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THE CANADIAN FORUM FOR
RARE DISEASE INNOVATORS

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House of Commons Standing Committee on Finance

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RAREi Recommendations

1 **–** That the federal government accelerate planned investments in improving access to treatments for rare disease patients.

2 **–** That the federal government repeal new economic factors from the *Patented Medicines Regulations* given their significant negative impact on access to rare disease treatments and clinical trials.

3 **–** That the federal government pursue a holistic national rare disease strategy to improve access to diagnosis, care and treatments for all Canadians with rare diseases.

About RAREi

RAREi is a network of 12 Canadian biopharmaceutical companies committed to improving the lives of rare disease patients by researching, developing and commercializing rare disease treatments.

Recommendations in Context

Recommendation 1 – That the federal government accelerate planned investments in improving access to treatments for rare disease patients.

In Budget 2019, the government announced that "Special consideration is required to ensure a nationally consistent approach for these (rare disease) medications," and committed $500 million per year "to help Canadians with rare diseases access the drugs they need" starting in 2022-23.[1] While encouraging, RAREi is concerned that waiting until 2022-23 is too long.

Rapidly evolving research and technological advances are leading to impressive breakthroughs for rare disease patients, most of whom struggle for years to obtain an accurate diagnosis only to find no reasonable treatment options are available. The recent emergence of many exciting new therapies has offered unprecedented hope for the future but this is significantly tempered by Canada's policy framework and pharmaceutical review and approval processes.

A February 2020 *CMAJ* blog outlined the many hurdles faced by Canadian rare disease patients in accessing available medicines, pointing out the barriers at each stage before a new medicine is listed on a formulary. Even then, restrictive criteria often derail attempts to receive treatment.[2] According to Innovative Medicines Canada, 78% of patients with rare diseases experienced challenges accessing needed medicines, with 70% facing delays and 58% denied access because of cost.[3]

Unlike most developed countries, Canada has no national rare disease treatment policy and payers are reluctant to reimburse them in the absence of any such formal assessment and management program.

Payers' are primarily concerned about their relatively higher public (list) prices. However, a recent evidence-based analysis found that non-oncology-related orphan medicines represented only 1.9% of total public medication expenditure in Canada in 2019, a percentage that is expected to increase to only 6.5% by 2025 for a total estimated spend of $1.39 billion. This estimate is likely far above actual spending too, given that savings to public payers via product listing agreements were not included. The analysis concluded that public expenditure for rare disease treatments is minimal in comparison to other important expenditures.[4]

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[1] Government of Canada, Budget 2019: Investing in the Middle Class, p. 62, March 19, 2019: https://budget.gc.ca/2019/docs/plan/budget-2019-en.pdf.

[2] Rawson N and Lawrence D, *Patient Access to Essential Rare Disorder Drugs: The Long and Winding Road*, CMAJ Blogs, February 12, 2020: https://cmajblogs.com/patient-access-to-essential-rare-disorder-drugs-the-long-and-winding-road/#more-6843.

[3] Fralick P, *People with rare diseases need help*, Innovative Medicines Canada, March 2, 2020: http://innovativemedicines.ca/people-rare-diseases-need-help/.

[4] Forte L et al., *The Current and Future Costs of Orphan Drugs in Canada - A Public Payer Budget Impact Analysis*, Patient Access Solutions, 2019 ISPOR Europe Poster, November 2019: https://www.ispor.org/heor-resources/presentations-database/presentation/euro2019-3122/96632.

Accordingly, earlier federal support, both financial and organizational, is needed to ensure patient access to new rare disease medicines. RAREi urges the federal government to implement its rare disease commitments immediately.

Recommendation 2 **– That the federal government repeal new economic factors from the *Patented Medicines Regulations* given their significant negative impact on access to rare disease treatments and clinical trials.**

In August 2019, the federal government amended the *Patented Medicines Regulations* (PMRs), changing how the Patented Medicine Prices Review Board (PMPRB) regulates patented pharmaceutical prices in Canada. The reforms, now to be implemented on January 1, 2021, give the PMPRB new powers to implement aggressive price controls,[5] including the application of new economic factors, leading to steep price reductions for almost all patented medicines in Canada. It is expected that public list prices for all patented medicines will fall by 15% on average. Medications deemed high-cost or high-volume will be subject to further 35-65% price reductions. These cuts are significantly higher than original Health Canada projections, creating unprecedented business uncertainty.

Research shows that Canada could lose its status as an early market for new medicines[6] and as a prime location for global clinical trials.[7] This means Canadians would not have prompt access to new medicines, causing poorer health outcomes and disruptions in provincial health systems.[8] A recent industry leaders' survey confirmed those findings, revealing unanimity regarding the negative implications.[9]

In fact, uncertainty is already affecting access to new medicines. One study found new Canadian medicine launches dropped dramatically recently, corresponding with the timing of the PMPRB reforms. Most medicines launched globally in 2018 but not yet commercialized in Canada are for rare diseases and cancer.[10] Moreover, the number of trials registered by Health Canada from November 2019 to mid-March 2020 fell by 52% compared with the average number registered during that timeframe in the previous six years. The number of new clinical trials also fell in the US, but only by 21%.[11]

This suggests that the reforms will result in fewer breakthrough medicines – including vaccines and treatments being developed for COVID-19 – to cover under any future national pharmacare or rare disease strategy. That is

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[5] *Patented Medicines Regulations,* Canada Gazette II, August 21 2019 : http://gazetteducanada.gc.ca/rp-pr/p2/2019/2019-08-21/html/sor-dors298-eng.html

[6] Skinner B., *Consequences of over-regulating the prices of new drugs in Canada*, Canadian Health Policy Institute, 2018 : https://www.canadianhealthpolicy.com/products/consequences-of-over-regulating-the-prices-of-new-drugs-in-canada.html.

[7] Skinner B., *Patented drug prices and clinical trials in 31 OECD countries 2017: implications for Canada's PMPRB*, Canadian Health Policy Institute, 2019: https://www.canadianhealthpolicy.com/products/patented-drug-prices-and-clinical-trials-in-31-oecd-countries-2017--implications-for-canada---s-pmprb-.html?buy_type=.

[8] Skinner B., *Consequences of over-regulating the prices of new drugs in Canada*, Canadian Health Policy Institute, 2018 : https://www.canadianhealthpolicy.com/products/consequences-of-over-regulating-the-prices-of-new-drugs-in-canada.html; and Ernst & Young report, *An assessment of Canada's current and potential future attractiveness as a launch destination for innovative medicines*, 2019: http://innovativemedicines.ca/wp-content/uploads/2019/02/2019_01_29_-IMC_PhRMA_LaunchSequencing_vFINAL3.pdf.

[9] Research Etc., Survey for Life Sciences Ontario, February 2020: https://lifesciencesontario.ca/news/new-federal-drug-pricing-rules-are-already-delaying-medicine-launches-and-costing-jobs-in-canada-survey-reveals/.

[10] Life Sciences Ontario Webinar: *New Medicine Launches: Canada in a Global Context*, June 2020 : https://lifesciencesontario.ca/wp-content/uploads/2020/06/EN_LSO_Global-Launch-Benchmarking_Webinar-June22-20_Final.pdf.

[11] Rawson, N, *Clinical Trials in Canada Decrease: A Sign of Uncertainty Regarding Changes to the PMPRB?* Canadian Health Policy, April 2020. Toronto: Canadian Health Policy Institute: https://www.canadianhealthpolicy.com/products/clinical-trials-in-canada-decrease--a-sign-of-uncertainty-regarding-changes-to-the-pmprb-.html.

the opposite of the federal government's access to rare disease medicines goal. They will also negatively impact the government's goal of doubling the size of the health and biosciences sector by 2025.[12]

The severity of the proposed price cuts will effectively delay or eliminate access to important new vaccines and therapeutics for patients – many of whom rely on new therapies to get better and even survive.

Recommendation 3 – That the federal government pursue a holistic national rare disease strategy to improve access to diagnosis, care and treatments for all Canadians with rare diseases.

Part of the Budget 2019 rare disease commitment was a pledge to work with stakeholders and other governments "to build a coordinated strategy for gathering and evaluating evidence on high-cost drugs for rare diseases, improve the consistency of decision-making and access across the country, negotiate prices with drug manufacturers, and ensure that effective treatments reach the patients who need them."[13] In doing so, it promised to be guided by the recommendations of the Advisory Council on the Implementation of National Pharmacare (ACINP).

The ACINP called for "a distinct national process for providing fair, consistent, timely and evidence-based access" to rare disease treatments and a "distinct pathway" for their review and approval led by a national expert panel that "would work with patients and their care teams, (and others) reviewing individual cases to determine whether a particular drug should be funded for a particular individual."[14]

RAREi supports these government commitments but in order to expand access to effective treatments, a comprehensive and distinct national rare disease treatment coverage program is required that enhances all elements of the Canadian process, including regulatory, pricing and access policies and funding. Each stage needs to be streamlined and customized to address the specific needs of patients with rare diseases rather than the current broad population-based approach. Changes should include:

- **A regulatory framework that incents the development and commercialization of rare disease therapies.** This would include a definition of rare disease, an orphan product designation process, additional market exclusivity, research promotion funds, tax incentives and regulatory submission fee reductions.
- **Pricing tests and tools that do not rely on cost-effectiveness assessments or have a disproportionate impact on rare disease treatments.** Federal government patented medicine pricing reforms would introduce a huge access barrier by requiring regulated price reductions of as much as 65% for rare disease medicines[15] and a disproportionate effect on rare disease treatments.[16] They should be reconsidered.
- **Canadian Agency for Drugs and Technologies in Health (CADTH) reviews should include processes, criteria and standards appropriately suited for rare diseases.** Health technology assessment reviews can be

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[12] Report from Canada's Economic Strategy Tables: Health and Biosciences, September 2018: https://www.ic.gc.ca/eic/site/098.nsf/eng/00025.html.

[13] Government of Canada, Budget 2019: Investing in the Middle Class, p. 62, March 19, 2019: https://budget.gc.ca/2019/docs/plan/budget-2019-en.pdf.

[14] Final Report of the Advisory Council on the Implementation of National Pharmacare, *A Prescription for Canada: Achieving Pharmacare for All*, September 24, 2019: https://www.canada.ca/en/health-canada/corporate/about-health-canada/public-engagement/external-advisory-bodies/implementation-national-pharmacare/final-report.html.

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

[16] See also Rawson N., *New Patented Medicine Regulations in Canada: Case Study of a Manufacturer's Decision-Making about Regulatory Submission for a Rare Disorder Treatment*, Canadian Health Policy Institute, October 2018: https://www.canadianhealthpolicy.com/products/new-patented-medicine-regulations-in-canada--case-study-of-a-manufacturer---s-decision-making.html.

enhanced by formally incorporating specialized clinician expertise, patient values, more frequent reliance on observational studies and real-world evidence (RWE) beyond randomized clinical trials, and recognizing the reality of smaller study sizes and the requirement for higher per patient prices for these treatments.

- **A reimbursement process, including negotiation and funding decisions, that provides timely and equitable access to rare disease treatments.** The public drug plan funding process should implement measures used elsewhere, such as allowing reimbursement at the time of regulatory approval, increased use of pay-for-performance, managed access programs and RWE to address clinical and economic uncertainties without delaying patient access.
- **A multi-stakeholder approach to inform provincial/territorial (PT) governments' proposed supplemental reimbursement review process for complex/specialty medicines.** Many of the initiatives listed above are under consideration by a PT working group on access to rare disease treatments.[17] The federal government can play a coordinating, supportive and policy role to ensure that all stakeholders are involved in designing any updated review process.
- **Investments in RWE research, infrastructure and policy development.** RWE can fill critical clinical gaps and increase certainty for public drug plans, payers and manufacturers, especially for orphan therapies. Health Canada is already leading efforts to expand RWE use but the federal government should continue to support a national RWE framework[18] by investing in research funding, patient registries and centres of clinical expertise, as well as pursuing enhanced regulatory reviews that support manufacturer data development submitted in regulatory applications.

Several of these initiatives were elaborated in RAREi's recommendations to the House of Commons Standing Committee on Health for a rare disease study,[19] and some were highlighted in the ACINP final report.[20] Both of these committees recognized the need for a distinct approach to rare disease treatments.

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[17] Expensive Drugs for Rare Diseases (EDRD) Working Group, Stakeholder Consultation: Supplemental Process for Complex/Specialized Drugs Background Document, November 2018: http://www.raredisorders.ca/content/uploads/EDRD-supplemental-process-background_24Oct2018_Final.pdf. *See also* RAREi Responses to Consultation Questions, December 2018: https://www.linkedin.com/feed/update/urn:li:activity:6559059577416007680, and EDRD Working Group, Stakeholder Engagement Summary: Supplemental Process for Complex and Specialized Drugs, July 2019: https://www.linkedin.com/feed/update/urn:li:activity:6558035111844073472.

[18] Institute for Health Economics (IHE), *Defining decision-grade real-world evidence and its role in the Canadian context: A design sprint* – Summary report, October 21, 2018: https://www.ihe.ca/events/past/conferences/ihe-capt-rwe/ihe-capt-rwe-about.

[19] RAREi, *Unique approach needed: Addressing barriers to accessing rare disease treatments*, October 31, 2018: https://www.linkedin.com/feed/update/urn:li:activity:6556579888877363200. *See also* Standing Committee on Health, *Canadians Affected by Rare Diseases and Disorders: Improving Access to Treatment*, February 2019: https://www.ourcommons.ca/DocumentViewer/en/42-1/HESA/report-22/.

[20] Advisory Council on the Implementation of National Pharmacare, *A Prescription for Canada: Achieving Pharmacare for All*, June 12, 2019: https://www.canada.ca/en/health-canada/corporate/about-health-canada/public-engagement/external-advisory-bodies/implementation-national-pharmacare/final-report.html.