National Strategy for Drugs for Rare Diseases

Jennifer Grandy – Director, Governance and Bilateral Agreements,
Drugs for Rare Diseases Directorate,
Office of Pharmaceutical Management Strategies, Health Policy Branch

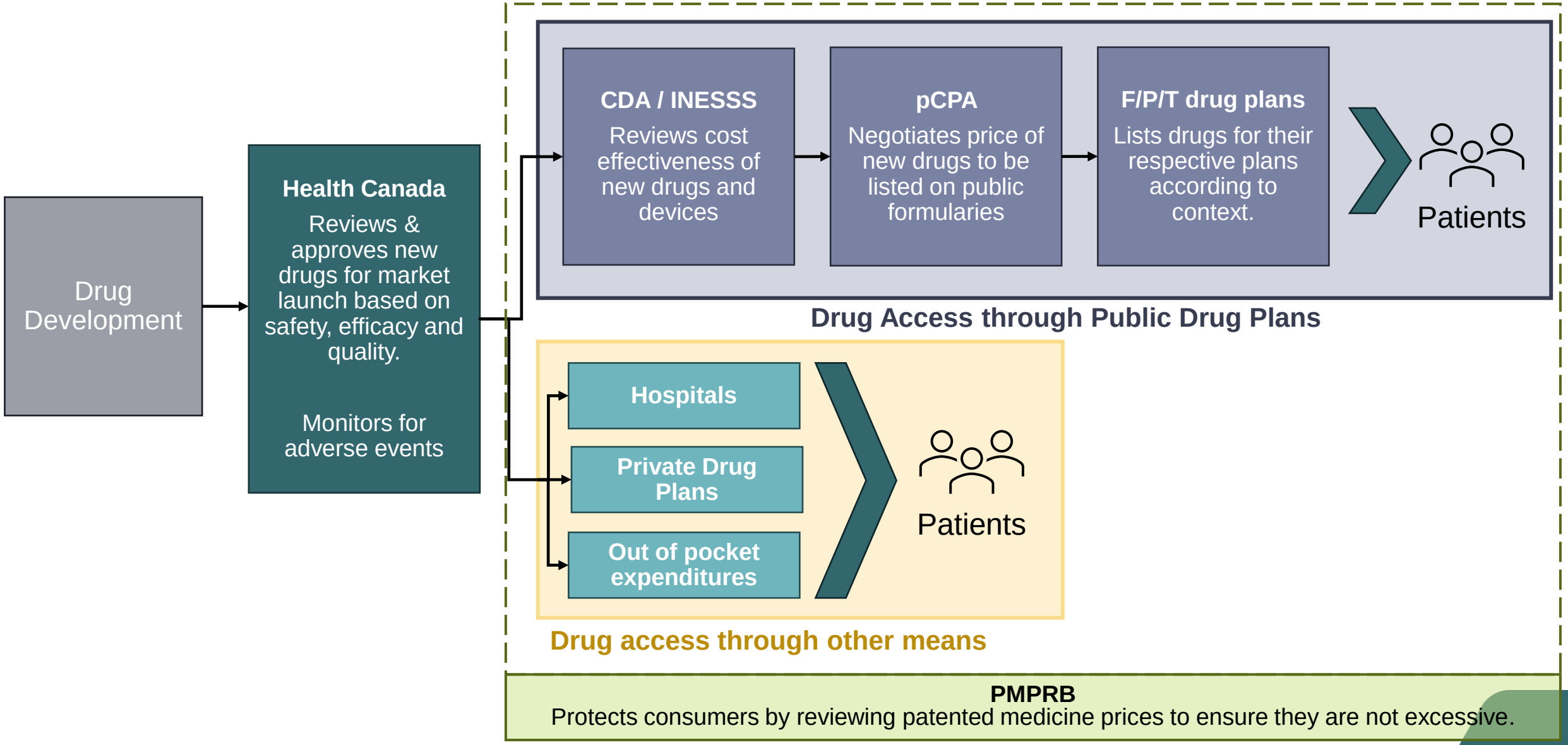
Health Canada Santé Canada

Canada

Background and Context

- Rare diseases are life-threatening, seriously debilitating, often genetic conditions, with onset either at birth or early childhood – an estimated **1 out of every 12 Canadians** has one of between 6,000 to 8,000 rare diseases
- There is significant **unmet clinical need** due to few treatment options, and high demand for available therapies
- Therapies **target small populations**, meaning randomized controlled trials are very difficult, and there is often **high uncertainty** about the effectiveness and value when making reimbursement decisions, which can delay access
- Accelerated pace of innovation in specialty drugs is a **key cost driver of drug spending**, creating sustainability challenges for both public and private drug plans
- **Patients experience variability in access** based on where they live, despite pan-Canadian efforts to align coverage

Canada's Prescription Drug Access and Reimbursement Chain



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 is supporting early diagnosis and screening for rare diseases and various foundational infrastructure to support ongoing decision-making related to the pharmaceutical system

Four Key Pillars of the Strategy

The Government of Canada committed up to $1.5 billion over 3 years, to support four pillars:

1. Bilateral funding agreements
2. Governance and stakeholders
3. Investing in innovation
4. Collecting and using evidence

Lessons learned will be incorporated into future phases, staying aligned with the Government’s broader pharmaceutical agenda

Bilateral Funding Agreements: Progress

- $1.4B towards 3-year bilateral agreements signed with all 13 provinces and territories, supporting public drug plans to:
  - Improve access and coverage to new and existing DRD
  - Improve screening and diagnostics
  - Build support for decision-making within the pharmaceutical management system, recognizing unique challenges related to DRD
- The eligible use of funding in the agreements changes over the course of the 3 years:
  - In years 1 and 2 there is significant flexibility on where the money is allocated
  - In year 3, 50% of funds must be used on drugs on the common list of DRD, and 10% must be allocated to activities related to screening and diagnostics
- Similar supports for eligible First Nations and Inuit patients living with rare diseases, via Indigenous Services Canada’s Non-Insured Health Benefits Program

Bilateral Funding Agreements: Next Steps

- With agreements now in place, the Government of Canada is working with provinces and territories on collective planning on screening and diagnostics and real-world evidence
- Through the agreements, Health Canada is providing funding for provinces and territories to identify, collaborate, and invest in activities to advance screening and diagnostics
- PTs have also committed to work together on pilot projects to further the development, collection, evaluation, and use of real-world data and evidence in decision making relevant for the listing and reimbursement of rare disease drugs
- All activities are designed to work within the existing pharmaceutical management system within Canada and are complementary to work under other pillars

Governance and Stakeholders: Progress

- The Pharmaceutical Executive Group (PEG):
  - Launched in December 2020 to inform the development of the Strategy and renewed in March 2023 to guide Strategy implementation
  - Representation from all provincial and territorial drug plans (QC is an observer), and Indigenous Services Canada Non-Insured Health Benefits Programs.
- The Implementation Advisory Group (IAG):
  - Launched in October 2023 with ~20 individuals drawn from a range of perspectives and roles, including patients, caregivers, clinicians, and continues dialogue that began with broad consultations on the Strategy in 2021
  - Multi-stakeholder group that provides advice to Health Canada and acts as a forum to exchange information and best practices on DRD
- Other ongoing stakeholder engagement as part of implementation of the Strategy

Governance and Stakeholders: Next Steps

Health Canada will:

- Continue to engage through PEG to discuss pan-Canadian best practices and collaboration and future opportunities
  - FPT tables have been established to collaboratively workplan and share best practices on real-world evidence and screening and diagnostics
- Continue engaging the IAG on implementation considerations and advice for future phases
- Engage with stakeholders on early learnings and future phases of the Strategy

Investing in Innovation: Progress

The Government of Canada has invested in several funding opportunities through the Canadian Institutes of Health Research (CIHR) to advance rare disease research:

- Supporting a **pediatric clinical trial and treatment network (RareKidsCAN).** Recipient: the Maternal Infant Child and Youth Research Network – MICYRN
- Funding two grants specific to **determining the prevalence, direct cost, and burden of rare diseases** in the Canadian health care systems. Recipients: researchers at the University of Calgary and Hospital for Sick Children
- Funding three grants focused on **improving the use of genomic testing and determining the best pathway to diagnosis.** Recipients: researchers at the Children's Hospital of Eastern Ontario + Ontario Research Institute, Hospital for Sick Children, and Queen's University
- Supporting three grants focused on increasing the **readiness for clinical trials specific to gene therapies** for rare diseases (in partnership with the National Research Council of Canada). Recipients: researchers at McGill University, Université Laval, and the Research Institute of the McGill University Health Centre

Investing in Innovation: Next Steps

With funding now in place for these research projects, Health Canada will:

- Focus on aligning the work of these teams with other work under the Strategy, such as work by Canada's Drug Agency and the Canadian Institute for Health Information
- Collaborate with key networks to explore how best to coordinate rare disease research across Canada
- Explore future activities to continue supporting a research agenda

Collecting and Using Evidence: Progress

- In addition to their inclusion in the bilateral agreements, evidence collection and use are included across other pillars in the National Strategy

- The Strategy is investing in projects through Canada's Drug Agency (CDA-AMC) and the Canadian Institute for Health Information (CIHI) to:
  - Consolidate information for PTs on emerging DRD entering Canada
  - Build guidance for consistent rare disease screening for use by PTs
  - Conduct customized health technology assessments as PTs request
  - Build standards, best practices, and capacity among rare disease registries and explore data sources and analytic work specific to DRD outcomes to support use in PT decision making on listing and reimbursement

Collecting and Using Evidence: Next Steps

- CIHI and CDA-AMC will:
  - Continue maintaining and expanding platforms to monitor DRD pipeline
  - Explore how to pilot and test processes recommended by expert panel to guide consistent rare disease screening in Canada
  - Conduct customized health technology assessments as PTs request
  - Maintain registry inventory, build additional standards, and launch additional funding opportunities to build capacity among rare disease registries
- Work by CIHI and CDA-AMC on the collection and use of evidence is also feeding into collaborative FPT work to further the development, collection, evaluation, and use of real-world data and evidence in decision making relevant for the listing and reimbursement of rare disease drugs

Looking Forward

- Lessons learned in Phase I will be incorporated into future phases, staying aligned with the Government's broader pharmaceutical agenda including regulatory work
- Health Canada will explore renewed strategic engagement to discuss future activities under the Strategy
- Health Canada will continue convening PTs to discuss potential collaboration and collective work
- Strategy partners will progress additional work to support collection and use of evidence