Canadian Organization for Rare Disorders

CORD's Submission to the
House of Commons Standing Committee of Health
regarding their study on Bill C-64, *An Act Respecting Pharmacare*

Rare Disease in Bill C-64: Similarities, Deviations, and Learnings

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The Canadian Organization for Rare Disorders (CORD) welcomes the opportunity to provide feedback on Bill C-64, drawing especially on our experience with the implementation of the Canadian Rare Disease Drug Strategy (RDDS), specifically the progress and challenges to date.

To put our comments into context, it is important to note that 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.

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. 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.

Journey to Canada's Rare Disease Drug Strategy

About 15 years ago, the federal government signed the first (and to date only) funding agreement with the provinces and territories to provide universal access to two transformational life-altering rare disease drugs. The risk-sharing agreement mandated a 10-year patient management program with data collection to report annually on the effectiveness of the drugs and to update the treatment protocol based on real-world evidence. At the time, this "pilot project" was considered as a potential framework for a Federal/Provincial/Territorial (F/P/T) Rare Disease Drug Program. In the ensuing years,

the Canadian Fabry Disease Initiative (CFDI) demonstrated that drug treatment was not only effective but cost-effective.

Since the CDFI, there have been many working groups and discussions, but nothing materialized until the 2019 federal announcement of $1 billion for a Canadian Rare Disease Drug Strategy (RDDS). CORD hosted multistakeholder consultations even during COVID to maintain pressure and ensure the needs of patients were central to the strategy.

Finally, in March 2023, the federal government officially launched the RDDS, referencing $1.4 billion to be allocated through bilateral agreements to make "new and emerging therapies" as well as existing therapy available "as early as possible, for better quality of life."

However, it's now been well over a year since the announcement, and it is unacceptable that *not a single penny has been spent to fund a single rare disease drug for a single patient*.

Given the clear and strong linkage in the Preamble of Bill C-64 to the RDDS as well as the explicit and unique reference within the commitment to "long-term funding for the provinces, territories and Indigenous peoples to improve the accessibility and affordability of pharmaceutical products, beginning with those for rare diseases", it is imperative to hear directly from the rare disease community on implementation of national pharmacare.

On behalf of the rare disease community, CORD expresses dismay and disbelief over the federal government's introduction of a national pharmacare bill without having made any visible progress on the implementation of the RDDS.

The RDDS was launched well over a year ago, and in the government's own words, to provide access to "new and emerging" therapies including "cutting-edge treatment options" that might "dramatically improve the quality of a patient's life" and should be accessible "as early as possible." Wow! How has that urgency and commitment to patients been manifest over this past year? It is unconscionable and unethical to introduce a program designed to transform and save lives and then fail to execute on it. Moreover, it does not portend well for the realization of national pharmacare.

Learnings from Bill C-64 and the RDDS

There are similarities but also key differences between Bill C-64 and the RDDS. In contrast to Bill C-64 which emphasizes narrowly the "appropriate use of pharmaceutical products" that "prioritizes patient safety, optimizes health outcomes, and reinforces health system sustainability", the RDDS is more comprehensively positioned toward the quality of life of the patient, to be achieved through improved "access to new and emerging drugs" as well as investment in rare disease infrastructure to "support enhanced access to existing drugs, early diagnosis, and screening for rare diseases."

Moreover, there is critical additional text in the RDDS that is not in Bill-64, giving us some hope of a different path to implementation. As noted previously: "[The RDDS

funding] will help patients with rare diseases, including children, have access to treatments as early as possible, for better quality of life."

Finally, in terms of implementation, the RDDS posits as a critical next step to the development of bilateral agreements, "the Government of Canada will engage with provinces and territories to jointly determine a small set of new and emerging drugs that would be cost-shared and covered in a consistent way across the country, for the benefit of patients."

Frankly, an overriding concern for CORD is that the RDDS does not have an articulated implementation plan nor a timeline for action and it would be retrogressive and indeed disastrous for rare disease patients if the implementation steps for Bill C-64 were applied to RDDS.

The following are some of our concerns and recommendations.

1. ***Pre-determined categories and lists of medicines:*** Bill C-64 has been introduced with two predetermined treatment categories and a list of therapies with no public consultation and no clear criteria as to why they were chosen. Moreover, these do not reflect the opportunity for individualized optimal treatment that would support best outcomes and quality of life. Based on the minister's announcement, CORD expects the RDDS to be implemented with an open, evolving list based on urgency, unmet need, and patient/clinician input with funding not only for drugs but also for screening, diagnosis, drug monitoring, and disease management. CORD feels that the goal of a national pharmacare program should be to deliver therapies with the best value for the individual patient, which requires support for precise diagnosis and disease management.
2. ***How will new and innovative therapies be included, and will patients have a voice?*** Bill C-64 calls for public pharmacare coverage through "universal, single-payer, first dollar" coverage of contraceptive and diabetes drugs. Moreover, Bill C-64 directs the appointment of an Expert Committee to propose operational and financing options for "national, universal, single-payer pharmacare." In contrast, the RDDS announcement directs the federal government with the provinces and territories "to jointly determine a small set of new and emerging drugs that would be cost-shared and covered in a consistent way across the country, for the benefit of patients." CORD feels that the best value for a national drug strategy, for rare and non-rare therapies, is focusing on how to make available new and cutting-edge therapies rather than being side-tracked on how to set up a single-payer financing model.
3. ***At least pharmacare gets timelines:*** The proposal for Bill C-64 lays out timelines for key components, including a one-year deadline to develop (1) an essential medicines list toward a national formulary; (2) a national bulk purchasing strategy; and (3) an appropriate use of drugs strategy. The RDDS does not lay out a timeframe for implementation so the only elements of the announcement which have been acted on are the CIHR research grants, the CDA's registries and

newborn screening initiatives, and the convening of an Implementation Advisory Group (IAG) organized by Health Canada's Directorate on Rare Diseases and Pharmacare. CORD calls for action steps with timelines that are publicly accountable with opportunities for input and feedback for both programs, starting with the initial implementation phase.

4. ***Pro forma "expert committees" with no genuine advisory roles and responsibilities:*** Bill C-64 calls for an Expert Committee to advise on the implementation of national pharmacare. CORD offers its learnings from experience on the RDDS IAG over the first year. The meetings have primarily consisted of IAG members receiving information, most of which we already knew and provided absolutely no insights on the bilateral agreements. We asked but received no feedback on *who, what, when, and where* discussions were taking place and any progress on the contents of potential agreements. Moreover, we are not asked to provide any input (advice) on the bilateral agreements, even as to the criteria by which the initial therapies for funding would be selected, the way in which funds would be allocated, and importantly, investments to assure screening, diagnosis, and optimal use. Despite the initial promise of public webinars, the IAG has provided no "official" updates and no consultations with the public. The lack of transparency, communication, and accountability create an environment of distress, distrust, and discontent with the RDDS (notwithstanding the commitment of $1.5 billion) and undercut the promise of delivering effective therapies to improve the lives of persons living with rare diseases. CORD calls for Pharmacare and RDDS advisory committees to be provided with the information needed to provide genuine advice rather than acting as a sounding board or rubber stamp to decisions that are being made behind closed doors.

5. ***Lowest-common denominator solutions do not work:*** Like Bill C-64, the RDDS proposes to initiate implementation with a small set of drugs. However, there are several advantages that we can identify with the RDDS (as announced though, not acted on) relative to Bill C-64. Unlike Bill C-64, the RDDS drugs were not defined in advance but are to be determined jointly with provinces and territories. Another important point of differentiation is the RDDS reference to "new and emerging drugs" and "enhanced access to existing drugs" rather than the "essential medicines" that seemingly define national pharmacare. Finally, we note that RDDS therapies "would be cost-shared and covered in a consistent way across the country" but not necessarily tied to Bill C-64's specification of "universal, single-payer, first-dollar coverage" in reference to the first two categories of drugs and the mandate to the Committee of Experts. CORD calls for National Pharmacare and the RDDS to aim HIGH and set out implementation procedures that would provide optimal access to individualized therapies with greatest value to the patient and society and NOT seek the "lowest common denominator" of population-based "essential medicines" that meet "minimal" standards of care. Pharmacare and the RDDS should assure the delivery of

innovative, state-of-the-art, cutting-edge therapies that treat not only symptoms but the underlying causes of disease, that not only slow disease progression but can prevent or reverse symptoms, and that not only prolong quality life but can ostensibly "cure" a disease.

6. ***Become a leader aim to be the best:*** Canada is entering the discussion on universal access to prescription therapeutics somewhat later than many other countries. On the one hand, this has forced/allowed us to develop a private drug insurance sector complemented by public drug plans at provincial and territorial levels. On the other hand, the Canadian government has shown no leadership to make Canada a leading country that is a good place for investing in pharmaceutical research, clinical trials, and access to leading-edge pharmaceutical products that will deliver the optimal returns for patients and society. It makes little difference who and how drugs are financed if Canada is not committed to bringing in the best therapies with the greatest impact on patients' lives.

About CORD

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.