National Strategy for Drugs for Rare Diseases

Health Canada Santé Canada

Canada

Presentation Outline

1. Context for the National Strategy for Drugs for Rare Diseases
2. Overview of the Strategy to date
3. Key aspects of the Bilateral Agreements

National Strategy for Drugs for Rare Diseases

- In March 2023, the Government of Canada launched the National Strategy for Drugs for Rare Diseases.
- The goal of this Strategy is to increase access to, and the affordability of, effective drugs for the treatment of rare diseases.
- Additionally, the Strategy will support enhanced access to existing drugs, and early diagnosis and screening for rare diseases.

Four Key Pillars of the Strategy

- The government of Canada has committed up to $1.5 billion over 3 years, to support the following:
    1. Support patient outcomes and sustainability
    2. Invest in innovation
    3. Seek national consistency
    4. Collect and use evidence

Support Patient Outcomes & Sustainability

- Up to $1.4 billion over three years to provinces and territories through bilateral agreements to
  - Improve access to new DRD.
  - Improve screening and diagnostics.
  - Improve coverage for existing DRD.
  - Support for decision-making within the pharmaceutical management system, recognizing unique challenges related to DRD.

- Similarly, to further support eligible First Nations and Inuit patients living with rare diseases, $33 million (over three years) to Indigenous Services Canada’s Non-Insured Health Benefits Program.

Improving Access to New DRD – Common Set

- Through the signing of bilateral agreements, provinces and territories are committing to making available elected drugs from a common set of new DRD
  - The Common Set is a result of over a year of development with provinces and territories to determine a small set of new drugs to be cost-shared and covered in a consistent way across the country, for the benefit of patients.
  - This list of drugs is designed to further the development, collection, evaluation and use of real-world-data and evidence in decision making about the listing and reimbursement of rare disease drugs.
  - This process is designed to work within the existing pharmaceutical management system within Canada.
  - Drugs will only be listed on Health Canada's website once the pCPA concludes price negotiations for each drug on the Common Set.

Commitment to Evidence Collection Projects

- Provinces and territories who sign a bilateral agreement agree to work with Canada to design and implement evidence collection projects.
  - This work will inform the future collection and use of real-world data and evidence for decision making about all new rare disease drugs and has the potential for system transformation.
  - This work is complementary to related projects currently underway through Canada's Drug Agency and Canadian Institute of Health Information.

Improving Screening & Diagnostics

- Provinces and territories will identify activities to advance this stream of work, with optional input from the supporting elements of the Strategy such as
  - Newborn Screening Panel (Canada's Drug Agency) – Clinician experts (guidance report to be published by end of March 2025).
  - Implementation Advisory Group – Multi-stakeholder group with a national perspective on possible common objectives, decision metrics, and synergies.

Improving Coverage of Existing DRD

- Provinces and territories must use unused funds to improve coverage for other available drugs for rare diseases.
- This provides provinces and territories with the flexibility to address the unique circumstances within their jurisdiction, which includes disease prevalence.

Specific Aspects of the Agreement – Use of Funds

- **Common Set:** Bilateral agreements specify that a minimum of 50% of total federal funding will be used to enhance access to drugs on the Common Set.
  - To allow for build-up over the first two years, there will be flexibility in Years 1 and 2 to use funds towards improving access to other new DRD that are not on the common set.
  - Year 3, provinces and territories must meet the 50% target, and are expected to cost share Eligible Expenditures.
- **Screening & Diagnostics:** Bilateral agreements specify that 10% of total federal funding will be used for improvements to screening and diagnostics.
- **Other Drugs:** Funds not utilized for the Common Set or Screening and Diagnostics must be used to incrementally improve coverage for new DRD (not in common set) and/or other existing DRD.

British Columbia DRD Agreement

- British Columbia will receive $194 million over 3 years under the National Strategy.
- By entering into the bilateral agreement, the Government of British Columbia has confirmed that it is electing to make Poteligeo and Oxlumo available to its residents.
- British Columbia will also support evidence collection and generation and data collection and sharing, as appropriate, for drugs funded through this agreement.
- British Columbia is committing to work with Canada and other provinces and territories developing and implementing a plan for improving screening and diagnostics for rare diseases.

Innovation, National Consistency and Evidence

- **Invest in Innovation** - $32 million over five years to the Canadian Institutes of Health Research to advance rare disease research with a focus on developing better diagnostic tools and establishing a robust Canadian rare disease clinical trials network.
- **Seek National Consistency** - $16 million over three years to support the establishment of national governance structures, such as a Health Canada secretariat and a stakeholder Implementation Advisory Group, to support the implementation of the Strategy.
- **Collect & Use Evidence** - $20 million over three years to the Canadian Drug Agency and the Canadian Institute for Health Information to improve the collection and use of evidence to support decision-making.