

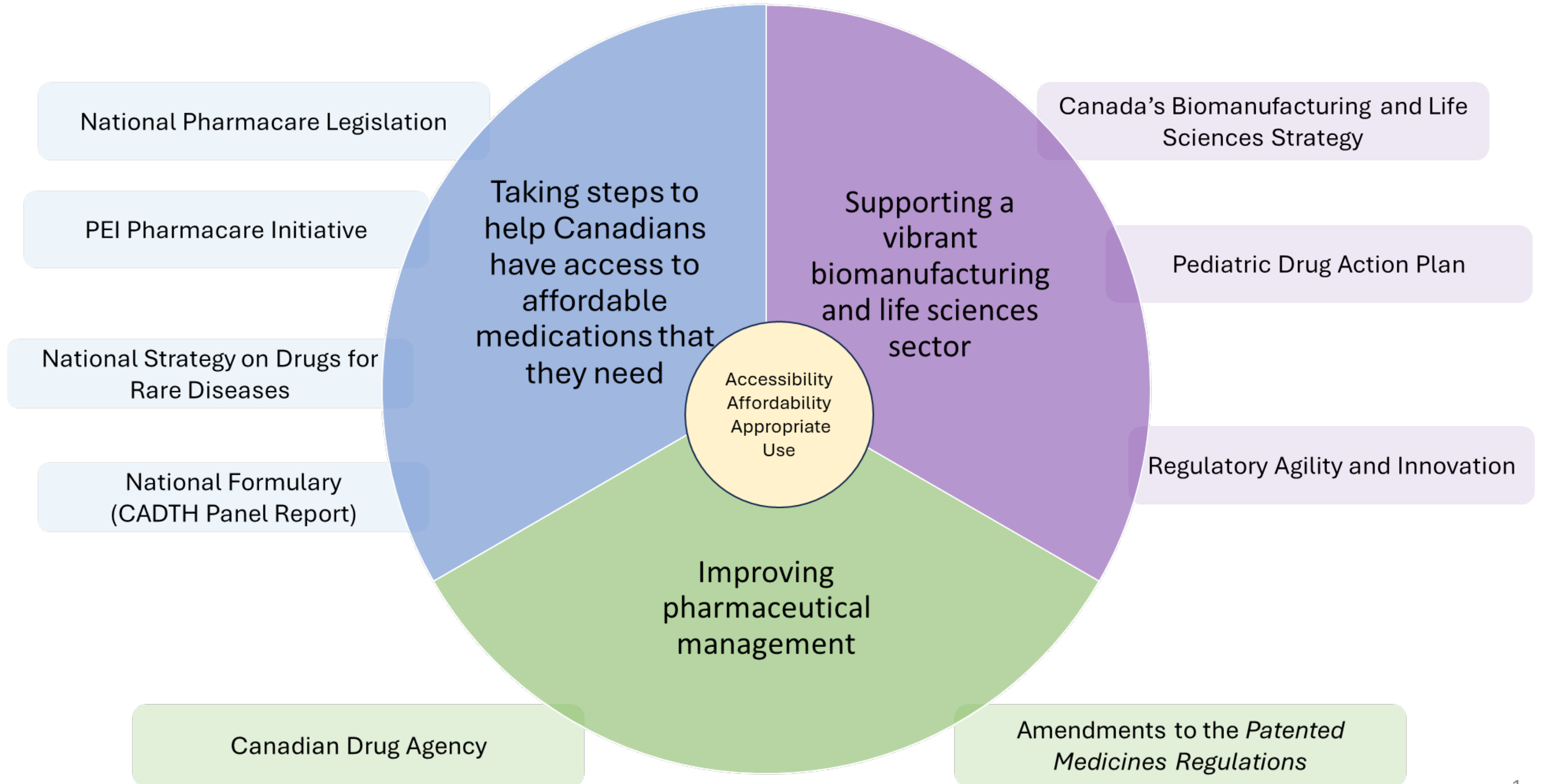
Drugs for Rare Diseases Strategy

Centre for Health Services and Policy Research (CHSPR) - Drug Coverage Conference
March 5th, 2024

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Strategic Policy Branch



Federal Pharmaceutical Policy Agenda



Federal Pharmaceutical Agenda Accomplishments

Since 2019, the federal government has made progress on foundational elements toward the implementation of national pharmacare:

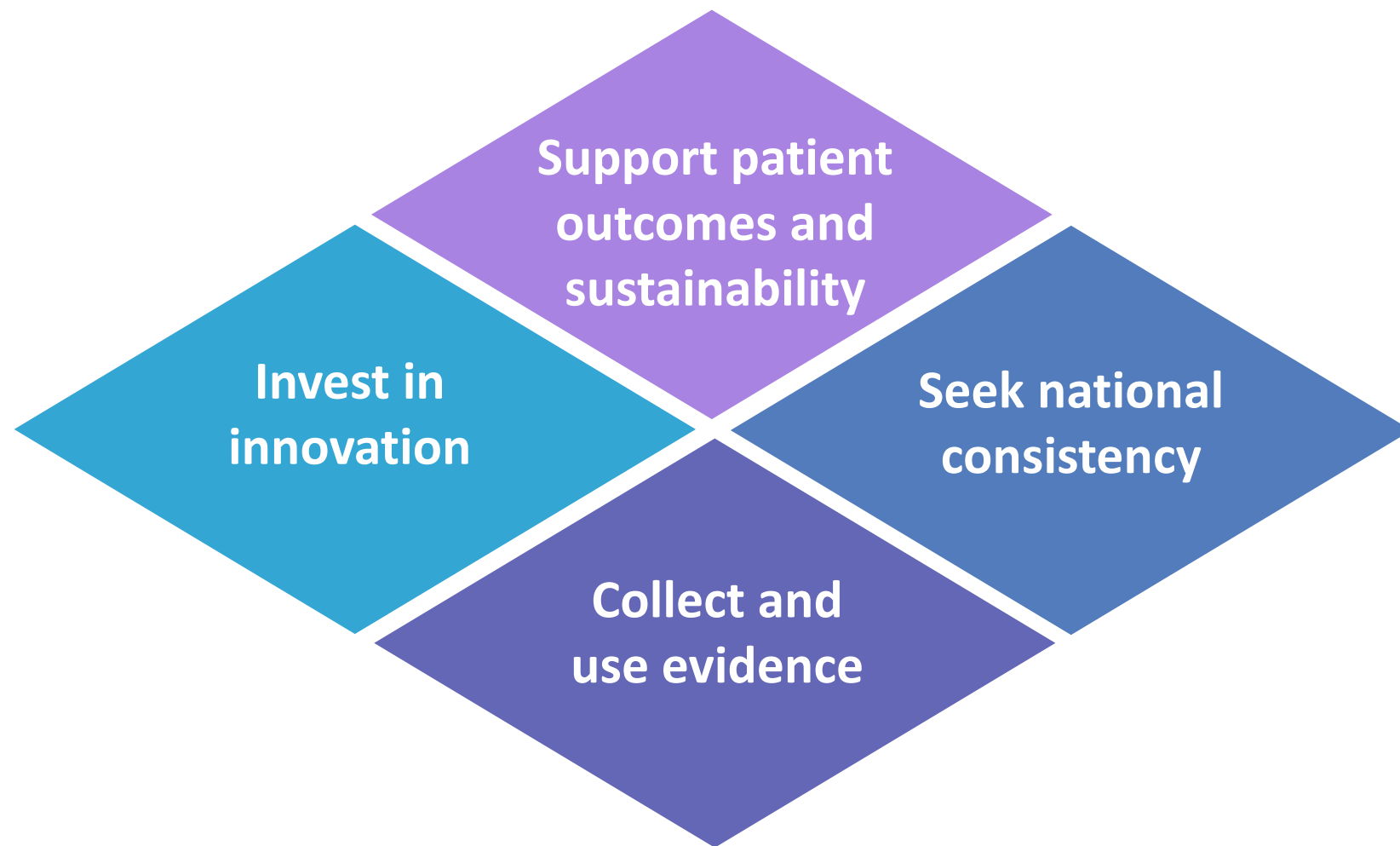
- **Pharmacare Act:** February 29, 2024 - introduced Bill C-64
- **Canadian Drug Agency (CDA):** December 18, 2023 – announced the creation of the CDA with an investment of \$89.5 million over 5 years, starting in 2024-25
- **National Strategy for Drugs for Rare Diseases (DRD):** March 2023 – launched with an investment of up to \$1.5B over 3 years
- **National Formulary Development:** June 2022 – a multidisciplinary national panel convened by the Canadian Agency for Drugs and Technology in Health (CADTH), at the request of Health Canada, recommended principles and a framework for developing a national formulary, along with a sample list of drugs for heart disease, diabetes, and mental health
- **Prince Edward Island:** August 2021 – announced that the federal government would partner with the province to improve affordable access to medications for Island residents with an investment of \$35M

National Pharmacare Legislation

- Introduction of Bill C-64, An Act respecting pharmacare on February 29, 2024
- Foundational legislation that lays out a collaborative path forward, with provinces and territories and Indigenous peoples, towards the step-by-step implementation of national universal pharmacare in Canada, guided by the Canada Health Act and the following principles:
 - accessibility
 - appropriate use
 - affordability
 - universal coverage
- Minister of Health will request the Canadian Drug Agency (CDA) to prepare a list of essential prescription drugs and related products to inform the development of a national formulary, and to develop a bulk purchasing strategy
- Requires the Minister, with support from the CDA, to publish a pan-Canadian strategy regarding the appropriate use of prescription medications
- Sets out the Government's commitment to long term funding, beginning with the funding announced for the National Strategy for Drugs for Rare Diseases

National Strategy for Drugs for Rare Diseases

- On March 22, 2023, the Government of Canada launched the National Strategy (up to \$1.5B / 3 years)
- The goal of the first three-year phase is to increase access to, and affordability of, effective drugs for rare diseases, which will contribute to improving the health of patients across Canada
- Lessons learned will be incorporated into future phases, staying aligned with the Government's broader pharmaceutical agenda



Alignment to Bill C-64 Principles:

- Access / Universal Coverage – different decisions across drug plans creates ‘postal code lottery’ for DRD coverage
- Affordability – high cost of drugs leaves patients financially vulnerable and unsustainable for drug plans
- Appropriate Use – inadequate real-world data on safety and effectiveness poses challenges to drug coverage and continued listing decisions

Elements of the National Strategy for Drugs for Rare Diseases

Pillar	Actions
Support Patient Outcomes and Sustainability	Working with provinces and territories through bilateral agreements, as well as Indigenous Services Canada's Non-Insured Health Benefits Program to: <ul style="list-style-type: none">• improve access to new and emerging drugs• support enhanced access to existing drugs• enhance screening and diagnostics activities
Seek National Consistency	Supporting the establishment of national governance structures, such as the stakeholder Implementation Advisory Group
Collect and Use Evidence	Partnering with the Canadian Agency for Drugs and Technologies in Health (CADTH) and the Canadian Institute for Health Information (CIHI) to improve the collection and use of evidence to support decision-making
Invest in Innovation	Advancing rare disease research with the Canadian Institutes of Health Research (CIHR) and a focus on developing better diagnostic tools and establishing a robust Canadian rare disease clinical trials network

Bilateral Agreements with Provinces and Territories

Objective: Increasing access to and affordability of promising and effective drugs for rare diseases

Progress:

- Since March 2023, mandated by Deputy Ministers, the ADM-level FPT Pharmaceuticals Executive Group has been:
 - 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
 - supporting the work of health system partners
- This is an ongoing collaborative process where the government is working closely with PTs to build towards a long-term approach

Next Steps: Finalizing bilateral agreements with individual Provinces and Territories

Governance and Implementation Advisory Group

Objective: a multi-stakeholder group that provides advice to Health Canada and acts as a forum to exchange information and best practices on DRD

Progress:

- The IAG launched with ~20 individuals drawn from a range of perspectives and roles, including patients, caregivers, clinicians, and industry
 - Co-Chairs: Dr. Gail Ouellette and Dr. Avram Denburg
- October 26, 2023 – First meeting with a focus on understanding mandate of the group
- December 18, 2023 – Second meeting with a focus on forward planning and committee priorities

Next Steps: Reviewing committee priorities and developing a forward agenda

Evidence and Research Partner Activities

Early Research and Development	Screening and Diagnostics	Regulatory Approval and HTA Decision-making	Monitoring Ongoing Use
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
<ul style="list-style-type: none"> • CIHR: Funding opportunities on pediatric clinical trial network, supporting clinical trial readiness • CADTH: Pipeline tracking and horizon scanning 	<ul style="list-style-type: none"> • CADTH: Newborn screening panel • CIHR: Funding opportunity on improving diagnosis 	<ul style="list-style-type: none"> • CADTH: Disease-based registries and real-world evidence • CADTH: Customized pharmaceutical work and assessment • CIHI: Public drug plan and formulary tool 	<ul style="list-style-type: none"> • CADTH/CIHI: Linked analysis for rare diseases • CIHR: Funding opportunity on enhancing the use of administrative data

Other Related GoC Initiatives

Innovation Investments

Biomanufacturing and Life Sciences Strategy (ISED)

pan-Canadian Genomics Strategy (ISED)

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

Regulatory Modernization

Pediatric Drug Action Plan (HC)

Regulatory Innovation Agenda (HC):

- Agile Licencing for Drugs
- Clinical Trials Modernization
- Enable Advanced Therapeutic Products

Drug Plan Decision-Making

Time Limited Recommendations (CADTH)

Temporary Access Program (pCPA)

Real World Data and Evidence

Real World Evidence (RWE) Steering Committee (HC, CADTH)

pan-Canadian Prescription Drug Data and Analytics Initiative (HC, CADTH, CIHI, Infoway, HDRN)

Next Steps

- Provinces and territories continuing to make progress toward signing bilateral agreements
- Delivery partners will continue to help improve the collection and use of evidence to support decision-making, and to advance rare disease research
- The Government will leverage lessons learned and a patient-centred approach to inform the way forward with National Strategy and national pharmacare activities

Questions?

Health Canada Drugs for Rare Diseases Directorate

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