RAREi
THE CANADIAN FORUM FOR RARE DISEASE INNOVATORS

Submission to House of Commons Standing Committee on Health (HESA)

# Prioritizing Rare Disease Patients

*HESA's review Bill C-64 (An Act Respecting Pharmacare)*

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## 1. Introduction

On behalf of the Canadian Forum for Rare Disease Innovators (RAREi), we appreciate the opportunity to share the members' perspective on *Bill C-64 (An Act Respecting Pharmacare)* with this committee. Despite the time limits imposed on the committee to review the legislation, we look forward to your thoughtful deliberations, and hope that our input helps drive and inform a more substantive parliamentary review and debate on this important legislation.

While Bill C-64 is directed at providing "universal, single-payer, first-dollar coverage" for certain contraceptives and diabetes treatments, it has been characterized by the government as a stepping stone to a more comprehensive national pharmacare plan that is intended to evolve over time based on this initial model. The bill would also commit the government to "maintaining 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***" [emphasis added]. If enacted the legislation could have a profound impact on how medicines for rare diseases are funded and accessed by Canadians.

## 2. Addressing the Question of Coverage

Foundationally, RAREi believes that all Canadians should have access to the medicines they need without any undue financial hardship. In particular, RAREi endorses federal efforts to enhance and improve timely and comprehensive public coverage to people with no existing health benefits, including medication coverage or coverage that imposes high out-of-pocket costs on them.

At the same time, the large majority of Canadians already have access to comprehensive, private health benefits coverage along with supplemental "catastrophic" coverage for publicly listed medicines in cases where out-of-pocket costs exceed a set proportion of their household income. While there is no doubt that there are limitations within the current system – including the restrictive and slow public reimbursement timelines for new medicines – Canadians' pharmaceutical needs are generally well-served

by it . As such, RAREi believes strongly that the focus of the government's efforts needs to be on addressing the system limitations rather than upending what is working rather well for most.

A national poll by Leger indicates that universal, single-payer national pharmacare is not a top health care reform priority for most Canadians. Only 22% of respondents said the new pharmacare plan should replace the coverage they receive from employers. Additionally, 44% supported a plan that would offer coverage only to people without existing coverage, seniors and people who make less than $90,000 per year. Only 17% of respondents said a new medication coverage plan should be a priority for the government.

A similar point-of-view was offered recently by former Ontario Health Minister George Smitherman, who characterized the bill as "poor public policy considering the current state of health care in Canada." He stated that the main problem is that pursuing a narrowly applied single-payer model is that it risks undermining confidence by disrupting existing coverage for diabetes medications and contraceptives and may also exacerbate the administrative burdens faced by doctors while failing to address existing coverage gaps for all other patient groups. He recalled the disruption created when the Ontario government introduced the OHIP+ program, which offered public coverage for medicines prescribed to Ontario residents younger than 25. The main problem was that the public plan offered less coverage than most were already receiving from their private plans. He said that "absent of resources to initiate a comprehensive single-payer pharmacare model it's not prudent to elevate the promise of one, recommending instead that the government focus on addressing gaps in coverage, not creating a new government-run program that will cost billions to mostly cover people who already are well-served.[1]

The reality is that the vast majority of Canadians have access to some form of medication coverage that could help meet their needs. A recent Conference Board of Canada (CBOC) analysis determined that fewer than 3% of Canadian residents have no access to reimbursement for their medicines.[2] Two-thirds of Canadians have coverage through private health benefit plans, which consistently have been shown to offer a broader range of medicines and faster access to new treatments than public pharmacare plans.[3] Half of those beneficiaries are eligible for public medication benefits as well, as a backstop for any limitations in their private coverage. Another 13 million are covered primarily by public plans, which, broadly speaking, offer coverage for most of the medicines that most Canadians need.

As the CBOC report points out, there are a small number of Canadians with no access and there are approximately 10% of Canadians who are eligible for medication coverage through a public or private plan, but who are not registered. In addition, there are real issues with the existing medication coverage that most Canadians enjoy, such as prescribed medications that are not listed as covered benefits, lengthy delays in the medication review and approval processes that leave some approved new medicines unreimbursed and, most importantly, out-of-pocket costs that can include premiums, annual fees, deductibles, and / or co-payments – all of which can add up quickly and represent a significant barrier to access. It must be stressed, however, that those access barriers exist for beneficiaries of both public and

[1] Smitherman G, *National pharmacare plan is poor public policy,* Toronto Star, April 12, 2024: https://www.thestar.com/opinion/contributors/smitherman-national-pharmacare-plan-is-poor-public-policy/article_7cf3484e-f766-11ee-9e66-834c82974f92.html.

[2] Conference Board of Canada, *Understanding the Gap 2.0, A Pan-Canadian Analysis of Prescription Drug Insurance Coverage,* July 13, 2022: https://www.conferenceboard.ca/wp-content/uploads/2022/10/understanding-the-gap-2.0_2022.pdf.

[3] Skinner B*, Public v private insurance coverage for new drugs in Canada, 2018-2023, 7th Edition*, Canadian Health Policy, April 22, 2024: https://www.canadianhealthpolicy.com/product/public-v-private-insurance-coverage-for-new-drugs-in-canada-2018-2023-7th-edition.

private plans. While the specifics may differ depending on the plan, many covered patients face such challenges every day regardless of which provider they rely on for coverage.

In that context, it is important to note that rare disease patients tend to be much more affected by the plan design-related coverage barriers addressed above than the majority of Canadians. This is because products developed for small patient populations face more significant hurdles than regular medicines in terms of securing timely coverage. They tend to come with higher per-patient prices given that investments in research, development and commercialization need to be covered by a much smaller pool of patients than for more common medicines. Rare disease treatments also often require additional support to ensure that they are being taken properly. As a result, they are rejected for coverage by payers more often, and even when they are covered, they are reimbursed based on highly restrictive criteria. In those latter instances, the patient cost sharing portion is often out-of-reach for patients and their families. These persistent orphan treatment challenges exist in both the private and public plan environments and represent an important rationale underlying the rare disease communities ongoing demand for comprehensive, national and provincial rare disease strategies in Canada.

In any case, RAREi members believe that the key to meeting Canadians' medication coverage needs is to direct our collective energies at ensuring that all Canadians have access to third-party coverage, the limitations that restrict access to available treatment options are addressed effectively and out-of-pocket costs imposed on patients and their families are reduced. RAREi asks the committee to endorse its recommendations and that it return the draft bill to the Commons with a request that the bill be revised and considered, accordingly.

## 3. A Word About the National Drugs for Rare Diseases Strategy

When the federal government launched its long-promised national drugs for rare diseases (DRD) strategy in March 2023, the entire rare disease community was thrilled and relieved that this neglected area of health care would start to receive some much-needed attention and resources.[4]

It was particularly notable that at least $1.4 billion of the initial three-year $1.5 billion pledge was to be dedicated to expanding patient access to existing and new rare disease treatments, as well as enhanced screening and diagnostics. It must be stressed too, that federal DRD strategy was identified as a core component of the government's national pharmacare vision.

That $1.4 billion carve out for the provinces and territories (PTs) was to be distributed through bilateral transfer funding deals with each PT, with a requirement that a small sub-set of emerging rare disease medicines be funded through the new system. Unfortunately, since the strategy was announced more than a year ago (**and more than five years after the initial Budget 2019 pledge to direct substantial dedicated resources to the rare disease community**), the initial list of medicines to be covered nationally has not been developed, none of the bilateral agreements have been completed and not one cent of the funding directed at improving patient access to treatments has been spent – and there is no public indication that the logjam will be broken anytime soon.

[4] Canadian Organization for Rare Disorders, *Canadian Organization for Rare Disorders welcomes federal funding for rare drug plan to accelerate access to diagnostics and treatments,* March 22, 2023: https://www.raredisorders.ca/canadian-organization-for-rare-disorders-welcomes-federal-funding-for-rare-drug-plan-to-accelerate-access-to-diagnostics-and-treatments.

In light of the already lengthy delays in implementing the national DRD strategy and getting funding for treatment access to flow to provinces and territories in order to help them improve access to orphan medicines, RAREi requests that every effort be made by all involved to finalize the steps necessary to get the money moving as soon as possible so that rare disease patients can start to benefit from the new resources.

It is important to appreciate that in contrast to many Canadians who rely on third-party coverage assistance for contraceptives and diabetes medications and supplies, who generally have good coverage for a wide range of treatment options, a wide swath of rare disease patients have poor to no access to the treatments they need. A recent comparative review of access to orphan medicines in Canada, the US and Europe revealed that Canadians wait an average of 18 months longer than Europeans and Americans for new rare disease medicine approvals and only 60% of the orphan medicines authorized in the US and/or Europe are approved in Canada.[5] Even when they are approved by Health Canada, many are not reimbursed publicly or only reimbursed for some patients who satisfy restrictive access criteria.
Given that, RAREi recommends that priority be given to completing the bilateral negotiations related to the DRD strategy before work gets underway on the negotiations to implement the national pharmacare initiative.

Regarding the requirement to agree on a nationally agreed-upon list of new and emerging rare disease medicines that would have to be covered in all jurisdictions and cost-shared by the federal funds, RAREi notes that such a common list of new medicines exists already and is updated every month with the agreement of all levels of government through the pan-Canadian Pharmaceutical Alliance (pCPA). The new federal funding should simply be directed at supporting the cost-sharing and expansion of faster public access to any existing, new and emerging therapies for rare disease medicines that have been and will be negotiated successfully by the pCPA. In other words, deploy the funding immediately to the PTs, as well as to the federal drug programs, in order to increase the speed and scope of coverage for publicly reimbursed rare disease medicines.

## 4. Conclusion

In February 2019, this committee issued a report following its study of the barriers that Canadians with rare diseases face in accessing treatments, and examining how the federal government, in partnership with the provinces and territories, could help remove these barriers. It concluded that the time had come to change Canada's approach towards DRDs and it made 19 recommendations that were intended to prompt the search for short- and long-term options for covering the costs of DRDs, including "that the reimbursement of drugs for rare diseases be ***included as part of a national pharmacare program*** (emphasis added) established by the Government of Canada." It added a call for a jointly funded compassionate care program that covers the costs of drugs for rare diseases while they are under review for market authorization and reimbursement.

Those recommendations suggest that the previous members of this committee clearly understood the needs of the Canadian rare disease community and felt that they should be prioritized in any national pharmacare initiatives to be pursued federally. RAREi urges you to build on the work of the previous

[5] Rawson N & Adams J, *Orphan drugs approved in Canada: health technology assessment, price negotiation, and government formulary listing*, Expert Opinion on Orphan Drugs, February 13, 2024: https://doi.org/10.1080/21678707.2024.2313766.

parliament's health committee to recommend that the national DRD strategy is pursued as the government's first priority on expanding access to medicines for Canadians.

This is an extremely exciting time for the rare disease community given the incredible scientific and technological advances in recent years, there is still a lot of work to be done to ensure the benefits of these new technologies reach the Canadian patients who need them. This underscores the importance of the committee's review of these reforms and indicates that it can play a vital role in improving access to needed treatments for the most vulnerable patients in Canada.

We appreciate the opportunity to provide our input and look forward to your report and recommendations on this important issue. Ultimately, we hope to work with all governments in Canada, patients and clinicians to help ensure that Canadians with rare disorders receive timely access to the health care that they need and deserve. RAREi would welcome the opportunity to participate in any policy dialogue to identify, evaluate and advance a different approach to these reforms.

## About RAREi

RAREi is an informal network of research-based bio pharmaceutical innovators committed to monitoring, responding and shaping policy issues in the Canadian rare disease environment. The members of RAREi are Alexion AstraZeneca Rare Disease Canada, Amgen Canada, Amicus Therapeutics, argenyx Canada, Biogen Canada, Biomarin Pharmaceutical, Boehringer Ingelheim Canada Ltd., GSK Inc., Ipsen Biopharmaceuticals Canada, Johnson & Johnson Innovative Medicines, Mitsubishi Tanabe Pharma Canada, Recordati Rare Diseases Canada, Sanofi Canada, Sobi Canada, Takeda Canada, Ultragenyx Pharmaceutical and Vertex Pharmaceuticals (Canada). For more information, see www.rarei.ca.