Response to Consultation on Pre-Budget

Submitted by Canadian Organization for Rare Disorders
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Executive Summary

The Canadian Organization for Rare Disorders (CORD) is a national registered charity serving as the umbrella organization for rare disease organizations, groups, and patients in Canada. Our mission is to improve the lives of all those affected by rare diseases. Our mandate: is to advance rare disease policy, improve screening, diagnose and access to clinical trials and treatment; develop patient group capacity; support research; collaborate

CORD provides a strong common voice to advocate for health policy and a healthcare system that works for those with rare disorders. CORD works with governments, researchers, clinicians and industry to promote research, diagnosis, treatment and services for all rare disorders in Canada. We are a membership based organizations comprised of over 100 rare disease organizations, individuals affected by rare diseases, healthcare providers serving rare diseases, and other stakeholders committed to improving the lives of those with rare disorders.

In response to the pre-budget consultations being held by the Finance Committee, CORD is submitting three recommendations:

1. The government of Canada should immediately implement Canada's Orphan Drug Regulatory Framework.
2. The government of Canada should take immediate steps to implement a national Rare Disease Strategy.
3. The government of Canada should assure that all Canadians have affordable, and equitable access to the prescription medicines most appropriate to their individual needs, as articulated by CORD in its submission to the consultations on the proposed amendments to the Patented Medicines Regulations.

Canada's Orphan Drug Regulatory Framework

The Canadian Organization for Rare Disorders calls for the immediate implementation of the federal Orphan Drug Regulatory Framework, announced by Health Canada in 2012 and fully developed in 2014. This framework would help incentivize the development of drugs for rare diseases and facilitate the timely approval and appropriate use of these therapies to ultimately improve health outcomes for Canada's most vulnerable patients. The ODRF has never been implemented.

Current estimates indicate that there are approximately 7,000 rare diseases worldwide and that the recognized number of such diseases rises at the rate of approximately 250 per annum. While the definition of a rare disease varies from

about 1 in 2,000 to 1 in 30,000, the common denominator is that the number of patients affected by any one disease often defies conventional drug development, hence the nomenclature of an "orphan" drug as one abandoned during the development process. Indeed, in the decade prior to the first Orphan Drug Act, there were only 10 new therapies approved for rare diseases. In 1983, the USA passed the world's first Orphan Drug Act, followed by Singapore in 1991, Japan in 1993, Australia in 1997, the European Union in 2000, and subsequently, Taiwan, South Korea, and others.

What are the consequences of the lack of orphan drug regulations in Canada? Currently, despite the tremendous advances in orphan drug development, therapies are available for only about 500 rare conditions, so most rare diseases do not have any effective therapies. Patients with progressive, debilitating, and life-threatening disorder often have to rely on clinical trials to access treatments. Unfortunately, in the absence of the federal Orphan Drug Regulatory Framework, researchers tend to not host clinical trials in Canada. Today, most Canadian patients have to travel to the United States to take part in clinical trials. Researchers in the USA receive direct and indirect incentives to discover, test, and fully develop drugs for small patient populations with unmet needs. Even when an orphan drug is first discovered in Canada by our researchers, it is often subsequently transferred to a company in the US for further development, as we are unable to attract enough venture capital in Canada. As a result, several orphan drugs never get to Canada, and when they do, they usually are available to Canadian patients several years after they are available in the USA.

But patients are not the only losers as Canada continues to decry, delay and even deny the implementation of an Orphan Drug Regulatory Framework that is almost fully developed. As noted by key economic forecasters and global policy scholars, orphan drugs represent the edge of the wedge that defines the next generation of innovative therapies, that is, precision medicines and gene-modifying therapies that will revolution treatment, cure, and prevention of all diseases, common and rare. So losers of Canada's not investing in this arena are Canadian researchers who will not benefit from new scientific knowledge and not be part of the international consortia that are discovering and testing these therapies; also losing out are clinicians, hospitals, and specialty clinics, which will be deprived of early experience with emerging therapies; there will be the negative downstream effect on technicians, students, community workers, and manufacturing facilities. Canada has now fallen behind India, which just announced a Rare Disease Strategy with its eye on investment in orphan drugs.

The decision not to implement an Orphan Drug Regulatory Framework (and it is indeed a deliberate decision) based on a fallacious premise that this will delay or prevent the entry of orphan drugs to market is as ill conceived as a notion that by not funding physicians or building hospitals, the need will go away and there is no net cost. The Canadian government needs to implement Canada's Orphan Drug Regulatory Framework and it needs to do it now.

Support for Canada's Rare Disease Strategy

While each rare disease affects only a small number of individuals, there are more than 7,000 rare diseases that together affect 1 in 12, or nearly 3 million Canadians. While 80% of rare diseases are due to a genetic mutation or defect, in 50% of cases there is no family history. About two-thirds of those with rare diseases are children; about one in three will not live beyond their fifth birthday. The number of Canadians living with any rare disease is greater than the number of Canadians with diabetes (2.4 million), greater than the number with heart disease (1.8 million), and greater than the number with all cancers combined (1.4 million). Many rare diseases are progressive, debilitating, and life threatening. A major difference is that there are national strategies or plans for the common conditions that have significantly improved detection, diagnosis, treatment, supportive care, and research. There has not been a national strategy for rare diseases…until now.

Awareness, knowledge and treatment of most of rare diseases are still limited and fragmented across the country. As a result, individuals face a host of extraordinary challenges, including:

- Misdiagnosis and/or long, difficult path to diagnosis
- No or limited treatments
- Barriers accessing available rare disease therapies
- Difficulties finding physicians or clinical centers with experience treating rare diseases
- Challenges accessing medical, social or financial services or assistance
- Feelings of isolation and of having been "orphaned" by the Canadian healthcare system.

All of these challenges lead to increased morbidity, loss of life or poorer quality of life and increased costs to the family, the healthcare system and ultimately the Canadian economy.

Realizing the benefits of a nationally coordinated approach for rare diseases, in 2009 the European Union mandated all member states to develop a National Plan for Rare Diseases by 2017. Most have fully complied and some are in their second and third plans. In 2017, the European Union took the next major step to implement 25 cross-border European Reference Networks for rare diseases.

In 2015, after a series of multi-stakeholder and cross-country consultations, the Canadian Organization for Rare Disorders launched Canada's Rare Disease, presented to the Parliament in February on Rare Disease Day. It proposes the following **five-goal strategy,** which includes recommended actions directed **at improving the lives of people with rare diseases.** While it is recognized that healthcare is provincial jurisdiction, CORD calls upon the federal government to provide the over-arching framework, strategic initiatives, and support towards implementation where appropriate.

Goal 1: Improving early detection and prevention.

1. Adopt a national approach to newborn screening.
2. Implement early detection and prevention services across Canada.

Goal 2: Providing timely, equitable, and evidence-informed care.

3. Improve education and capacity of healthcare providers related to rare diseases, including genetic counselors
4. Address gaps in social care programs for people with rare disorders
5. Develop provincial guidelines to ensure appropriate accommodation for people with rare diseases in the workplace
6. Provide people with rare diseases the same coverage for healthcare services (e.g., physiotherapy) as people with more common diseases
7. Establish Centres of Excellence on rare diseases to generate and support research and patient care, develop and implement clinical practice guidelines, develop and provide professional and patient education to general healthcare practitioners and the public, and develop and support extended diagnostic, clinical and educational services, for example, through telemedicine or satellite specialized clinics
8. Explore the creation of a national registry for all rare diseases, and support new and existing disease-specific registries
9. For diseases where specialized clinics and virtual clinical networks may not be feasible, ensure better integration of care for patients with rare diseases into existing Complex Care Clinics or medical homes
10. Adopt measures to facilitate linkages between healthcare administrative databases across the country to support health service delivery to patients with rare diseases

Goal 3: Enhancing community support

11. Rare disease-specific patient organizations, as well as CORD and the Regroupement Québécois des Maladies Orphelines, should be adequately funded to achieve their missions, which include involvement in research initiatives, knowledge translation, policy development, education, engagement and support initiatives for patients
12. Increase resources to optimize the utility of Orphanet for all

stakeholders

Goal 4: Providing sustainable access to promising therapies

13. Implement a regulatory framework for orphan drugs
14. Explore adaptive clinical trial designs for market authorization and post-market phases of therapies

15. Enhance and formalize the role of patients in the market authorization process and post-market evidence-generation and provide resources to support the participation of rare disease patient groups in this process

16. Establish a separate, more flexible health technology assessment process tailored to the specific attributes of orphan drugs

17. Provide increased support to assist rare disease patient groups in engaging in health technology assessment reviews, including in preparing patient input submissions

18. Develop a consistent funding approach to ensure timely and equitable patient access to orphan drugs

Goal 5: Promoting innovative research.

19. Provide dedicated and increased funding for rare disease research and the Centres of Excellence on rare diseases

20. Establish a new Canadian Partnership for Rare Diseases to help coordinate a national rare disease research agenda and Centres of Excellence on rare diseases, among other actions recommended throughout this strategy

Sustainable Access to Affordable Drugs

The Canadian Organization for Rare Disorders would like to reference our submission in response to Health Canada's consultation on proposed amendments to Patented Medicines Regulations. Our conclusions were:
CORD welcomes the opportunity for on-going dialogue and consultation with the Government of Canada, Health Canada, and the PMPRB. We do not feel that a focus on controlling the risk of excessive drug prices can be approached separate from assurance of patient access to optimal medicines and appropriate utilization. We also urge earlier, closer, and continuous engagement with the patient and clinical communities to assure all of this work is done with full understanding of the impact on patients and families, for their perspectives, as well engagement of patients to

participate in exploring the issues, generate alternative solutions, and implementing consensual approaches that will meet the needs of affordability, access, and appropriate use. We know these optimal pathways exist and we can learn not only from other jurisdictions but each other.

Respectfully submitted:
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