagnosis, after which they may wait even longer to see the right specialist and receive the right treatment. Treatments that are being developed are specific to a small patient population and often require special expertise and infrastructure to administer. For example, currently in Ontario, only one hospital offers chimeric antigen receptor T-cell therapy for pediatric and young adult patients, and only three hospitals offer it to adults, all of which are clustered in Toronto and Ottawa leaving large gaps throughout the province.

RAREi believes Canada has a unique opportunity to create one of the best health systems in the world to test for, diagnose and treat rare diseases. In this context, it is pleased to offer a RAREi vision for optimizing rare disease care in Canada using key learning and successes from other countries.[5]

VISION 1: BETTER ACCESS TO THERAPIES

Patients need better and faster access to medicines that can save or improve their lives. For this, a special system needs to be designed for reviewing rare disease medicines, taking into account the unique characteristics of these treatments. Patients should be able to benefit from safe and effective treatments immediately following Health Canada approval, without making them wait for additional value assessments and price negotiations. In order to ensure value, and subject to considerations (e.g., feasibility, cost, etc.), RAREi members commit to generate and submit real-world evidence to demonstrate the impact of the treatments as they are being utilized by patients and to negotiate value-based agreements that reflect that impact.

VISION 2: IMPROVED CARE PATHWAYS

Current rare disease care pathways are difficult to understand and navigate, for patients and care providers alike. The system can be made much faster and easier to access by creating rare disease centres across Canada (similar to current cancer centres), to provide specialized care and treatment.

VISION 3: FOCUSED ON RESULTS FOR PATIENTS

All too often, health care systems prioritize bureaucracy and administrative processes over the well-being of patients. Canada needs to embrace value-based health care, which rewards quality (improved results for patients) rather than quantity (volume of services delivered). That is not to say the rare disease system in Canada should have a blank cheque. Rather, it means it should be structured in a way that provides maximum value to all, including both patients and their families as well as governments and other payers.

RAREi recommends the Ontario government demonstrate leadership by developing and implementing a provincial rare disease strategy to ensure that patients in need are able to access new and emerging treatments and diagnostics in a timely and equitable way. Such a strategy should be integrated and aligned with both the federal government's *National Strategy for Drugs for Rare Diseases*, and other provincial initiatives to ensure that expertise, diagnostics, and treatments are made available in a timely manner, and capacity can be built, from coast to coast. In particular, the strategy should be focussed initially on leveraging the promised federal funding to help ensure patient access to treatments, programs and services and to take advantage of the research and economic development opportunities it would generate.

[5] RAREi's vision paper, *Finding the Missing Piece: A Vision for Rare Disease Care in Canada,* offers a variety of examples of initiatives to support rare disease patients that can be found in other countries among other things is available at rarei.ca.

<u>Recommendation 3:</u> The Ontario government should invest in rare disease infrastructure as an economic driver.

Investing in rare diseases should not be viewed a cost, but rather an investment in patients, their families, and the economy.

The COVID pandemic has demonstrated the importance of shifting the way health care investments are viewed, from expenses with no benefit to value generation and investment in innovation. Patients benefit at an individual level as a result of timelier diagnosis and treatment, allowing them to be productive members of society instead of remaining ill (and in most cases utilizing many other health care resources) and sometimes living in institutional settings. The burden on informal caregivers, such as family members, caring for individuals with rare diseases is enormous. One study found that 89.2% of informal caregivers of people living with sickle cell anaemia reported that they spent 24 hours per day caring. In another case, the mean self-reported hours spent caring for people living with mucopolysaccharidosis was 51.3 hours per week.[6] Informal caregivers would benefit greatly from investments in rare disease to help ease the burden of unpaid caregiving, allowing them to reintegrate into society. The economy also benefits as a result of the creation of jobs and expertise in emerging fields of medical research, manufacturing of treatments, and care delivery.

Investing in rare disease infrastructure could mean putting Ontario on the map as a world leader in this area. These investments would attract private sector investments, further strengthening and developing the life science and rare disease ecosystem in Ontario as well as continued commitment to Ontario's Life Sciences Strategy.

To that effect, RAREi encourages the Ontario government to consider investments in rare disease infrastructure as an economic opportunity rather than simply a cost.

[6] Sandilands, K., Williams, A. & Rylands, A.J. Carer burden in rare inherited diseases: a literature review and conceptual model. *Orphanet J Rare Dis* **17**, 428 (2022). https://doi.org/10.1186/s13023-022-02561-w