Canadian Organization for Rare Disorders

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April 26, 2021

The Right Honourable Justin Trudeau, PC, MP
Prime Minister of Canada
80 Wellington St.
Ottawa, Ontario K1A 0A2

The Honourable Patty Hajdu
Minister of Health
Brooke Claxton Building, Tunney's Pasture
Ottawa, Ontario K1A 0K9

Dear Prime Minister Trudeau and Health Minister Hajdu:

Subject: Time to Jump Start Rare Disease Drug Strategy

On behalf of the Canadian Organization for Rare Disorders (CORD), which represents over 100 rare disease patient groups and hundreds of thousands of patients and their families, I am writing to say "thank you" and to urge taking action now.

JUMP START RARE DISEASE DRUG STRATEGY!

On April 19 with the tabling of the 2021 budget, Finance Minister Freeland announced lots of new money for lots of new initiatives. For the rare disease community, there was no new money. There was just one reference, just one sentence, on just one page (p. 238) in the 739-page document, but it was enough to signal that the future is bright for rare disease patients in Canada. "To maintain momentum, the government will proceed with its announced plan to provide ongoing funding of $500 million for the program for high-cost drugs for rare diseases."

The funding commitment was made in the 2019 federal budget. This confirmation underscores and reifies the multi-stakeholder drug strategy consultations conducted by Health Canada this past February and March. CORD had already been leading the way by hosting public dialogues over the past year, bringing together international and Canadian experts to discuss global best practices and ways forward. Drawing upon this input, CORD set forth a collective vision for a Canadian Rare Drug Strategy that is undergird by a comprehensive Rare Disease Strategic Framework, with a 12-step implementation plan.

As importantly, the bright future envisioned in a Canadian Rare Drug Strategy can begin right now. We can jump start at least half of CORD's proposed 12 steps in advance of the $500 million realization. In reality, the $500 million "ongoing" funding should not be allocated as an annual expenditure but as an annual investment. We are not spending $500 million a year for rare disease drugs but investing into an optimal rare disease system that will assure the right drugs are being used effectively and cost-effectively. Moreover, CORD calls upon the pharmaceutical industry and other stakeholders to match the annual $500 million to produce a $1 billion annual investment fund.

How can we invest an annual $500 million federal plus $500 million private funding to leverage the best patient and societal outcomes? Rare disease therapies are increasingly specialized, complex, uncertain, and expensive. The goal of getting effective rare disease drugs to patients as early as possible so as to avoid or minimize symptom impact requires investment in screening, testing, and early diagnosis. To achieve

optimal drug use, we must provide excellent comprehensive care and treatment through networked centres of rare disease expertise linked to local healthcare providers and community services. Patient support programs and rare disease patient organizations are essential to educating patients, promoting adherence, monitoring outcomes, and collecting and reporting real-world data. In short, we need to deliver comprehensive coordinated rare disease care through a network of integrated rare disease specialists, allied health providers, primary care providers, and community-based resources.

In Canada, there are many world-class, state-of-the-art national research-supported comprehensive care programs that focus on a single rare disease or a cluster of conditions. Examples include but are not limited to: the Canadian Inherited Metabolic Diseases Research Network, the Canadian Fabry Disease Initiative, the Neuromuscular Disease Network for Canada, Pediatric Bone Health Clinical and Research program, Canadian Hemophilia Treatment Centres, and the Cystic Fibrosis Care Centers.

The imperative for a Rare Disease Strategy to assure the success of a Rare Disease Drug Strategy cannot be overstated. The 2021 budget rightly recognized pediatric cancers as a leading cause of death among Canadian children. in reality an estimated 12% of pediatric deaths are due to (rare) cancers and 46.6% are due to rare disease NOT including cancers. That translates to 29,000 Canadian children under the age of 15 dying as a consequence of a rare disease every year. As concerning, more than half of pediatric hospital beds are occupied by a child with a rare disease. Collectively, more children and adults are affected by rare diseases than by all cancers combined or by all cardiovascular diseases, including stroke. More than 80% experience delayed, wrong, or no diagnosis. The result is an insidious cycle of numerous consultations with doctor after doctor, predictably frequent ER admissions, referrals for inappropriate, useless, and costly treatments and prescriptions for wrong, ineffective, or sometimes harmful medications.

Unlike cancers, the burden of rare disease on the healthcare system, the impact on healthcare spending, and the widespread familial and societal costs are mostly unrecognized. Many cases of rare conditions are undocumented or undiagnosed and there is no cumulative accounting of up to 7,000 rare diseases. Nevertheless, the impact is real and it is enormous; we are just beginning to realize how much it costs NOT to prevent, diagnose, and appropriately treat rare diseases. With a burgeoning number of rare, complex, and precision therapies in the pharmaceutical pipeline, inaction is not an option. Health Canada's consultations touched upon a number of key issues that are essential to a Rare Drug Strategy, notably patient engagement, expert committee(s), managed access programs, patient registries, and use of real-world data for post-launch assessment. All of these are components of a Rare Disease Strategy and Framework, as articulated by CORD initially in 2015. Many of these already exist in the disease-specific programs previously introduced but many programs require additional resourcing to be sustainable and to support the new drugs and other technologies.

We can "jump start" the new Canadian Rare Drug Strategy by building on existing infrastructure and expanding to other disease areas, especially those which can be flagged with imminent innovative therapies.

CORD absolutely supports fair drug pricing in Canada, for all payers, including public and private drug plans and individual consumers. But if "fair pricing" compared to other countries is the goal, then the proposed reference basket of similar countries is reasonable and sufficient. What is deeply concerning is the proposed economic factors that will create significant confusion and uncertainty in the Canadian marketplace and have a profound impact on patient access to medicines, especially for those patients with severe, life-threatening conditions where alternative therapies are not available. This is truly a matter of life

and death for many patients. The federal government needs to take the time to rethink its approach and properly consult with stakeholders.

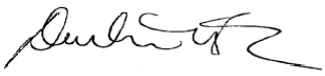
CORD RARE DRUG STRATEGY ACTION STEPS	JUMP START ACTION TO DO NOW
1. Patient empowerment: Empower patient organizations and patient advocates to participate as active full partners in all aspects of Canada's Rare Drug Strategy and Rare Disease Strategy.	1. All stakeholders participate in patient-led consultations and initiatives. Others, engage patients as full members in all planning and implementation committees.
2. Creation of a Canadian Rare Drug Agency: Establish an independent, transparent, publicly accountable agency with responsibility for all aspects of the review of drugs for rare diseases …	2. Consult multiple stakeholders through multiple processes the principles for an independent Rare Drug Agency. Distill key learnings and best practices from international and Canadian agency models
3. Create R&D incentives: Invest in Research and Development, [to] support therapeutic product accessibility, monitoring, and evaluation through clinical trials, patient registries and real-world data collection as well as best practices for usage and support.	3. Through survey and audit, develop inventory of wide variety of rare-related research projects, programs, research teams, and individuals in public, private and public-private sectors. Engage stakeholders in visioning and gaps analysis.
4. Speed up access to treatment: Ensure timely availability of new treatments … by establishing a competitive and viable environment for early drug launches, … clinical trials, early access programs, clinical site development, patient registries, and patient support programs.	4. Conduct unbiased, apolitical, data-driven analysis of availability and time-to-access of rare disease drugs in Canada compared to other relevant jurisdictions and across public and private providers within Canada. Discuss and set Canadian goals for access.
5. Address regulatory barriers: Roll back 2019 PMPRB regulatory changes to focus on mandate of ensuring list prices of drugs are not excessive while also assuring goals and objectives for new drug availability.	5. Halt PMPRB changes. Bring together all stakeholders to set target list prices that promote timely patient access, sustainable budgets, and sufficient ROI. Conduct unbiased, apolitical, data-driven analysis of PMPRB regulatory impact.
6. Improve regulatory approvals process: Ensure Health Canada regulatory processes encourage clinical trials and new drug submissions for rare disease drugs. … actively collaborate and coordinate with other jurisdictions to promote harmonization.	6. Bring together multiple stakeholders from multiple jurisdictions to share best practices and evolve new coordinated and collaborative practices for regulating innovative, complex, and emergent therapies.
7. Ensure pathways for special cases: through Special Access Program, Early Access Programs. Assure early access to urgently needed therapies by pre-NOC negotiation of access and funding.	7. Bring together all stakeholders, including patients, payers, and industry to accelerate updates to SAP and needs for EAP with respect to current and future medicines.

8. Provide multiple funding options: Multiple pathways with early differentiation based on population size, disease severity, unmet need, evidence uncertainty, potential therapeutic value, budget impact, annual unit price, and industry.	8. Conduct inventory of international and Canadian access pathways among public and private payers. Index according to multiple factors; identify benefits/risks and strengths/weaknesses. Consider relevance to current and emerging therapies.
9. Leverage Managed Access Programs (MAPs): especially for drugs receiving NOC-C with evidence uncertainty at time of approval but unmet needs and benefits outweigh risks.	9. Conduct review of MAPS across multiple jurisdictions including Canada and multiple therapies including cancer and rare diseases to identify success factors and challenges.
10.Facilitate concurrent HC and HTA reviews: Where appropriate, Health Canada data, analyses and conclusions are made available to HTA body with goal of timely, appropriate, and accepted assessment.	10. Review concurrent HC and HTA submissions for impact on process, timeliness, outcomes, and acceptance. Identify recommended applications and success factors.
11. Support real-world evidence generation: Be responsible for real-world monitoring, data collection, and evaluating the benefits, risks, and uncertainty for each drug.	11. Survey existing Real-World Evidence collection and analysis practices related to rare drug usage. Determine impact on drug access, use, and monitoring toward best practices for optimal use.
12. Enhance centres of clinical expertise: Partner in developing Networked Centres of Expertise for drug management. Treatment should be directed by expert and appropriate support team.	12. Develop annotated inventory of existing rare disease Centres of Expertise, infrastructure, training, research, and practices supporting drug use and monitoring.

These are all actions we can undertake almost immediately with minimal resourcing if there is a collective will. As the patient community, we are ready to take part and indeed willing to lead where appropriate.

Finally, on behalf of all Canadian rare disease families, our heartfelt appreciation for advancing the vision of optimal access to rare disease care and treatment.

Sincerely,



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