Government of Canada / Gouvernement du Canada

National Strategy for Drugs for Rare Diseases

Discussion Guide for Stakeholder and Citizen Roundtables

Canada

Introduction

To help people across Canada living with a rare disease access the drugs they need, Budget 2019 proposed to invest up to $1 billion over two years, starting in 2022-23, with up to $500 million per year ongoing. The current mandate letter for the Minister of Health highlights the ongoing commitment to work with provinces, territories, and stakeholders to establish a national strategy for drugs for rare diseases.

From January to March 2021, Health Canada launched extensive stakeholder engagement and spoke with patients and caregivers, patient organizations, clinicians, pharmaceutical companies, insurance carriers, benefit advisors and associations representing businesses and employers, researchers, and other key stakeholder groups. A discussion paper outlining key issues and considerations guided this engagement, and a *What We Heard* Report was published in July 2021. In August 2021, Health Canada held follow up one-on-one meetings with stakeholders from multiple perspectives to continue the discussion on elements for the potential strategy. Concurrently, Health Canada has had ongoing dialogue with federal, provincial, and territorial drug plans through an executive table dedicated to the collaborative development of the strategy. In addition, discussions are ongoing with Indigenous partners and health system partners.

For additional background about access to treatment for rare diseases, please refer to notable reports, including the *Barriers to Access to Treatment and Drugs for Canadians Affected by Rare Diseases and Disorders* (Standing Committee of Health, 2019), and the *A Prescription for Canada: Achieving Pharmacare for All* (Advisory Council on the Implementation of National Pharmacare, 2019).

What We Heard: Summary of views from stakeholders and partners

There were wide ranging views from various stakeholders and partners. However, based on engagement to date, stakeholders and partners have identified some common themes for the national strategy for drugs for rare diseases. This short summary reflects areas of emerging consensus to plot a potential path forward.

Improving access and consistent decisions for rare disease drugs

- Focus on the value drugs for rare diseases can bring to patients such as improvements in health outcomes and enable access in ways that are sustainable.
- Recognize the need to build consistent coverage of rare disease drugs across Canada.
- Start somewhere and move forward with iterative implementation to leverage or build infrastructure, test and learn as experience is gained.
- The strategy should aim to expand coverage, while ensuring that existing coverage is not compromised.
- Recognize opportunities for access to promising treatments, despite clinical uncertainty, in cases where no treatments exist or outcomes could be lifesaving or transforming.
- Develop a national coordinating mechanism, advisory structures, and common decision-making tools to allow for centralized and evidence-informed decision-making for drugs for rare diseases and to enable better communication.

- Ensure transparency and clear communication in decision-making, including how to access drugs, eligibility criteria, rationale for coverage, timelines, and appeals.
- Ensure sustainability by encouraging the involvement of multiple payers in sharing risks and costs for drugs for rare diseases, to maximize inclusion of patients across Canada.

Coordination and collection of data

- Enhance coordination and avoid duplication throughout the pharmaceutical management system.
- Leverage and strengthen the current pharmaceutical management system to ensure there is meaningful evidence generation across the drug development lifecycle.
- Build opportunities for continuous review as real world drug safety and efficacy evidence accumulates, with modification of treatment initiation and discontinuation criteria on the basis of the evidence that is developed.
- Support the creation of an independent national data system or patient registries to monitor treatment outcomes and disease progression and improve our understanding of the value and clinical effectiveness of drugs for rare diseases.
- Enable international collaboration.

Evidence generation and capacity building

- Improve our knowledge of rare diseases and encourage the development of effective treatments by promoting research, clinical trials, and open science approaches.
- Be mindful of the financial impact the COVID-19 pandemic has had on employers/businesses, their employees, and the public health care system.
- Recognize the importance of partnership and including patients, caregivers, Indigenous representatives, clinicians, and other experts in future decision-making, including identifying meaningful outcome measures.
- Invest in infrastructure that could help address gaps when making reimbursement and formulary decisions.
- Support clinicians and researchers in building knowledge of rare diseases for evidence-informed care decisions.

A proposed draft framework for the strategy

Based on what we heard from stakeholders and partners, Health Canada has developed a draft framework for the national strategy. Health Canada is seeking feedback on this draft; accordingly, this guide provides discussion questions to gather input on specific elements and gaps.

A potential common vision for the national strategy:

Patients with rare diseases have improved access to effective drugs and better health outcomes.

Preliminary underlying principles to help guide the strategy:

- Patient-centered

- Transparent and accountable
- System alignment and sustainability
- Ethical
- Efficient and effective
- Evidence-informed
- Collaborative and inclusive
- Adaptive

The strategy would invest in activities across four strategic pillars:

1. Improve access to rare disease treatments and make it consistent across Canada
    - Adopt a common vision and commitment for the national strategy
    - Enhance coordination and shared decision-making around reimbursement decisions
2. Optimize, collect, and use evidence that meets the needs of decision-makers along the pharmaceutical management continuum and across the lifecycle of the drug
    - Develop data standards to enable the collection of data and building of knowledge of rare diseases and effective treatments
    - Leverage and analyze health data, including real-world evidence generation, to improve ongoing decision-making processes
3. Support optimal patient outcomes and sustainability of the Canadian health care system by ensuring spending on drugs for rare diseases brings value for money
    - Streamline process and efforts for DRD
    - Promote risk-sharing and equitable sharing of costs among payers
    - Explore innovative drug reimbursement models
4. Strengthen alignment of research and innovation systems with drugs for rare diseases access objectives
    - Support research by building on Canada's rare disease research capacity
    - Support access to data that can spur clinical trials

Discussion questions:

1. What are your initial reactions on the principles and pillars as described in the draft framework for the strategy?
2. What are your thoughts on the potential activities proposed to support the strategy? Which activities would you prioritize?
3. Where could you see yourself, your organization, or your industry contributing or playing a role to support and advance the draft framework for the national strategy?

An iterative approach to implementation

Stakeholders have stressed the importance of an iterative approach to strategy implementation allowing for continuous learning and agility to evaluate, adapt, and improve. Potential strategy activities could start with the building of a foundational phase and a corresponding set of activities. Activities in the foundational phase could help address immediate and common pressures and yield several outputs to help shape future decisions and adaptations. Potential activities would involve action across drug coverage, governance, and evidence generation elements.

Drug coverage

Through the strategy, the federal government could work with partners and payers to improving accessibility of rare disease drugs by supporting coverage for select drugs of common concern. This initial list of drugs could form the basis to work toward a formulary for drugs for rare diseases.

Example activities:

- Establishing an initial set of drugs for rare diseases
- Adopting common principles and developing a decision-making framework to assess and manage a formulary of Drugs for Rare Diseases
- Conducting horizon scanning and planning for the pipeline of rare disease drugs

Governance

To ensure national consistency, decision-making and advisory structures would need to be in place to manage and improve the list of select drugs and evidence generation activities overtime. Since there are already existing players and structures in the pharmaceutical management system in Canada, any governance mechanisms for the strategy should leverage existing structures as much as possible.

Example activities:

- Building fit-for-purpose advisory committee(s) and working groups that include a range of partners and stakeholder groups (e.g., patients, NGOs, industry, clinicians)
- Exploring the feasibility of sustainable and innovative cost-sharing/risk-sharing models with multiple payers
- Building relationships with international partners and networks, and fostering information sharing among health system partners

Evidence generation infrastructure

Invest in infrastructure that could support evidence generation to improve drug coverage decision-making over time and across the drug lifecycle. This could include investment in real-world data and evidence activities (e.g., creation or adaptation of patient registries), support for a data framework / data standards, and where needed data-sharing agreements. Investments could also support improved knowledge of rare diseases and drugs for rare diseases and patient outcomes.

Example activities:

- Developing a data governance framework
- Creating a plan for a future national data system, including assessing, piloting, and enhancing existing databases and patient registries
- Engaging with Indigenous Peoples for input and collaboration on governance and infrastructure requirements, including data governance

Discussion questions:

4. How do we ensure collaborative decision-making that is inclusive of multiple perspectives?
5. What types of advisory committees / working groups would be key for successful implementation, while ensuring efforts are not duplicated?
6. What would be the first steps to planning a national data system / patient registry to support the implementation of the strategy?

Next Steps

The engagement guided by this discussion document will inform the finalization of the national strategy as Health Canada moves toward the launch of the strategy in 2022.