Canadian Organization for Rare Disorders

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Pre-Budget Consultations on the 2020 Budget

House of Commons Standing Committee on Finance

August 2, 2019

Recommendation #1

That the federal government develop a national strategy for rare disease treatments that is equitable and high quality, meaningfully involves patients, supports innovation and ensures value for money.

Recommendation #2

That the federal government refrain from implementing the proposed reform to the federal Patented Medicine Prices Review Board (PMPRB) as it will create a significant barrier for timely patient access to rare disease treatments.

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. Given that Canada remains one of the few developed countries that has not adopted a national plan for rare diseases, CORD urges the federal and provincial governments to do their part to implement Canada's Rare Disease Strategy.

Recommendation #1

That the federal government develop a national strategy for rare disease treatments that is equitable and high quality, meaningfully involves patients, supports innovation and ensures value for money.

CORD was pleased that the federal budget 2019 announced plans to develop a rare disease strategy and allocate $1 billion over two years as of 2022-23 to improve patients' access to rare disease therapies, with up to $500 million annually thereafter.[2]

The lack of access to the right therapies significantly impacts patient outcomes and can result in increased morbidity, loss of life or poorer quality of life and increased costs to the family, the healthcare system and the Canadian economy.

Implementing a national strategy for rare disease drugs represents a unique opportunity for the federal government to lead and coordinate efforts to improve access to medicines for the 3 million Canadians suffering from a rare disorder.

We recommend that the federal government include these core principles as the basis of its rare disease strategy:

1. **Equity:** Patients have timely, appropriate access, based on individual needs, regardless of where in Canada they live and whether they rely on public or private drug plans.
2. **Quality:** Patients receive the best possible therapy within a system that also provides optimal care and support.
3. **Patient involvement:** Patients and families co-design the program with policy-makers and are embedded in the decision-making process on an ongoing basis.
4. **Support for innovation:** Reimbursement of rare disease treatments contributes to a better understanding of rare diseases and future research and development of innovative therapies.

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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.

5. **Value for money:** Lifecycle learning models update access and reimbursement based on real world outcomes and health system impacts across the country.

Further, we encourage the federal government to include these features when developing the rare disease strategy:

- **Comprehensive:** Rare disease patients have a comprehensive public benefit plan that is not restricted to only offsetting catastrophic expenses but addresses the need for appropriate and timely access to medicines and related therapies.
- **One formulary:** A single formulary that incorporates managed access based on a set of universal eligibility criteria is the foundation to assuring equity in access across the country.
- **Flexible:** Different funding approaches and mechanisms are incorporated to address varying symptoms and manifestations of different rare diseases, including exceptional adjudication measures to address the unique circumstances of each patient (e.g., funded access to off-label and off-formulary medicines that may be the right therapy for specific patients).
- **Integrated with the healthcare systems:** Treatment is integrated into a system of patient care that ensures accurate diagnosis, patient education, expert patient management, monitoring for safety and outcomes, and data analysis to assure appropriate use and value for money.
- **Universal:** All rare disease patients are eligible for all services and treatments regardless of their coverage plan.

Finally, we believe that a strategy for rare disease treatments must be uniquely designed to respond to the context of rare diseases. This has been acknowledged by an interprovincial working group of public drug plans when designing a Supplemental Process for Complex and Specialty Drugs.

In implementing the national strategy, the federal government should therefore work with Health Canada, the Canadian Agency for Drugs and Technologies in Health (CADTH), the pan-Canadian Pharmaceutical Alliance (pCPA) and provincial governments to take the following actions at various steps of the review process.

Regulatory process

It is essential that Health Canada officially defines “rare disease” and “rare disease drugs” as part of a formal regulatory framework for rare diseases to ensure alignment for patient care across the country.

Despite advances in the regulatory process, it is disconcerting that patient access to, and research on, rare disease therapies in Canada continues to lag behind other countries. A review of regulatory approval timelines found that 84% of drugs for rare or ultra-rare disorders were authorized by Health Canada roughly eight months later than in the US and Europe. Worse, smaller companies received regulatory authorization two years later than in these other jurisdictions.[3]

[3] Rawson, N., Fraser Institute, *Regulatory, Reimbursement, and Pricing Barriers to Accessing Drugs for Rare Disorders in Canada*, 2018: https://www.fraserinstitute.org/sites/default/files/barriers-to-accessing-drugs-for-rare-disorders-in-canada.pdf.

In developing an effective rare disease strategy, the federal government should implement a distinct orphan drug regulatory framework that incorporates international best practices and closely aligns with key international regulatory agencies to encourage companies to develop treatments for rare diseases and make them available in Canada in a timely fashion.

Health technology assessment

Since the health technology assessment (HTA) process results in recommendations to public drug plans about whether they should reimburse a medicine, the approach used to evaluate rare disease therapies must be fit for purpose.

Currently, HTA recommendations from CADTH often come with very limited access criteria and expectations of substantial reductions in drug prices (up to 98.5%), despite recent changes to help address the challenges of rare disease treatments. This leads to delays and breakdowns during subsequent pricing negotiations between drug manufacturers and governments under the pCPA, ultimately resulting in delayed patient access to new medicines.

We urge the federal government to work with provinces and CADTH to revise the current approach and include criteria better suited to rare disease treatments. An appropriate HTA review process would accommodate uncertainties in the evidence, which are inherent for drugs researched with small patient populations suffering from serious conditions for which there are few, if any, existing therapies with which to compare clinical evidence and cost-effectiveness. As well, the process should include input from clinicians with expertise in the specific rare disease under review. This process should result in criteria that can be incorporated into real world evidence generation and managed access programs (MAPs), which will help reduce uncertainty and generate important outcomes data.

Reimbursement by public drug plans

Across the country, reimbursement of drugs for rare diseases is inconsistent, restrictive and lengthy. Currently, the HTA process and pCPA negotiations result in a longer delay for all patients using public plans (449 days on average) than private plans (142 days on average).[4] Negotiations for rare disease treatments can take between one to two years, if they are concluded at all.

The federal government should include a new reimbursement process tailored to the unique characteristics of rare disease treatments that makes them available during pCPA negotiations. For instance, Germany offers immediate access to new medicines during negotiations between the government and drug manufacturers. The new reimbursement process should also incorporate MAPs that can help address uncertainties related to rare disease drugs in order to facilitate and speed up reimbursement of these therapies.

[4] Canadian Health Policy Institute, *Coverage of new medicines in public versus private drug plans in Canada 2008-2017*, 2018: https://www.canadianhealthpolicy.com/products/coverage-of-new-medicines-in-public-versus-private-drug-plans-in-canada-2008-2017.html.

Investments in research and national clinical practice networks

The identification and understanding of rare diseases and development of diagnostic and therapeutic options will significantly reduce global public health costs and the burden of disease while improving the quality of life for those suffering from a rare disease. Rare disease research can also contribute to the understanding of more common diseases leading to therapeutic options for these conditions.

Canada already benefits from enormous pre-clinical rare disease research strengths both in terms of molecular diagnostics and the pathogenic elucidation that follows rare disease gene identification. We have dozens of open and inclusive research teams, strong international collaborations and a research capacity that spans the research continuum, including basic biomedical research, clinical research and health services and policy research. These strengths can be further leveraged, and Canada can learn from and contribute to international networks and leadership in rare disease research, including collaborative research community, cutting edge "omics" platforms, model systems, etc.

In this context, we encourage the federal government to continue to play an important coordinating and funding role in personalized medicine and rare disease research, notably, through the granting councils, and especially the Canadian Institutes of Health Research and Genome Canada, among others. These investments are critical to ensuring Canadian researchers and clinicians stay at the forefront of research and clinical practice globally. To ensure our on-going participation in the international rare disease research environment, Canada should assure an investment of $5 million per year.

Recommendation #2

That the federal government refrain from implementing the proposed reform to the federal Patented Medicine Prices Review Board (PMPRB) as it will create a significant barrier for timely patient access to rare disease treatments.

Health Canada has proposed changes to the *Patented Medicines Regulations*, which govern the PMPRB, to substantially lower the prices of drugs – especially drugs for rare diseases. At first glance, much lower prices sounds like an idea that everyone should endorse. In reality, this change will create a significant barrier for accessing rare disease treatments. This would be counterproductive to a national strategy on rare disease drugs, which aims to improve patient access to therapies.

In particular, this reform would set prices of new medicines so low most pharmaceutical companies will be unable to bring them to Canada, which would have a truly devastating effect on patients who depend on new life-saving medicines to survive and get better. Although Health Canada has been stressing that this risk will not materialize, it has produced no compelling evidence to support its position, while there have been numerous studies indicating that market price and availability of medicines are linked.[5]

While we support fair and sustainable pricing of pharmaceuticals, we are very concerned that the proposed changes provide insurmountable disincentives for bringing innovative medicines to Canada.

[5] See Canadian Health Policy, Medical resources and spending across provincial healthcare systems in Canada, 2019: https://www.canadianhealthpolicy.com/products/medical-resources-and-spending-across-provincial-

Based on PMPRB's analysis, the reform will reduce pharmaceutical prices from 40-70%.[6] As well, the proposed changes are inconsistent with the policies and practices of other countries, none of which apply cost-effectiveness analyses to cap prices, as currently proposed. As a way to address affordability concerns, we have repeatedly encouraged the use of MAPs in pCPA negotiations instead of the proposed regulatory reform.

Finally, the consultation process undertaken by Health Canada and the PMPRB on the reform was deeply flawed, disrespectful, misleading and tokenistic. At the conclusion of the consultations, there remains no analysis conducted on the proposed changes to evaluate their impact on patient access, despite more than 100 patient groups calling for this.[7]

We therefore urge the federal government not to implement the drug pricing reform as proposed, as it will end up harming one the most vulnerable segment of the Canadian population, those who suffer from a rare disorder.

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healthcare-systems-in-canada-.html; and Ernst & Young report, An assessment of Canada's current and potential future attractiveness as a launch destination for innovative medicines, January 2019: https://innovateforlife.ca/sites/all/themes/imc3/files/2019_01_29_IMC_PhRMA_LaunchSequencing_vFINAL3.pdf

[6] These case studies were shared with the Steering Committee on Guidelines Modernization in December 2018: http://www.pmprb-cepmb.gc.ca/view.asp?ccid=1378&lang=en

[7] PDCI website: http://www.pdci.ca/sharing-responses/