Health Canada Santé Canada

Canada

Implementation of the Drugs for Rare Diseases Strategy

Canadian Organization for Rare Disorders (CORD) – Rare Disease Day 2024 Summit

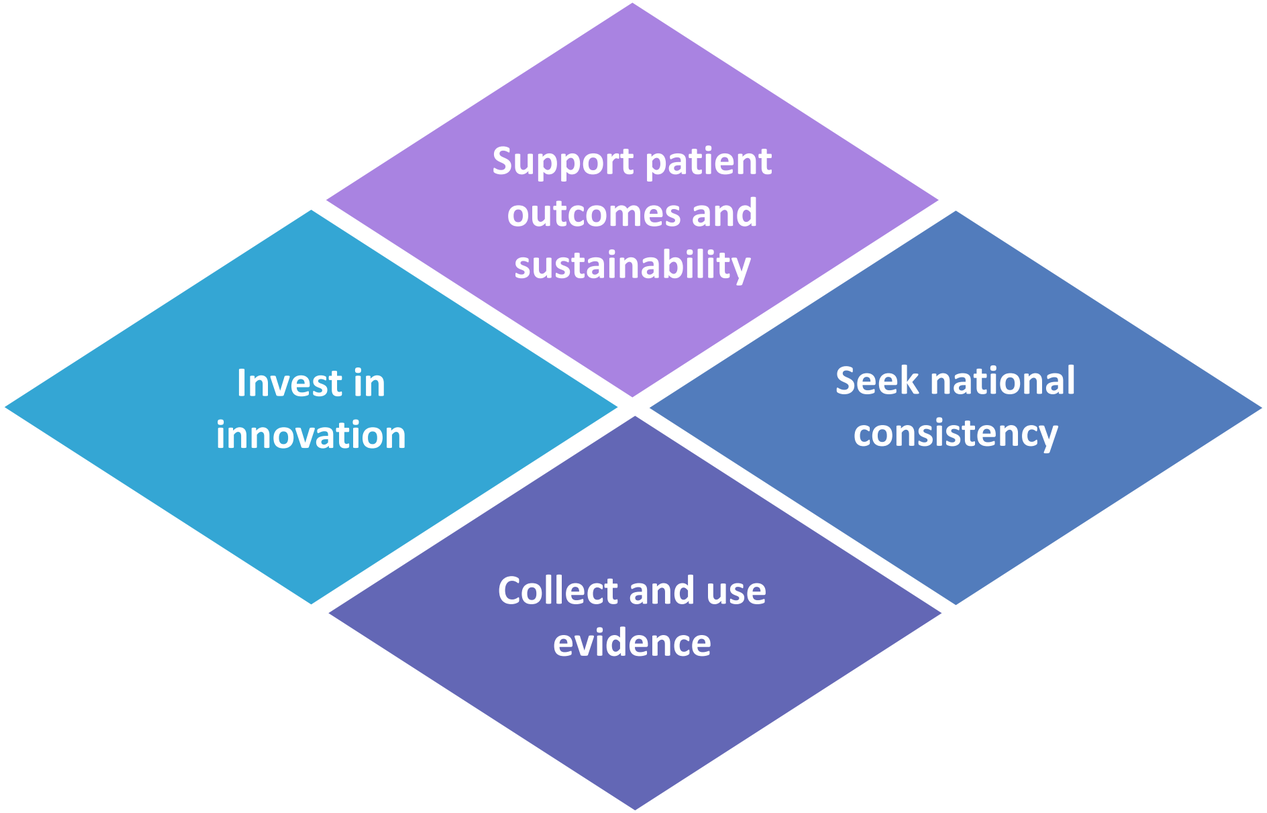
February 28th, 2024

Daniel MacDonald, Director General of the Office of Pharmaceutical Management Strategies and Drugs for Rare Diseases

Strategic Policy Branch

National Strategy for Drugs for Rare Diseases

- On March 22, 2023, the Government of Canada launched the National Strategy
- 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



Elements of the National Strategy for Drugs for Rare Diseases

Pillar	Actions
Support Patient Outcomes and Sustainability	Up to $1.4B to work with provinces and territories on bilateral agreements to: • improve access to new and emerging drugs • support enhanced access to existing drugs • enhance screening and diagnostics activities $33M for Indigenous Services Canada's Non-Insured Health Benefits Program
Seek National Consistency	$16M for national governance structures: • Health Canada secretariat • Implementation Advisory Group
Collect and Use Evidence	$20M for Canadian Agency for Drugs and Technologies in Health (CADTH) and Canadian Institute for Health Information (CIHI) to improve the collection and use of evidence to support decision-making
Invest in Innovation	$32M for Canadian Institutes of Health Research (CIHR) rare disease research • developing better diagnostic tools • establishing a robust Canadian rare disease clinical trials network

Roadmap for Today's Discussion

Support Patient Outcomes and Sustainability	Seek National Consistency	Collect and Use Evidence	Invest in Innovation
• Implementing Bilateral Agreements between Health Canada and Provinces and Territories • Funding to Indigenous Services Canada's Non-Insured Health Benefits Program	• FPT Pharmaceutical Executive Group (PEG) • Health Canada Secretariat • Implementation Advisory Group (IAG)	**CADTH** • Disease based Registries and Real World Evidence • Newborn Screening Panel • DRD Pipeline Repository • Customized Pharmaceutical Work on Request **CIHI** • Linked Analysis for Rare Disease (w **CADTH**) • DRD Data Landscape • Pharmaceutical Data Web Tool	**CIHR** • Improving Health and Administrative Data and Monitoring for Rare Diseases • Pediatric Rare Disease Clinical Network • Improving Diagnosis for Rare Disease Patients • Bringing Rare Disease Gene Therapies to Clinical Trial Readiness

Implementation Advisory Group (IAG)

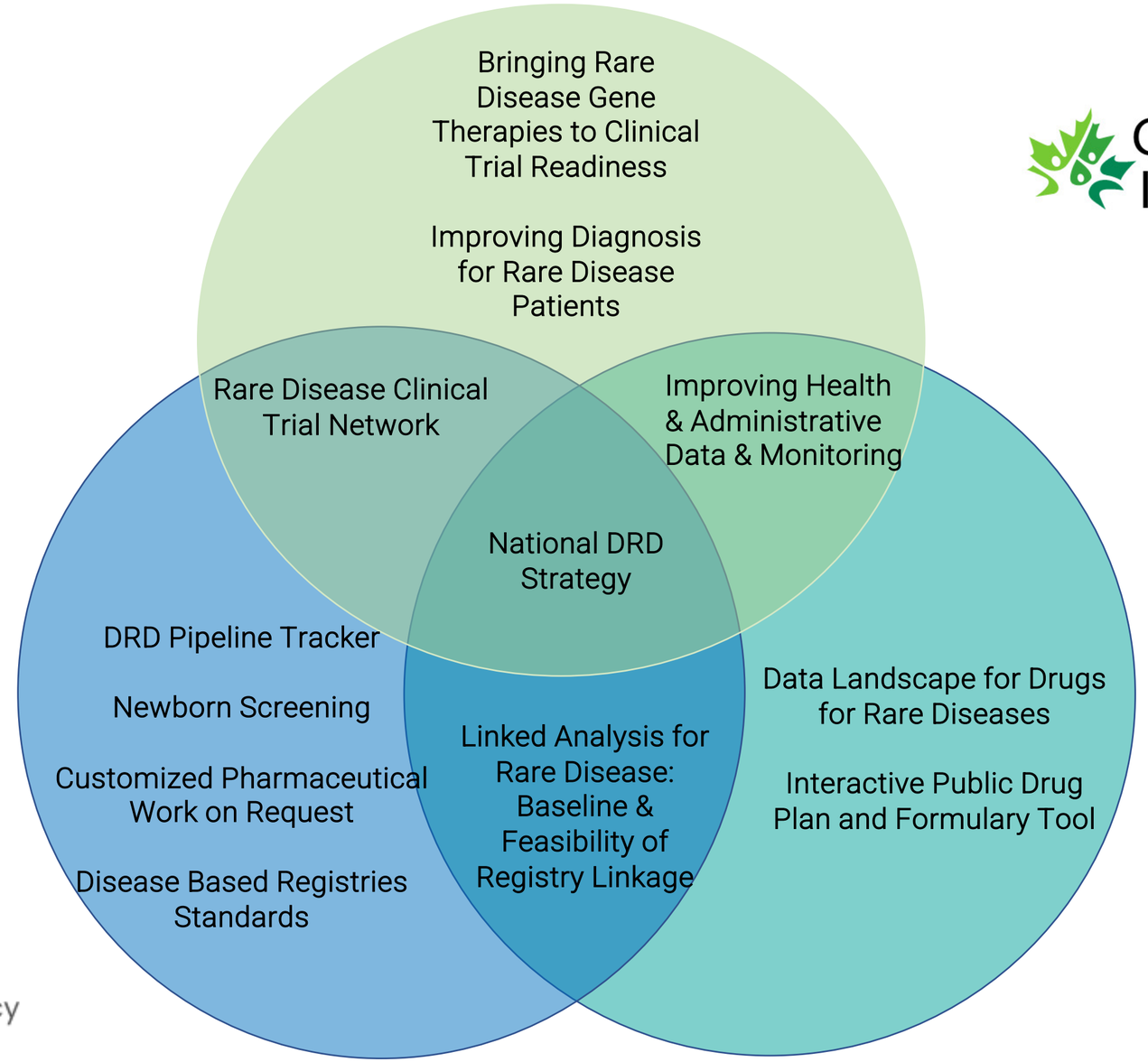
Objective: a multi-stakeholder group that provides advice to Health Canada and acts as a forum to exchange information and best practices on DRD

Progress:

- The IAG launched in October 2023 with ~20 individuals drawn from a range of perspectives and roles, including patients, caregivers, clinicians, and industry:
  - Current co-chairs: Dr. Gail Ouellette and Dr. Avram Denburg
  - Members: John Adams, Dr. Shawn Bugden, Andrew Casey, Dr. Pranesh Chakraborty, Dr. Shelita Dattani, Stephen Frank, Declan Hamill, Dr. Bashir Jiwani, Joanne Jung, Dr. Aneal Khan, Elizabeth Kwan, Erin Little, Bob McLay, Dr. Sandra Sirrs, Gary Walters, Dr. Durhane Wong-Rieger
- October 26, December 18, February 22 – Initial meetings with a focus on understanding mandate of the group, and forward planning and committee priorities

Next Steps: Reviewing committee priorities and developing a forward agenda

Delivery Partner Projects



CIHR IRSC Canadian Institutes of Health Research / Instituts de recherche en santé du Canada

cadth Canada's Drug and Health Technology Agency

CIHI