RAREi
THE CANADIAN FORUM FOR
RARE DISEASE INNOVATORS

July 21, 2025

OPEN LETTER to Canada's First Ministers

Subject: Urgent action needed to improve access to rare disease treatments in Canada

Dear First Ministers,

The 2025 Council of the Federation meeting, which will include a discussion with Prime Minister Mark Carney, is a unique and important opportunity for renewed national attention and action on rare diseases.

Canadians living with rare diseases are among the most vulnerable and least well-served members of our society. With more than 7,000 rare diseases identified, and more discovered each year, these conditions collectively affect one in 12 Canadians. Yet, because each disease affects only a small number of individuals, many of whom are children, patients often face a fragmented system ill-equipped to meet their needs.

Challenges include limited clinical knowledge, data gaps and insufficient incentives for innovators to develop new treatments. Compounding this is the fact that **only about 60% of rare disease treatments launched globally are approved in Canada**. Even when Health Canada approves these therapies, **public reimbursement can take up to six years longer than in the U.S. and Europe**.[i] In 2022, **the average time from regulatory approval to public formulary listing was 736 days, which was twice the wait seen in peer countries**.[ii]

In light of this, RAREi welcomed the joint commitment made at the December 16, 2024 premiers' meeting to "accelerate patient access to new and lifesaving publicly funded medicines," and the corresponding mandate to health ministers.[iii] It was also encouraged by the federal government's March 2023 launch of the National Strategy for Drugs for Rare Diseases and the commitment to transfer more than 90% of the $1.5 billion, three-year fund to provinces and territories. The national election earlier this year also featured a key promise by the Liberal Party to accelerate funded access to life-saving medicines, recognizing that Canada is behind its G7 peers.

Unfortunately, these promising announcements have yet to deliver meaningful change. The rare disease community is growing increasingly anxious for tangible progress.

As a first step, RAREi urges all Canadian governments to endorse the May 2025 resolution of the United Nations World Health Assembly,[iv] which recognizes rare diseases as a global public health priority and calls for the development of national action plans. It asks further that any pilot projects aimed at accelerating access to innovative medicines explicitly include rare disease therapies as a targeted category.

At the same time, RAREi believes there is a real opportunity to reinvest in rare disease care using resources already identified within the system. The pan-Canadian Pharmaceutical Alliance (pCPA) estimated that its product negotiations saved public drug plans approximately $4.9 billion in 2024–25 alone.[v] A portion of these savings could be strategically allocated to improve access to rare disease

therapies, without compromising broader health care budgets. **Investing even a fraction of these savings toward innovative treatments, infrastructure and care coordination for rare diseases would yield life-changing health outcomes for thousands of Canadians and their families.**

Looking ahead, RAREi encourages pan-Canadian collaboration to evolve the federal rare disease strategy into a comprehensive national rare disease action plan. Such a plan must address not only access to treatments but also the broader infrastructure needed to support patients and caregivers throughout their journey. In this context, an effective national strategy should be developed collaboratively with the entire rare disease community, and especially with patient leaders with lived experience who are most affected by rare disorders. The action plan should have clear next steps that cover a number of elements, including:

- Universal and consistent newborn screening across provinces and territories
- Timely and equitable diagnosis
- Efficient patient referral systems to clinical centres of excellence
- Seamless interprovincial treatment coverage
- Sustainable access to new therapies
- Wraparound support for families and caregivers
- Incentives for innovative research
- Robust data collection and analytics
- Streamlined and customized regulatory review processes for orphan medicines

Achieving these goals will require a fundamental shift in how Canada evaluates and approves rare disease treatments. Current medication review systems were designed for therapies targeting large patient populations and are ill-equipped to assess the effectiveness, value and urgency of treatments intended for small or highly individualized groups. RAREi believes these systems must be modernized to reflect the unique characteristics of orphan medicines and keep pace with the rapid evolution of medical science.

This is a crucial time for health care in Canada. Advances in artificial intelligence, genomics and personalized medicine are rapidly reshaping how we understand and treat disease. Supporting rare disease innovation now will improve outcomes for patients and strengthen Canada's position in the global life sciences sector. Improving access to rare disease treatments is both a matter of equity and smart public policy.

RAREi is ready to work with your governments to build a system that delivers timely, effective care for all Canadians affected by rare diseases.

Thank you for your consideration.

Sincerely,

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About RAREi

RAREi is a network of biopharmaceutical companies dedicated to improving the lives of patients with rare diseases by developing and commercializing treatments. This network includes the following members: Alexion AstraZeneca Rare Disease, Amgen Canada Inc., Amicus Therapeutics Canada Inc., argenx Canada Inc., Boehringer Ingelheim Canada, Biogen Canada Inc., BioMarin Pharmaceutical (Canada) Inc., Ipsen Biopharmaceuticals Canada Inc., Johnson & Johnson Innovative Medicine, Medison Pharma Canada Inc., Mitsubishi Tanabe Pharma Canada Inc., PTC Therapeutics ULC, Recordati Rare Diseases Canada Inc., Sanofi-Aventis Canada Inc., Sobi Canada Inc., Takeda Canada Inc., Ultragenyx Pharmaceutical Inc., Vertex Pharmaceuticals (Canada) Inc. and UCB Canada Inc. For more information, please visit www.rarei.ca**.**

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[i] Ward L et al, An international comparative analysis of public reimbursement of orphan drugs in Canadian provinces compared to European countries, *Orphanet Journal of Rare Diseases*, March 4, 2022: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC8895096.

[ii] Conference Board of Canada, Access and Time to Patient Prescription Drugs in Canada, January 4, 2024: https://www.conferenceboard.ca/wp-content/uploads/2022/10/access-and-time-to-patient_jan2024.pdf.

[iii] Canada's Premiers, *Premiers Discuss Important Issues for Canadians*, December 16, 2024: https://canadaspremiers.ca/premiers-discuss-important-issues-for-canadians.

[iv] World Health Organization, First-ever rare diseases resolution underscores equity and inclusion, May 24, 2025: https://www.who.int/news/item/24-05-2025-seventy-eighth-world-health-assembly---daily-update--24-may-2025.

[v] pan-Canadian Pharmaceutical Alliance, June dashboard. July 2025: https://www.pcpacanada.ca/article-june-dashboard.