Health Canada Santé Canada

Canada

Drugs for Rare Diseases Strategy

Canadian Organization for Rare Disorders (CORD) - Fall Conference

November 29th, 2023

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Strategic Policy Branch

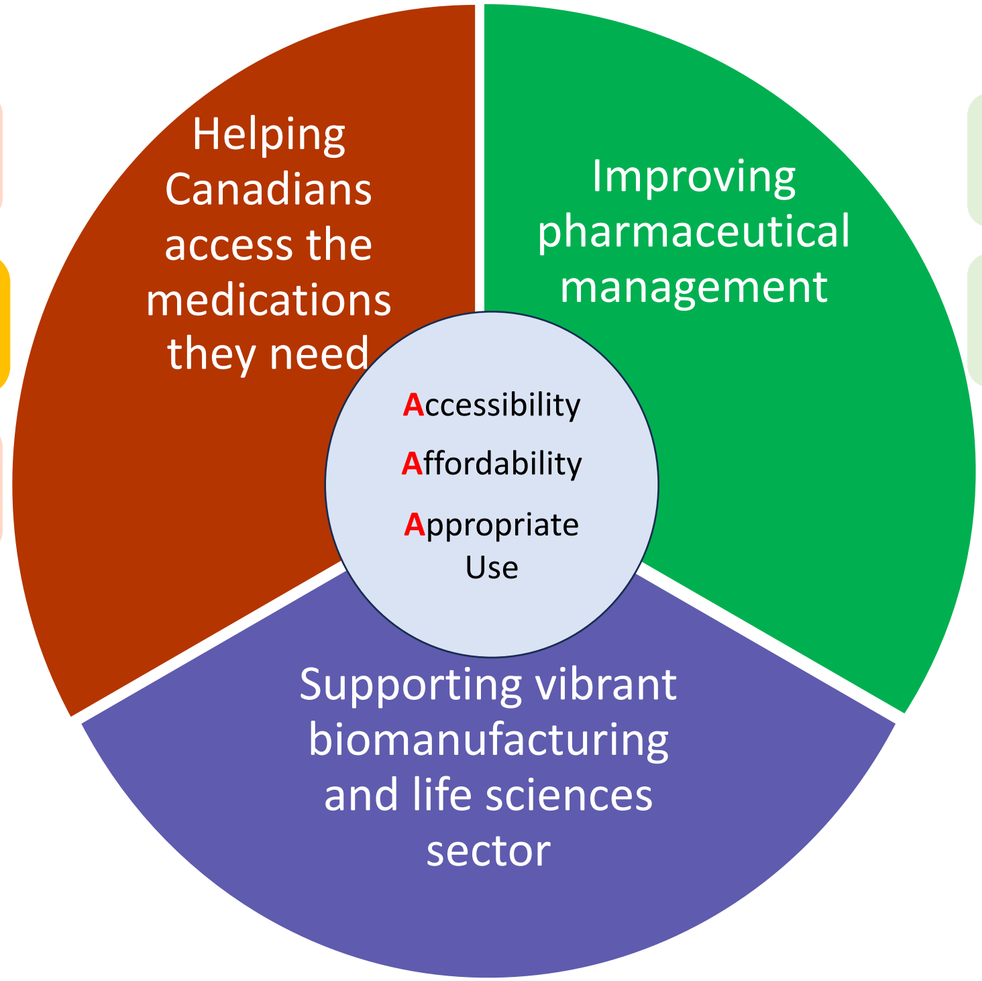
YOUR HEALTH AND SAFETY... OUR PRIORITY.

Federal Pharmaceutical Policy Agenda

PEI Pharmacare Initiative

National Strategy on Drugs for Rare Diseases

National Formulary (CADTH Panel Report)



Canadian Drug Agency Transition Office

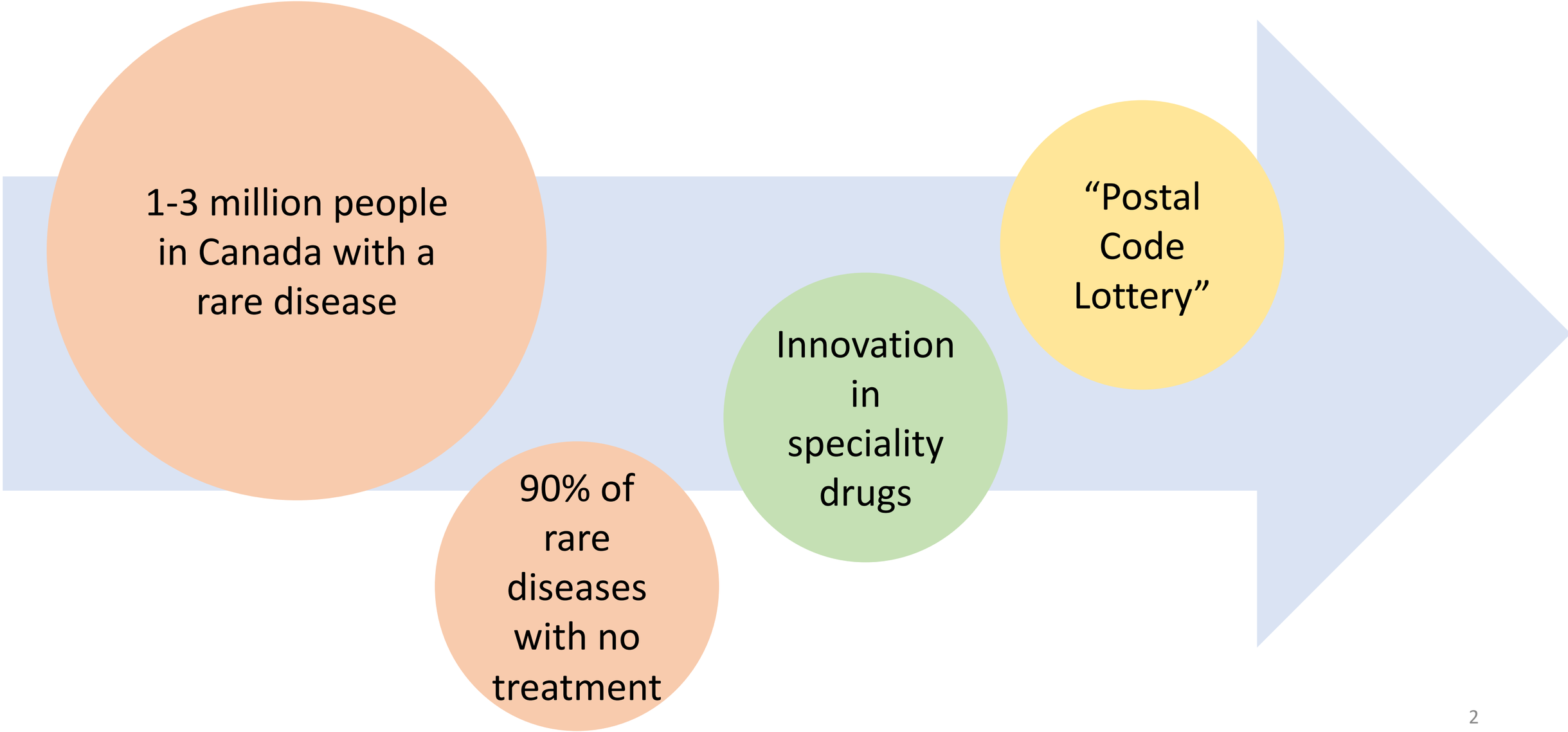
Amendments to the *Patented Medicines Regulations*

Canada's Biomanufacturing and Life Sciences Strategy

Pediatric Drug Action Plan

Regulatory agility and innovation

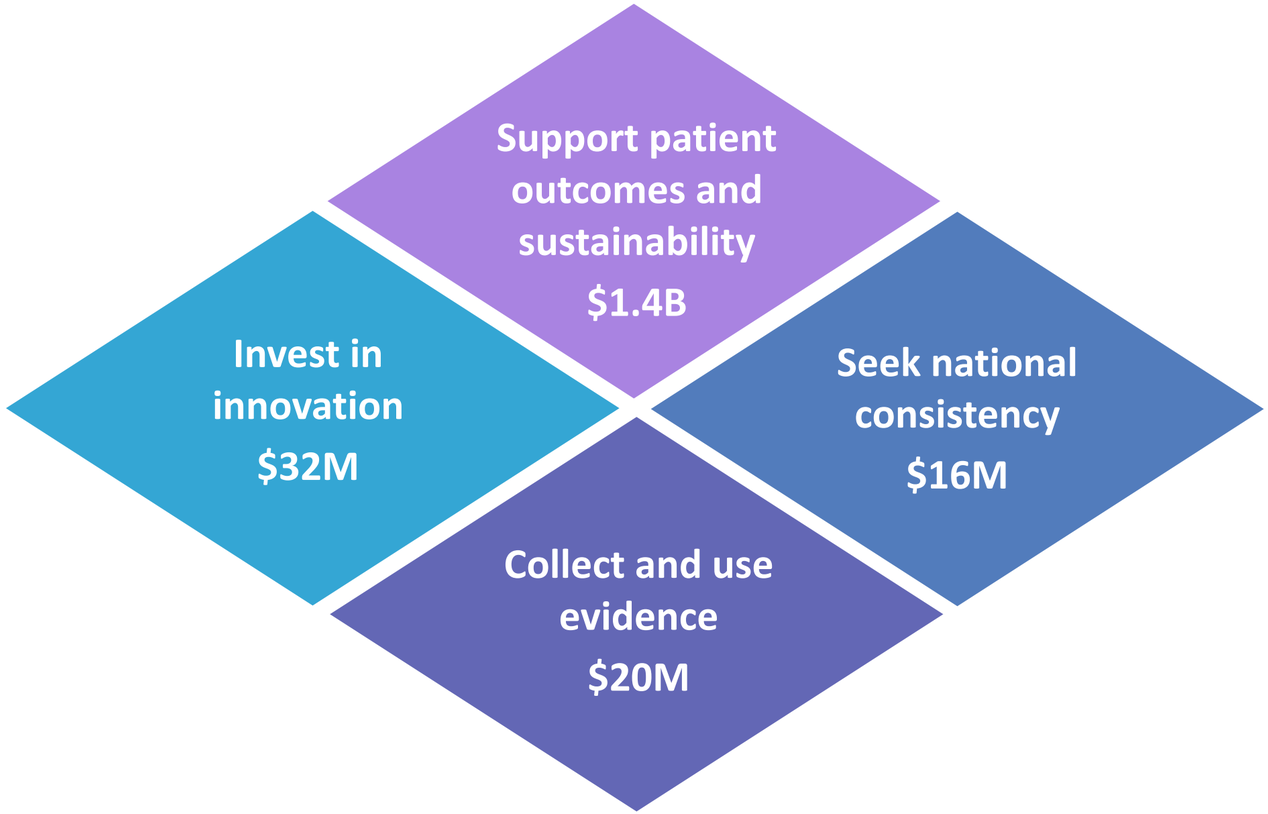
Drugs for Rare Disease - Today



National Strategy for Drugs for Rare Diseases

…increase **access** to, and the **affordability** of, **effective** drugs for the treatment of rare diseases…

Up to $1.5 B over three years



National Strategy for Drugs for Rare Diseases

Pillar	Actions
Support Patient Outcomes and Sustainability	Up to $1.4 B to work with provinces and territories on bilateral agreements to: •improve access to new and emerging drugs •support enhanced access to existing drugs •enhance screening and diagnostics activities $33M for Indigenous Services Canada's Non-Insured Health Benefits Program
Seek National Consistency	$16M for national governance structures: •Health Canada secretariat •Implementation Advisory Group
Collect and Use Evidence	$20M for Canadian Agency for Drugs and Technologies in Health (CADTH) and Canadian Institute for Health Information (CIHI) to improve the collection and use of evidence to support decision-making
Invest in Innovation	$32M for Canadian Institutes of Health Research (CIHR) rare disease research •developing better diagnostic tools •establishing a robust Canadian rare disease clinical trials network

Bilateral Agreements with Provinces and Territories

Update:

- Supported by a Health Canada Secretariat, FPT officials are proceeding with collaborative discussions to advance the components of the Strategy announced, including:
  - Developing a small set of new and emerging drugs that would be cost-shared and covered in a consistent way across the country;
  - Developing recommendations for a path forward on achieving national consistency in screening and diagnostics practices with respect to rare diseases; and
  - Supporting the work of health system partners on data

- The next step is to finalize bilateral agreements, informed by these discussions.

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Launch of Implementation Advisory Group (IAG)

Update:

- The Implementation Advisory Group (IAG) for the National Strategy for Drugs for Rare Diseases launched on Oct 26th, 2023.
- CORD, represented by Dr. Durhane Wong-Rieger, is a founding member of the IAG.
- Dr. Gail Ouellette and Dr. Avram Denburg are the current co-Chairs.
- The IAG will advise Health Canada on implementation of the National Strategy for Drugs for Rare Diseases and be a forum to exchange information and best practices on drugs for rare diseases.
- The rest of the membership, the mandate of the IAG, terms of reference, participant bios, meeting summaries and more can be found on the **IAG government webpage** which is now live:
  - https://www.canada.ca/en/health-canada/services/prescription-drug-system/implementation-advisory-group-national-strategy-drugs-rare-diseases.html

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Partners in DRD Data Initiatives: CADTH, CIHI, CIHR

Update:

Canadian Agency for Drugs and Technologies in Health (CADTH) / Canadian Institute for Health Information (CIHI)

- Leading six key activities to support decision-making across the pharmaceutical lifecycle
- Progress includes hiring of resources, engaging PTs and experts/stakeholders in needs assessment, drafting criteria for registry linkage, and identification of registries for analysis

Canadian Institutes for Health Research (CIHR)

- Launched four funding opportunities between June and August 2023, with funding for the largest project expected to begin early in 2024, and the remaining grants in the spring of 2024

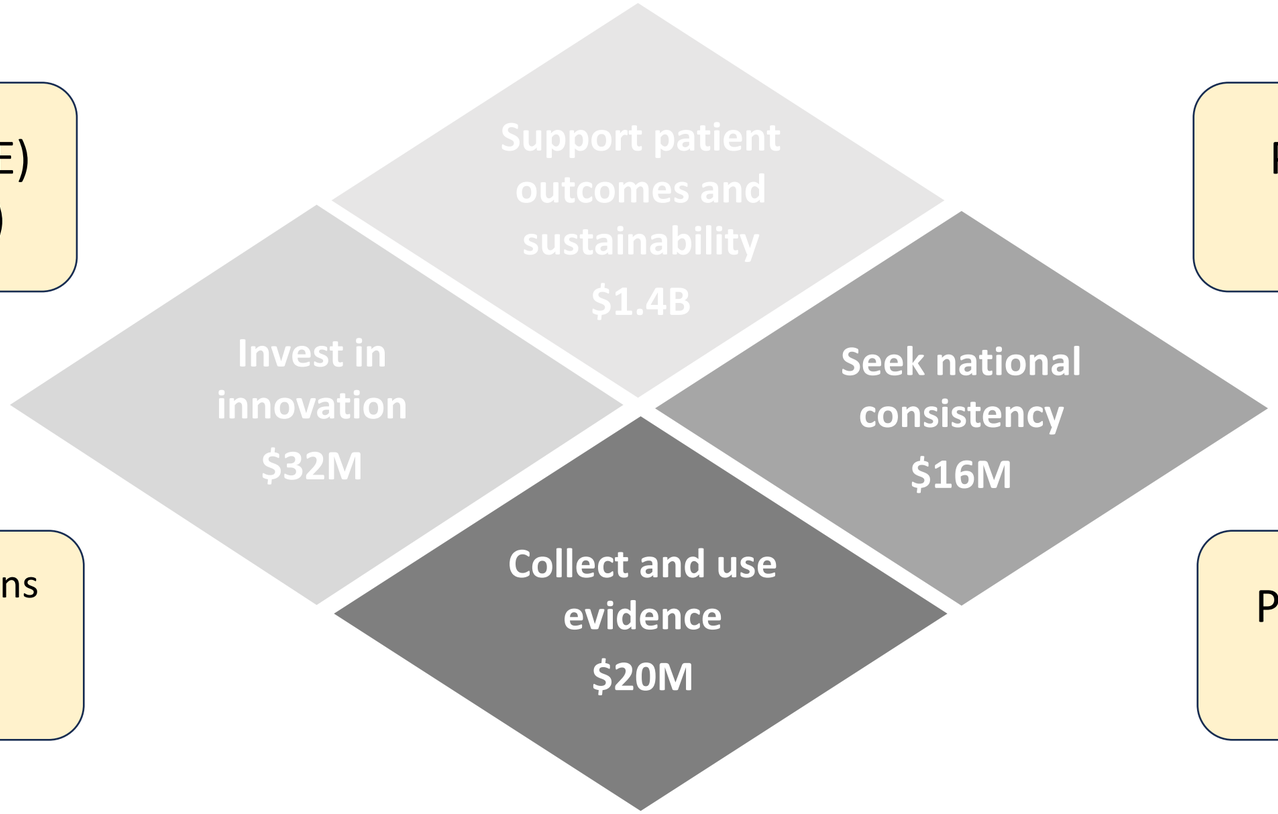
Early Research and Development	Screening and Diagnostics	Regulatory Approval and HTA Decision-making	Monitoring Ongoing Use
• **CIHR**: Funding opportunities on pediatric clinical trial network, supporting clinical trial readiness • **CADTH**: Pipeline tracking and horizon scanning	• **CADTH**: Newborn screening panel • **CIHR**: Funding opportunity on improving diagnosis	• **CADTH**: Disease-based registries and real-world evidence • **CADTH**: Customized pharmaceutical work and assessment • **CIHI**: Public drug plan and formulary tool	• **CADTH/CIHI**: Linked analysis for rare diseases • **CIHR**: Funding opportunity on enhancing the use of administrative data
Supporting development of new therapies and early identification of drugs likely to impact the system	Ensuring earlier intervention and reduced diagnostic odyssey	Improving business intelligence to inform jurisdictional decision-making	Achieving and optimizing long-term monitoring and analysis

Other Drug for Rare Disease Initiatives…

Time Limited Recommendations (CADTH) / Temporary Access Program (pCPA)

Politique québécoise pour les maladies rares (Gouv. du Quebec)

Real World Evidence (RWE) (CADTH, HC, INESSS, etc)



Pan-Canadian Genomics Strategy (ISED)

Disruptive Technology Solutions for Cell and Gene Therapy Challenge Program (NRC)

Pediatric Drug Action Plan (HC)