Canadian Organization for Rare Disorders

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Submission to FINA's 2025 Federal Pre-Budget Consultations

August 2, 2024

CORD Recommendations

1. Finalize the funding for the *National Strategy for Drugs for Rare Diseases* to include all provinces and territories and expand the plan to consider the broader aspects and issues related to rare disorders, so that Canada finally has an official *National Rare Disease Strategy*.

2. Ensure that the federal government's national pharmacare legislation, Bill C-64, prioritizes and supports enhanced access to medicines for Canadians with rare diseases.

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* to provide recommendations to improve the care and treatment for rare disease patients in Canada.[1]

Recommendation 1: Finalize the funding for the *National Strategy for Drugs for Rare Diseases* to include all provinces and territories and expand the plan to consider the broader aspects and issues related to rare disorders, so that Canada finally has an official *National Rare Disease Strategy*.

Rare disease is a significant public health issue which directly impacts over 3 million Canadians (1 in 12 Canadians). Each rare disease, by definition, affects a very a small number of citizens.

Most rare diseases have severe, debilitating, or life-threatening consequences. While most affect children, there are a significant number of adult-onset rare conditions being diagnosed. Among the 7,000 known rare diseases, only 5% have an effective drug therapy; it is frustrating and unconscionable that only 60% are approved here in Canada, due, in part, to Canada's long, slow, multi-step, highly uncertain coverage process.[2]

Moreover, only about 25% of rare disease therapies approved by Health Canada and recommended by Canada's Drug Agency (CDA) and/or Institut national d'excellence en santé et services sociaux (INESSS) ever get to patients through the public drug plans.[3] Rare disease patients with no access to effective therapy undergo suboptimal surgeries or rely on off-label drugs. Many experience avoidable decline in functionality, forcing them to drop out of school or leave their jobs; others suffer preventable life-threatening or life-ending events leading to disability and early death.

The development of Canada's Strategy for Drugs for Rare Diseases (DRDs) has been marked by significant milestones and ongoing challenges. Approximately 15 years ago, the federal government entered into its first funding agreement with the provinces and territories. This agreement aimed to provide universal access to two groundbreaking and life-changing rare disease drugs for Fabry's Disease. As part of this risk-sharing arrangement, a 10-year patient management program was established. This program included data collection to evaluate the drugs' effectiveness annually and allowed for adjustments to the treatment protocol based on real-world evidence. Initially, this "pilot project" was envisioned as a potential model for a broader Federal/Provincial/Territorial (F/P/T) Rare Disease Drug Program. Over time, the Canadian Fabry Disease Initiative (CFDI) demonstrated not only the efficacy of the drug treatments but also their cost-effectiveness.

Despite numerous discussions and working groups in the years following the CFDI, tangible progress was slow. It wasn't until the federal announcement in 2019 of a $1 billion investment in the *National Strategy for Drugs for Rare Diseases* (DRDs)[4] that significant momentum was generated. During the COVID-19 pandemic, CORD hosted multistakeholder consultations to keep the needs of patients at the

forefront of the strategy. By March 2023, the federal government officially launched the Strategy for DRDs, with a commitment of $1.4 billion to be distributed through bilateral agreements. This funding was intended to make "new and emerging therapies" as well as existing treatments available "as early as possible" to improve the quality of life for patients.[5]

However, it took nearly 18 months before the first bi-lateral agreement was signed to get funds released to support access to rare disease medicines. On July 23, 2024, the federal government announced its agreement with British Columbia,[6] with notice that two rare disease medicines were being reimbursed by the province with the new funding. The speed at which negotiations with jurisdictions have been going is both unacceptable and disheartening, especially when we consider that other developed countries have had rare disease policies and strategies in place for many decades.

Finally, while CORD is encouraged with the opportunity for increased financing for rare disease treatments, there are several other critical elements of a national rare disease strategy that need to be incorporated or – even better – officially endorsed.

In 2015, CORD worked with stakeholders throughout the rare disease community to launch Canada's Rare Disease Strategy with the following five key elements:[7]

1. Improving early detection and prevention,
2. Providing timely, equitable and evidence-informed care,
3. Enhancing community support,
4. Providing sustainable access to promising therapies and
5. Promoting innovative research

An effective drug strategy needs to be grounded in and support rare disease patients in other areas as well, so that investments in medicines are fully aligned with the broader rare ecosystem, including diagnosis, care and community support and research.

In the absence of government leadership on the broader aspects of rare diseases, CORD has worked tirelessly to help stand up and create the Canadian Rare Disease Network (CRDN), a pan-Canadian network that unites the country's leading clinical, scientific, and patient expertise to improve the lives of the millions of children and adults affected by a rare disease across Canada.[8] By bringing together all partners and mobilising the rare disease community, the CRDN aims to accelerate innovative scientific and medical advances and improve standards of care across Canada for patients and their families.

In sum, the federal government needs to finalize further funding arrangements with all provinces and territories to expand enhanced access to medicines for rare diseases and – just as importantly – expand the scope of the national strategy for drugs for rare diseases by endorsing, adopting and supporting the broad community efforts of CORD, the CRDN and other partners across Canada.

Recommendation 2: Ensure that the federal government's national pharmacare legislation, Bill C-64, prioritizes and supports enhanced access to medicines for Canadians with rare diseases.

CORD has been closely monitoring and contributing to the parliamentary review that is currently underway on Bill C-64, the national pharmacare legislation. We have provided extensive written input to the House of Commons Health Committee and appeared as a witness, however, we were very disappointed with respect to the rushed, perfunctory and cursory nature of the House of Commons review on the bill. This is particularly concerning for CORD, given that provinces and territories are mixing up the national pharmacare legislation and related funding and the rare disease funding, with no clarity on whether or not the federal government is going to be a long-term partner on either of these new federal initiatives.

There are interesting contrasts that also deserve highlighting, between the pharmacare approach and the federal government's DRD plan. Bill C-64 focuses narrowly on the "appropriate use of pharmaceutical products" to prioritize patient safety, optimize health outcomes, and reinforce health system sustainability. In contrast, the Strategy for DRDs is more broadly aimed at improving patients' quality of life through enhanced access to both new and emerging drugs and investments in rare disease infrastructure to support better access to existing treatments, early diagnosis, and screening.

The overall approach to the Strategy for DRDs is therefore, technically, more holistic and potentially suited for rare disease patients. For instance, the Strategy for DRDs includes critical elements that are not present in Bill C-64, offering some hope for a different approach to implementation. Notably, the Strategy for DRDs states that its funding will help patients with rare diseases, including children, access treatments as early as possible to improve their quality of life. Furthermore, the Strategy for DRDs emphasizes that a critical next step is the development of bilateral agreements, where the federal government will collaborate with provinces and territories to determine a small set of new and emerging drugs that would be cost-shared and consistently covered nationwide for patient benefit.

The process for including new and innovative therapies and ensuring patient representation is unclear. Bill C-64 proposes universal, single-payer coverage for specific drug categories and appoints an Expert Committee to propose operational and financing options for national pharmacare. The Strategy for DRDs, however, focuses on jointly determining a limited set of new and emerging drugs with provinces and territories. CORD believes that prioritizing the availability of cutting-edge therapies is crucial, rather than being sidetracked by the establishment of a single-payer financing model, which is what the Strategy for DRDs is currently proposing.

While Bill C-64 outlines specific timelines for developing an essential medicines list, a national bulk purchasing strategy, and an appropriate use of drugs strategy, the Strategy for DRDs does not specify a timeframe for implementation. The only actions taken so far have been related to research grants, registries, newborn screening, and the formation of an Implementation Advisory Group (IAG). CORD advocates for clear action steps with publicly accountable timelines for both programs, starting with the initial implementation phase.

Both Bill C-64 and the Strategy for DRDs propose starting implementation with a limited set of drugs. However, the Strategy for DRDs should be focused on new and emerging drugs and not being tied to specific essential medicines, offers a more flexible and potentially advantageous path. CORD urges

both pharmacare and the Strategy for DRDs to aim high and ensure that implementation procedures provide optimal access to individualized therapies that offer the greatest value to patients and society, rather than settling for minimal standards of care.

Canada's late entry into the universal access discussion has resulted in a complex landscape of private and public drug insurance. Despite this, Canada has not yet demonstrated leadership in becoming a top destination for pharmaceutical research and access to cutting-edge therapies. The focus should be on bringing the best therapies with the greatest impact on patients' lives, rather than merely addressing financing mechanisms.

Finally, CORD cannot emphasize enough the importance of having patients and the rare disease community more involved in the development and implementation of Bill C-64.

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[1] https://www.raredisorders.ca/canadas-rare-disease-strategy/
[2] https://www.raredisorders.ca/our-work/
[3] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC8895096/
[4] https://www.canada.ca/en/health-canada/news/2023/03/investments-to-support-access-to-drugs-for-rare-diseases.html
[5] Ibid.
[6] https://www.canada.ca/en/health-canada/news/2024/07/backgrounder-drugs-for-rare-diseases--british-columbia-agreement.html
[7] https://www.raredisorders.ca/canadas-rare-disease-strategy/
[8] https://canadianrdn.ca