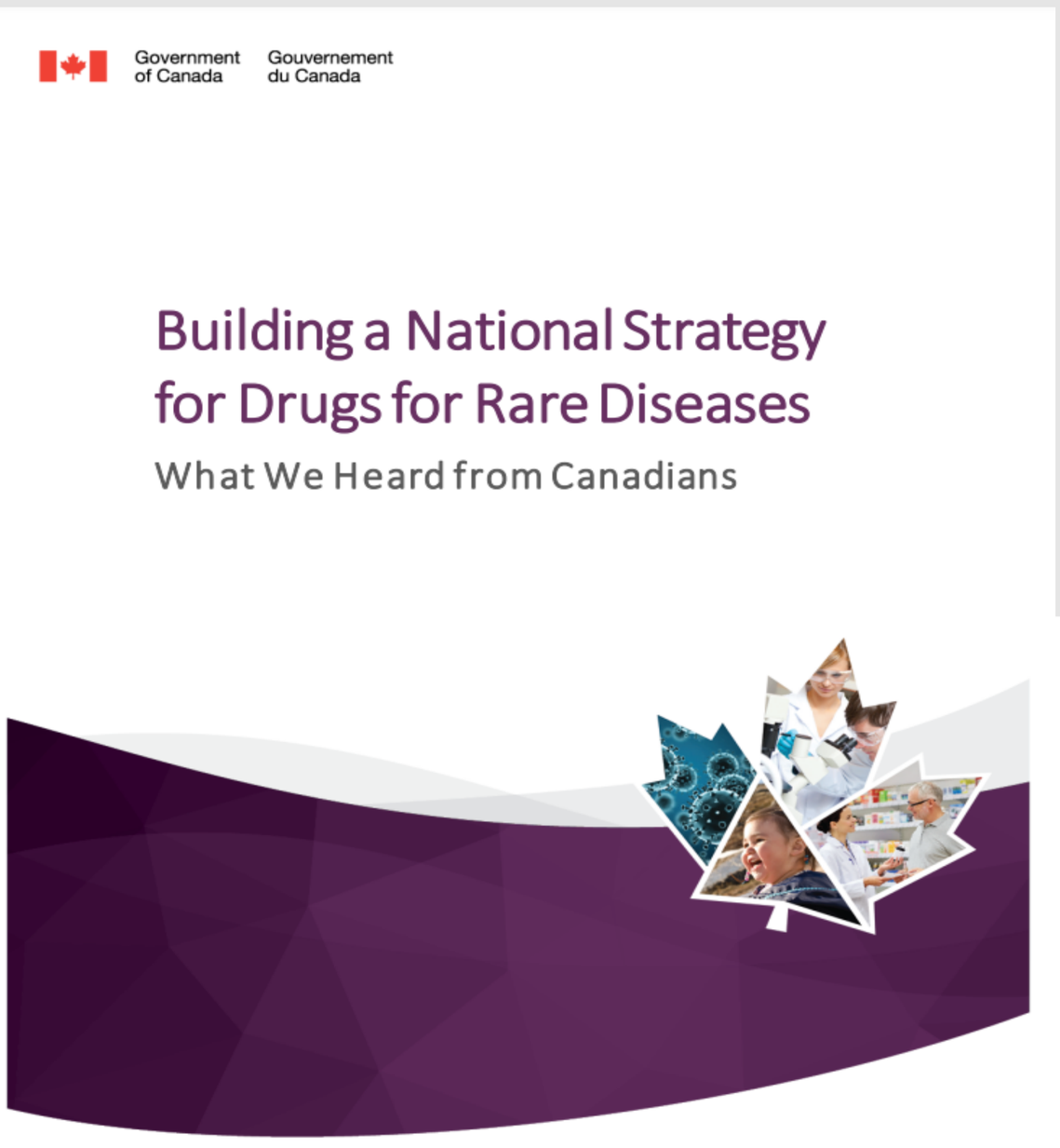
Health Canada's National Strategy for Drugs for Rare Diseases



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Disclaimer

This technical report is intended to provide information to quality and regulatory professionals on the April 2022 consultation by Health Canada in regard to the draft framework for the national strategy approach for drugs for rare diseases. This technical report should be read in conjunction with the relevant laws, regulations, and guidance's that apply to your situation.

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Drugs for Rare Diseases

National Strategy for Drugs for Rare Diseases

Canada's Minister of Health is continuing to work with provinces, territories, and stakeholders to establish a single national framework for drugs for rare diseases (orphan drugs). Since 2021, Health Canada has spoken with patients, caregivers, patient organizations, clinicians, pharmaceutical companies, insurance carriers, benefit advisors and associations representing businesses and employers, researchers, and other key stakeholder groups to build a formalized and directed approach to advancing Canada’s approach on drugs for rare diseases. Many gaps and opportunities were identified, and the next steps will focus on improving access and consistency by creating a more unified and integrated approach. To aid in this journey, Budget aims to invest up to $1 billion over two years, starting in 2022-23, with up to $500 million per year ongoing.

Did You Know?

Drugs to treat rare diseases account for 30% to 40% of all new drugs approved in Canada.

In 2017, 16 of the 36 brand-name drugs approved by Health Canada are classified as drugs for rare diseases in Europe or the United States.

Current list prices for drugs for rare diseases range from $0.5 million to $4.9 million per person per year. Since these conditions are chronic, treating one patient for 10 years can cost from $1 million to $49 million.

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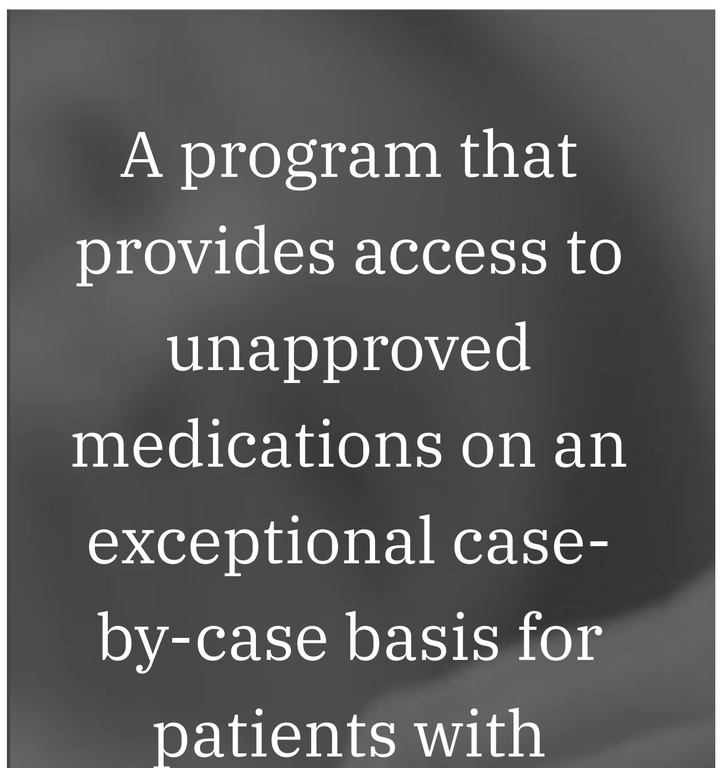
Regulatory Pathways for Drugs for Rare Diseases

There are three main regulatory avenues that support Canadians' access to drugs for rare diseases:

1 Accelerated or Priority Status Drug Approvals

A process designed to accelerate the approval of drugs that are intended to treat serious or life-threatening diseases, including rare diseases.

2 The Special Access Program



A program that provides access to unapproved medications on an exceptional case-by-case basis for patients with serious or life-threatening conditions when conventional treatments have failed, is unsuitable, or unavailable.

3 Clinical Trials

Drug manufacturers advise on the design of clinical trials in small patient populations in a clinical trial database which helps patients and primary care providers find suitable clinical trials in which to participate.

Gaps in Current Regulatory Pathways

There are 6 main barriers to access treatment and drugs for Canadians affected by rare diseases and disorders, whereby three are in regard to the main regulatory avenues discussed above that support Canadians' access to drugs for rare diseases:

- **Priority Status Drug Approval for Drugs for Rare Diseases:**
  The review process for accelerated or priority approval does not take into consideration all the different types of data available to evaluate the efficacy of drugs for rare diseases, such as quality-of-life data, impact on daily living, cost-analysis and meta-analysis data. Flexibility to this approach via more comprehensive reviews of scientific evidence would help facilitate greater approval rates and thus access.

- **Special Access Programme:**
  Access granted through this program is typically limited to 3-6 months before requiring reapplication. Proactive measures should be taken to ensure patients have access to drugs through this program for extended periods, given that the majority of these rare diseases and disorders are permanent and stable conditions. Ideally, approvals should remain in place until a doctor revokes the approval or there is a significant change to the patient's condition.

- **Accessing Drugs for Rare Diseases Through Clinical Trials:**
  Patients are frequently denied continued access to treatment once a clinical trial ends. To address this issue, it is suggested that Health Canada take on the US FDA's approach to automatically allow an open-label extension at the end of a trial, permitting that there are no safety concerns during the trial.

Current Gaps Cont'd

Gaps with Accessibility of Drugs for Rare Diseases

Prices Of Drugs for Rare Diseases:

Orphan drugs have increasingly high prices due to the costs and risks, such as lack of information about rare diseases which lead to a high failure rate of treatments in clinical trials, associated with their research and development (R&D). However, there is lack of transparency by manufacturers to demonstrate a detailed breakdown of the costs associated with the R&D of these drugs.

- As a response, three main amendments to the CG *Patented Medicines Regulations* are being proposed:

1. Expanding the list of countries used for price comparison to include countries that reflect drug prices that are closer to the OECD average and that have health care systems/economies that are similar to Canada's. The proposed list includes Australia, Belgium, France, Germany, Italy, Japan, the Netherlands, Norway, South Korea, Spain, Sweden, and the United Kingdom.
2. Understanding the impact the drug of interest has on a patient's length or quality of life and the potential market share of the new drug, to determine whether a price is excessive relative to the Gross Domestic Product.
3. Requiring manufacturers to provide the PMPRB with information regarding confidential price rebates that they offer to public and private drug coverage payers, to better understand the market drug price.

Reimbursement of Rare Disease Drug Costs Through Federal Provincial and Territorial Public Drug Coverage Plans:

Varying reimbursement pathways across Canada create inequitable access to drugs for rare diseases to patients that reside in different provincial or territorial areas, putting emphasis on the need for a national approach.

Access to Early Diagnosis of Rare Diseases:

Lack of rapid access to genetic testing which allows for patients to take proactive measures on their health, avoiding unnecessary investigations and procedures that can cost the health care system upwards of US$8,000 per patient.

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Potential Next Steps

Improving Access + Consistent Decisions For Rare Disease Drugs

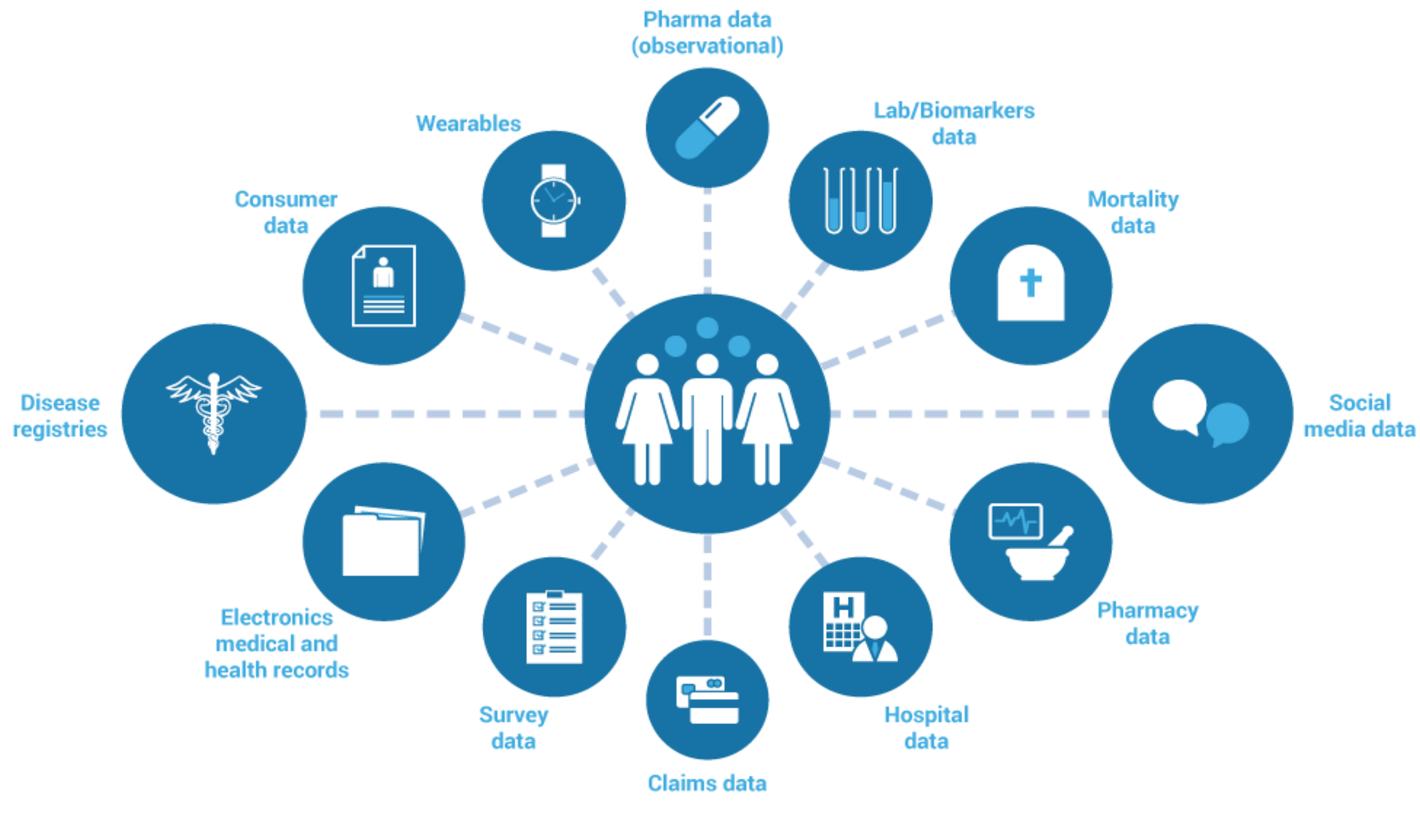
In January 2021, Health Canada launched a national online engagement to better understand how to improve access and consistency for rare disease drugs. The following key issues and recommendations were posed, reflecting a potential path forward:

1

Ranking as most important, is the need to develop a single national framework with advisory structures, and common decision-making tools to allow for centralized and consistent coverage of rare disease drugs across Canada. The strategy should aim to expand coverage while ensuring that existing coverage is not compromised.

2

The second most important element is the need to expand the acceptability of evidence used during regulatory decision-making, to include real-world evidence such as database from other trusted regulatory authorities, national databases or international evidence-sharing networks, and patient registries that monitor patients' treatment outcomes. To improve this evidence-based approach the government of Canada must promote research, clinical trials, and open science approaches.



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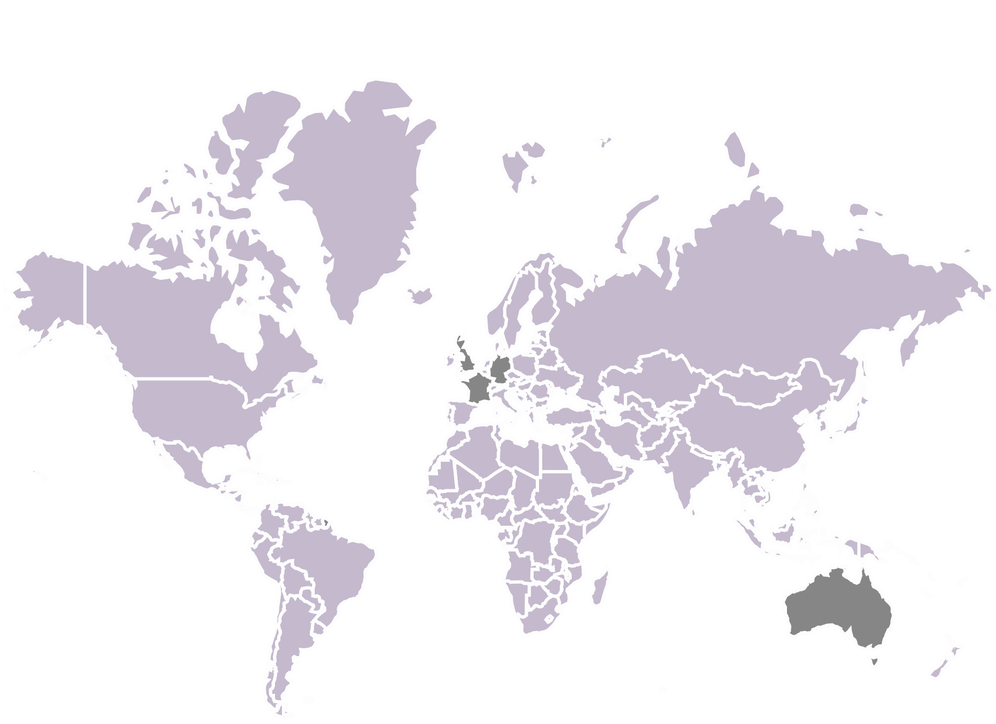
Potential Next Steps

Other Important Aspects

Recognizing the importance of partnership and including patients, caregivers, clinicians, and Indigenous representatives (to facilitate a distinctions-based approach between First Nations, Inuit, and Métis) in future decision-making.



Enabling international collaboration with jurisdictions including France, Germany, the U.K., and Australia to enhance coordination.

Implementing proposed changes to the *Patented Medicines Regulations* to address high drug prices in Canada.

Working towards ensuring greater transparency and information sharing throughout the life cycle of drugs for rare diseases to ensure timely access for health care providers and patients. This extends into ensuring transparency in decisions on an individual patient's eligibility for rare-disease drug funding, including an appeal process where patients denied funding have an opportunity to challenge the decision.

Establishing universal definitions of "rare diseases" and more specific guidelines on what constitutes "benefits" and "improvements" for assessing rare-disease treatments. The definition should include elements like disease severity, burden, and lack of available treatments rather than being based on numerical value (i.e., 1 in 2,000 people).

Proposed Draft - April 2022 Roundtable

Based on the key concerns and recommendations, Health Canada has developed a draft framework for the national strategy. On April 25, 2022, a roundtable discussion for citizens was held to help further inform the development and implementation of the national strategy for drugs for rare diseases and to seek feedback on the proposed draft framework.

The 4 pillars of the draft include:

1. Improving access and consistent decisions across Canada for rare disease treatments.
2. Strengthening alignment of research and innovation systems with drugs for rare diseases access objectives.
3. Supporting optimal patient outcomes and sustainability of the Canadian health care system by ensuring spending on drugs for rare diseases brings value for money.
4. Optimizing, gathering, and using evidence that meets the needs of decision-makers along the pharmaceutical management continuum and across the lifecycle of the drug.

Aside from seeking feedback on the draft framework, the roundtable aimed to explore how stakeholders can help achieve the objectives of the national strategy, implementation considerations for governance and data infrastructure, and stakeholder preferences for implementation involvement and engagement.

As Health Canada progresses toward the launch of the strategy in 2022, the interaction informed by this roundtable discussion will shape the finalization of the national strategy.

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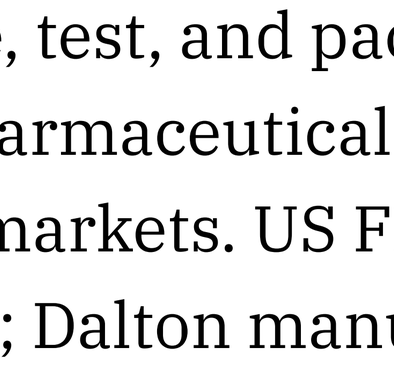

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