RAREi
THE CANADIAN FORUM FOR RARE DISEASE INNOVATORS

Submission to Senate Standing Committee on Social Affairs, Science and Technology (SOCI)

Prioritizing Rare Disease Patients

SOCI's review of Bill C-64 (An Act Respecting Pharmacare)

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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.

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.

In this context, we hope the following considerations can help inform a more substantive review and debate on this important legislation.

Recommendations

1) Focus on addressing existing coverage gaps

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, most 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. A recent Conference Board of Canada (CBOC) analysis determined that fewer than 3% of Canadian residents have no access to reimbursement for their medicines.[1] Meanwhile, approximately 10% of Canadians are eligible for medication coverage through a public or private plan, but are not registered.

While the current system undoubtedly has limitations, Canadians' pharmaceutical coverage needs are generally well-served. As such, RAREi strongly believes that the focus of the government's efforts needs to be on addressing the system limitations rather than upending what is working well for most.

One patient community whose medication treatment needs have not been well-served is people coping with rare diseases. Canadians who rely on third-party coverage assistance for contraceptives and diabetes medications and supplies generally have good coverage for a wide range of treatment options already, but 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.[2] Even when they are approved by Health Canada, many are not reimbursed publicly or only reimbursed for some patients who satisfy restrictive access criteria.

It is also important to note that rare disease patients tend to be much more affected by plan design-related coverage barriers 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 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 community's 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 1) ensuring that all Canadians have access to good quality third-party coverage, 2) addressing the many limitations that restrict access to available treatment options

[1] 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.

[2] 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.

effectively, 3) reducing out-of-pocket costs imposed on patients and their families and 4) ensuring effective and timely mechanisms are in place to permit exceptional access when patients require a treatment that is not listed as a benefit, but nevertheless is the best option to meet their individual circumstances.

2) Consider potential unintended consequences

RAREi recommends that SOCI take the time to carefully review Bill C-64 to mitigate any potential unintended consequences. For instance, former Ontario Health Minister George Smitherman, who characterized Bill C-64 as "poor public policy" highlights some of the potential challenges.[3] He believes that the pursuit of a narrowly applied single-payer model could disrupt 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 what 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.

3) Ensure that the national pharmacare initiative does not divert from or upend Health Canada's efforts to negotiate separate funding deals related to the national DRD 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 as well that the 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 subset of emerging rare disease medicines be funded through the new system. Unfortunately, since the strategy was announced more than eighteen months ago (**and more than five years after the initial Budget 2019 pledge to direct substantial dedicated resources to the rare disease community**), only one bilateral agreement has been signed with British Columbia, and there is no indication when additional deals will get signed.

[3] 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.

[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.

Unfortunately, the effort to move the DRD Strategy forward and get the funding flowing is now complicated further by the introduction of Bill C-64, which requires yet another set of bilateral negotiations with PTs to distribute the new five-year, $1.5 billion pledge that will facilitate expanded public coverage for contraceptives and diabetes medicines and supplies.

It must also be noted that Bill C-64 has also been characterized as a component of the government's longer-term effort to implement national universal pharmacare. Even so, the recent DRD bilateral agreement with BC represents a notable departure from the proposed national pharmacare legislation in terms of respecting provincial jurisdiction for health care delivery and existing public drug plan structures.

Bill C-64 would require PTs to offer "first-dollar, universal" coverage for a select list of "essential" medicines recommended by Canada's Drug Agency, while the DRD deal allows the PTs to fund existing and new rare disease medicines based on their current systems of eligibility and coverage. These fundamentally contradictory approaches would need to be reconciled before a comprehensive national pharmacare vision could be realized.

From RAREi's perspective, the reliance on a defined list of medicines eligible for public coverage is problematic for rare disease patients, who by the nature of the rarity of their condition, may not fit the criteria for access to a given treatment neatly and who may have to try several treatment options before finding one that works for them. RAREi has called for customized processes for reviewing, approving, and funding orphan medicines that recognize the unique challenges of managing rare diseases affecting small populations. While respecting the current pharmaceutical management system and PT formularies may not be ideal, it is certainly preferable to creating an entirely new list and forcing the jurisdictions to replace the coverage that they currently offer.

In light of the already lengthy delays in implementing the national DRD strategy and getting funding for treatment access to flow to PTs to help them improve their access to orphan medicines, 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.

4) Examine the implications of introducing a national formulary and national bulk purchasing strategy before implementing anything

Bill C-64 would require much more than just extending universal access to certain contraceptives and diabetes treatments for Canadians. It would also require the development of a national formulary, a national bulk buying strategy, a national appropriate use strategy, and the appointment of an expert committee with a mandate to make recommendations respecting options for the operation and financing of national, universal, single-payer pharmacare.

Unfortunately, most of the debate related to the bill has focused on the coverage of contraceptives and diabetes treatments, without much attention paid to the other elements, which it could be argued might have a wider and much more significant long-term impact on Canadians' medication coverage.

As indicated above, RAREi has concerns about reliance on a restrictive national formulary that might limit access to important treatment options for patients in need. Those concerns would be particularly troublesome in a single-payer situation, where it would be challenging to obtain access to treatments that are available for sale but are not included on the national list.

It also has a long list of questions about what a national bulk purchasing strategy for prescription drugs and related products might look like (keeping in mind that broadly-speaking, public and private payers reimburse, rather than purchase, medicines). Let us remind you that Canada already has a highly effective national price negotiation process that successfully negotiates very favourable prices for medicines reimbursed by all public plans in Canada and that private payers have an unfettered ability to negotiate terms with pharmaceutical suppliers as well. This raises the question, what is the government hoping to achieve with a bulk-purchasing plan, and what impact might that have on patient access to needed treatments?

RAREi urges the committee to challenge the government to address these questions clearly and to consider their implications for ongoing research and development and commercialization of innovative medicines as well as their potential impact on access to treatments now and into the future before proceeding with those elements of the legislation.

Conclusion

This is an extremely exciting time for the rare disease community given the incredible scientific and technological advances in recent years. However, there is still a lot of work to ensure these new technologies' benefits reach the Canadian patients who need them. This underscores the importance of the SOCI 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 Canadian governments (federal and provincial), 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 in the context of Bill C-64.

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.