Canadian Organization for Rare Disorders

Submission to 2024 FINA Pre-Budget Consultations

August 4, 2023

CORD Recommendations

1. Bring to the table the rare disease community, as the stakeholder with the most expertise on the impact of rare disease, to expedite the federal/provincial/territorial bilateral agreements to make available as soon as possible the rest of the already committed funding for the Rare Disease Drug Strategy, to start to realize optimal benefits and return on investment.
2. Ensure appropriate financial investment in the other areas relevant to the effective operation of the Rare Disease Drug Strategy. In particular, we need investment in infrastructure to ensure drugs are used appropriately by the appropriate patients to deliver optimal benefits and avoid adverse effects. This is the only pathway to ensure that the health system and society will also realize optimal outcomes and return on investment, thus assuring sustainability of the Rare Disease Drug Strategy, in perpetuity.

ABOUT CORD

The Canadian Organization for Rare Disorders (CORD) is Canada's national network for organizations representing all those with rare disorders. CORD provides a strong common voice to advocate for health policy and a healthcare system centred on the needs of patients and families living with rare disorders, that is also sustainable and beneficial to all. CORD works with governments, researchers, clinicians, and industry to promote research, diagnosis, treatment and services for all rare disorders in Canada. In 2015, CORD launched Canada's Rare Disease Strategy to provide recommendations to improve the care and treatment for rare disease patients in Canada.[1]

BACKGROUND ON CURRENT RARE DISEASE CHALLENGES

The needs and potential benefits are staggering: 3.2 million individuals in Canada live with a rare disease – more than the number of cases of diabetes, cardiovascular disease, and all cancers combined. Tragically, more than two-thirds of those affected are children, and a heartbreaking one-third of these children will not live to see their fifth birthday. Every 39 minutes, we lose a child to a rare disease in Canada. This can be characterized only as a crisis; the good news is that we have the means, tools, and expertise to take meaningful action. We also have the political commitment; now we need the political will to act.

The evidence of the burden is strong. The journey to obtain an accurate diagnosis is a grueling one, often taking five to ten years, with countless misdiagnoses and inappropriate treatments along the way. The consequences of delayed diagnosis and limited access to optimal treatment are profound, not only for the individuals affected but also for society at large.

However, the evidence of the benefits and cost-effectiveness of investing in timely diagnosis and optimal treatment are equally strong, in high-income countries with excellent universal healthcare but also in low-and-middle-income countries with emerging healthcare systems. Children with undiagnosed rare disorders account for up t0 80% of pediatric emergency room patients, while parents withdraw from employment and often experience not only loss of income but also marital disruption and mental health trauma. Additionally, we are increasingly aware of adults with late-onset rare disorders, like ALS, Huntington's, and amyloidosis, whose symptoms are often misdiagnosed and intervention delayed, resulting in worse outcomes and greater dependency on family and societal care. We should and can do much better. The federal government commitment of an additional $1.4 billion in bilateral agreements will be an important "starter fund" with immediate results … if it were only liberated for use.

RECOMMENDATIONS

1. **Bring to the table the rare disease community, as the stakeholder with the most expertise on the impact of rare disease, to expedite the federal/provincial/territorial bilateral agreements to make available as soon as possible the rest of the already committed funding for the Rare Disease Drug Strategy, to start to realize optimal benefits and return on investment.**

In March of this year, the federal government took a significant step by announcing the *National Strategy for Drugs for Rare Diseases,* with a commitment to provide up to $1.5 billion over three years, with the majority of this funding ($1.4 billion) designated for provinces and territories to help improve access to new and emerging drugs for Canadians with rare diseases.[2]

Albeit long overdue, this significant investment by the federal government in rare disease was much appreciated and heralded by the community. Nevertheless, we were concerned that the expert patient community had not been engaged in the development of an implementation plans. Subsequently, we have been dismayed that the patients are still not engaged as partners in implementing the plan, belying a commitment to transparency and accountability. To add "injury to insult" there is still no evidence of an imminent flow of allocated funding, enabling Canadians with rare disorders to timely access to life-changing treatments.

2. Ensure appropriate financial investment in the other areas relevant to the effective operation of the Rare Disease Drug Strategy. In particular, we need investment in infrastructure to ensure drugs are used appropriately by the appropriate patients to deliver optimal benefits and avoid adverse effects. This is the only pathway to ensure that the health system and society will also realize optimal outcomes and return on investment, thus assuring sustainability of the Rare Disease Drug Strategy, in perpetuity.

At the core of optimal drug access and management lies a crucial requirement—a coordinated approach that ensures that people with rare conditions have equal access to specialized knowledge, screening and diagnosis, comprehensive care and community-based resources, regardless of where they live in Canada.

In this context, the investment in the rare disease drug plan can only generate maximal returns on investment if there are corresponding enhanced investments in a comprehensive rare disease strategy as presented to the Parliament in CORD's 2015 broader strategy.

For this reason, CORD strongly recommends that the *National Strategy for Drugs for Rare Diseases* be expanded beyond treatments and funded appropriately. The other elements of the rare disease journey that matter for patients, include:

- Effective screening (including newborn screening), genetic testing and early diagnosis (to prevent disability and premature death)
- Comprehensive care and treatment support through nationally networked centres of rare disease expertise that are linked to local healthcare providers and related community services
- Patient support programs and support from rare disease patient organizations to educate patients and promote adherence
- Monitoring of outcomes through the routine collection and analysis of real-world data that, among other things, can be used to evaluate funding effectiveness

- Investing in and supporting world-class research and science, building on and leveraging both public and private innovation in Canada and around the world

Thank you for the opportunity to provide input on the 2024 federal budget. We remain available to provide clarification or further comment.

Sincerely,

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[1] https://www.raredisorders.ca/canadas-rare-disease-strategy/
[2] https://www.canada.ca/en/health-canada/news/2023/03/investments-to-support-access-to-drugs-for-rare-diseases.html