Canadian Organization for Rare Disorders

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Submission to the Pre-Budget Consultations on the 2022 Budget

House of Commons Standing Committee on Finance

August 6, 2021

Recommendation #1

Ensure return on investment for Canada's Rare Disease Drug Strategy by implementing Canada's Rare Disease Strategy, first released by the Canadian Organization for Rare Disorders in 2015. A comprehensive rare disease strategy is essential to assuring optimal value of innovative therapies to patients and families as well as to the health systems and society by supporting timely diagnosis and referral, appropriate prescribing and usage, and monitoring of outcomes and re-assessment.

Recommendation #2

Refocus the Patented Medicine Prices Review Board (PMPRB) because the reforms will limit research and treatment availability for Canadian patients, particularly for those with rare diseases, and conduct a review to determine the best role for the agency moving forward.

Canadian Organization for Rare Disorders

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 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.

In 2015, CORD launched Canada's Rare Disease Strategy[1] to provide recommendations to improve the care and treatment for rare disease patients in Canada.

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In February 2019, the federal budget committed $1 billion over two years starting in 2022–23 to launch Canada's Rare Disease Drug Strategy,[2] and reaffirmed in the 2021 budget,[3] up to $500 million per year ongoing to assure "fair, consistent and evidence-based access" to specialized drugs for rare disease patients. An election, new ministerial appointments and then COVID-19 sidelined federal consultations until January 2021. Now, with another election looming and unabated challenges with COVID-19, we cannot wait three more years before implementing the Rare Disease Drug Strategy.

Why can't we wait? In three years, an infant born today with Type 1 spinal muscular atrophy will lose her ability to sit, feed, and breathe on her own and possibly lose her battle for life without access to one of three available therapies. In three years, a 45-year-old man newly diagnosed with ALS will have lost out on extending his life by months and perhaps years without access to new promising therapies. In three years, a teenage boy with pigmentosa retinitis will have lost his ability to see at night, to continue martial arts, and perhaps the independence to move from home to attend university.

Unwilling to compromise the 2022 target date jeopardized by delayed federal consultations, in September 2020 and extending into July 2021, CORD hosted a series of 16 public consultations, engaging hundreds of patients, clinicians, researchers, health organizations, drug developers, and policy advisors. Not surprising, many of our participants who also responded to the Health Canada townhall, roundtable, and online consultations voiced a similar vision for a national framework and equitable, timely access to rare disease medicines.

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[1] CORD, *Canada's Rare Disease Strategy*, 2015: https://www.raredisorders.ca/canadas-rare-disease-strategy/.
[2] Government of Canada, Budget 2019: Investing in the Middle Class, March 19, 2019: https://budget.gc.ca/2019/docs/plan/budget-2019-en.pdf.
[3] Government of Canada, Budget 2021: A Recovery Plan for Jobs, Growth, and Resilience, p. 238, April 19, 2021, https://www.budget.gc.ca/2021/home-accueil-en.html

We were gratified to read in Health Canada's National Strategy for Drugs for Rare Diseases: *What We Heard from Canadians* report[4] (WWH) the key findings on all three main discussion points, which are aligned with findings of CORD in its discussions and consultations with stakeholders:

- **On drug access:** That "a single national framework for rare disease treatments would make access fairer and was the most important element for a national strategy" as long as it does not result in removing access from those who already have it.
- **On using the best evidence:** That rare-disease drugs need to be assessed differently than other drugs and for "patients and caregivers to be involved in defining what constitutes a 'benefit' or 'improvement' when treatment outcomes are assessed." It was encouraging that respondents "overwhelmingly" said innovative approaches to approval and coverage are needed such as "fast-tracking drug approval of drugs already approved in other countries, having expert panels assess evidence using both international and Canadian data, and joining international evidence-sharing networks."
- **On prices and spending:** That "many people felt the emphasis on the high-cost of drugs overlooked their value for patients, the health system and society as a whole" but that "there is a need for more transparency in how drug prices are set and suggested governments should work together to lower prices."

However, what was also said but not so clearly "heard" was the fundamental principle that assessment of "value" to patients, families, and society must drive the assessment of "willingness to pay" (value to the payer) and not vice versa, that is, predefined budgets should not limit what should be made available. This is reflected in the continued "muddling" of managed access approaches; it is important to de-couple access defined by benefits to patients and access limited by financing. Patient-centric "early access schemes" and "patient outcomes-based agreements" are developed to accommodate best available and evolving evidence while "pay for performance" and "risk-sharing schemes" are fundamentally payer-centric approaches. The challenge for the national Rare Disease Strategy will be to re-imagine assessment models that clearly delineate the goals of each stakeholder, including the manufacturer's need for incentives to innovate, but also balance these diverse but not disparate concerns

Moreover, the WWH report clearly reflects broad agreement among stakeholders that it is not just about funding but recognizes that "access to effective treatment is a complex and multi -faceted challenge, requiring creative solutions, coordination, and collaboration."

The report also reflected stakeholder call for a "strategy [that] improves patient outcomes, builds on public programs and existing infrastructure, develops better system coordination and alignment, and involves international collaboration. Consistent with these ends was CORD's input to the consultation, laying out twelve recommendations that draw on our 2015 national Rare Disease Strategy, importantly now as an adjunct to implementation of Canada's Rare Disease Drug Strategy.

To assure optimal return on investment of a Rare Disease Drug Strategy, there has to be concurrent development of the key areas that will ensure optimal prescribing, monitoring, and on-going assessment on patient (and societal) outcomes. We have previously defined these as:

- Effective screening, testing and early diagnosis

[4] Health Canada, *Building a National Strategy for Drugs for Rare Diseases: What We Heard from Canadians*, July 26, 2021, https://www.canada.ca/en/health-canada/programs/consultation-national-strategy-high-cost-drugs-rare-diseases-online-engagement/what-we-heard.html

- Comprehensive care and treatment through networked centres of rare disease expertise linked to local healthcare providers and community service
- Patient support programs and support from rare disease patient organizations to educate patients and promote adherence
- Monitoring of outcomes to collect and report real-world data to evaluate funding effectiveness.

In order to achieve the outcomes of a comprehensive strategy, several important structural changes are required:

Establish a Collaborative Canadian Network for Rare Diseases: We have a tremendous opportunity to take advantage of a global initiative under the auspices of the World Health Organization and Rare Diseases International to create the WHO-RDI Collaborative Global Network for Rare Diseases (CGN4RD).[5] CORD, as one of the advisors to this network, proposes using the framework to set up Collaborative Canadian Network for Rare Diseases (CCN4RD), which would also serve as a national "hub" for the global network. We have begun to identify interested foundational partners, such as the Maternal Youth Children Research Network (MYCRN) and the Canadian Inherited Metabolic Diseases Research Network, 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.

Continue modernization of the regulatory process: Core to timely and appropriate access to clinical trials and approved therapies is the continued modernization of the regulatory process to align with the challenges and opportunities presented by that broad category known as "advanced therapeutic products" (ATPs) which include cell and gene modification therapies and tested with a variety of innovative clinical trial designs. CORD has previously responded to Health Canada's Clinical Trials Modernization Initiative in support of an "agile life cycle approach" as well as single authorizations for multiple products. We support Health Canada's further collaborations on regulatory reviews, including the ACCESS Consortium with Australia, United Kingdom, Singapore, and Switzerland and the ORBIS Project which current encompasses some rare and ultra-rare cancer therapies.

CORD urges Health Canada to resist officially defining "rare disease" and "rare disease drugs" as part of a formal regulatory framework. This would be a step backwards. There is no universal definition; the definitions embedded in the USA and European Union regulations differ but have the common objective of incentivizing drug development for neglected and small patient populations and are becoming increasingly challenged (and irrelevant) as drug developments shifts toward ultra-rare conditions, targeted (precision) therapies for genomic-specific subgroups (within rare and common diseases, and genomic-based therapies that treat multiple conditions. Moreover, a "rare disease" designation has little direct impact on access or reimbursement policies across countries or insurance programs.

Pivot from health technology assessment (HTA) to patient outcomes-based assessment: Currently, HTA recommendations from CADTH and INESSS focus primarily on price reductions, provide limited guidance on appropriate access, and are unsuited to the cornerstone of the proposed national Rare Disease Drug Strategy, namely, managed access for drugs with "evolving" evidence requiring on-going monitoring and reassessment. We urge the federal government to take leadership in working with CADTH, INESSS and the public and private payers to set up appropriate patient-outcomes value-based assessment programs with tools for real-world data collection and re-assessments in real-time.

[5] Towards a WHO Collaborative Global Network for Rare Diseases: Needs Assessment and Concept development. RDI (April 2020). https://www.rarediseasesinternational.org/wp-content/uploads/2020/05/4.-WHOCGN4RD-19.05.2020-v3.pdf

Set up risk-sharing reimbursement programs: A major obstacle to timely access to urgently needed rare disease therapies through the public drug plans is the time used for price negotiations. Negotiations can take between one to two years, and some are never concluded. As noted in the WWH report, the federal government should take the lead to establish reimbursement process that is "fit for purpose" urgently needed rare disease treatments that makes them available to appropriate patients immediately after Health Canada approval, under a managed access process that defines criteria for access, outcomes for monitoring, and pricing to be negotiated as evidence evolves. These MAPs could be facilitated by risk-sharing agreements with the manufacturer.

"Own" innovation in rare disease research: The federal government should invest in creating and coordinating the infrastructure and providing incentives to stimulate research in developing and making available innovative treatments for rare disease. Canada needs a forward-looking vision that is focused on deriving "return on research investment." The federal government should leverage the initiatives among the granting councils, including the Canadian Institutes of Health Research and Genome Canada and foster public-private collaborations among academic health institutions and private funders, including those providing pharmaceutical, medical devices, digital, and artificial intelligence (AI) solutions. Canada has the expertise, capacity, and creativity to be a major R&D player to contribute significantly to the rare disease innovation.

Determine right pricing for rare disease therapies: The right price for a rare disease therapy cannot be set by a single stakeholder (researcher, manufacturer, or payer) nor can it be based on the input costs (R&D, production, clinical education and patient support, return to investors, and future product innovation) and similarly not on purchaser "willingness to pay" which may factor in alternative treatments, healthcare and other cost offsets, opportunity costs for other healthcare uses, implementation costs, and available finances. The optimal strategy for a small country like Canada which accounts for about 3% of the global drug market may be to collaborate with international partners to negotiate and maybe even purchase collaboratively. This consortium approach is already being used by some European countries (BeNeLuxA Initiative, the Nordic Pharmaceuticals Forum and the central European Sofia Declaration. Even if we do not act in concert on pricing and purchasing, the sharing of information would be help assure greater equity and transparency in pricing for Canada and the world.

Recommendation #2

Refocus the Patented Medicine Prices Review Board (PMPRB) back to its original mandate primarily to incentivize pharmaceutical investment in research and development and secondarily to assure non-excessive pricing of medicines. Align the PMPRB's regulations and guidelines with a robust pharmaceutical strategy that encourages investment in innovation in Canada.

CORD supports without reservation fair drug pricing in Canada, for all payers, including public and private drug plans and individual consumers. CORD has expressed its concerns with the substantial price restrictions imposed by the regulatory changes and the lack of justification, reasonableness, and consultation in the guidelines. We have previously submitted on the impact to the rare disease community in delayed and even forestalled clinical trials and timely launch of new medicines.

Particularly in the realm of treatments for rare diseases which will increasingly come under the jurisdiction of public payers due to the federal government's new drug funding commitment, effective price controls can be far better achieved through price negotiations with pharmaceutical companies for product listing agreements, conducted through the pan-Canadian Pharmaceutical Alliance. The heavy hand of price regulation by the PMPRB is not needed, particularly at the expense of making Canada a far less desirable location for international biopharmaceutical research, investment and treatments.

In the wake of the COVID-19 pandemic, we have learned the vital importance of having a strong, thriving and multi-faceted domestic pharmaceutical industry capable of meeting a broad range of Canadians' needs. The proposed PMPRB changes threaten the tremendous life sciences ecosystem that already exists and would greatly inhibit any future growth and development at a time when we need more, not less, activity in this crucial area. This is particularly true for rare diseases which affect one out of every 12 Canadians, two-thirds of them children.

Further, the PMPRB has not acted in good faith towards its stakeholders in proposing and consulting on these changes over the past three years, as was demonstrated clearly by the recent release of the agency's communications plan earlier in 2021 to smear the valid opinions and inputs of many of those, including CORD, with whom it was supposedly consulting.[6]

CORD strongly urges the government to withdraw the current proposed changes and initiate a formal review of the PMPRB that will refocus its role in incentivizing pharmaceutical investment in research and development and assuring non-excessive drug prices for the benefit of Canadian patients.

CONCLUSION

CORD is very appreciative of and excited about the coming into effect in a few months of the new federal funding for drugs for rare diseases.

This is a pivotal moment for the rare disease community in Canada and an important opportunity for governments, our health systems, industry, academia, health professionals and patients to work

[6] Patented Medicine Prices Review Board, Modern and Proactive Communications Strategy, Feb. 9, 2021, https://www.dropbox.com/s/eusxuabcq26uqt9/PMPRB%20ATIP%20Disclosure.pdf?dl=0

together to launch an exciting and promising new reality for Canadians with rare diseases at a time of unprecedented scientific progress and new hope.

We look forward to ensuring we all make the most of this pivotal event for the benefit of Canada and Canadians.