National Strategy for Drugs for Rare Diseases - Update

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Health Canada Santé Canada

Canada

Screening and Diagnosis

- The Strategy is supporting screening and diagnosis to make sure that people can identify options quickly for conditions where drugs are available

- This includes a focus on identifying best practices and lessons learned, developing guidance for building consistency, and resources for making improvements
  - Canada's Drug Agency (CDA-AMC) launched a panel made up of experts to assess best practices and lessons and develop guidance on newborn screening across Canada – they have published a discussion paper for public consultation and a final report on improving pan-Canadian consistency in newborn screening is expected by March 2025
  - The Strategy, through the Canadian Institutes for Health Research (CIHR), is providing funding for projects focused on developing lessons on how to better integrate genomic diagnostic tools for rare diseases – three team grants have been awarded and learning will help to inform future phases of the Strategy
  - Through bilateral agreements, Health Canada is providing funding for provinces and territories to identify, collaborate, and invest in activities to advance screening and diagnostics

Access to Treatments

- The Strategy is working to ensure that people have access to drugs and innovative treatments by investing in innovation, research, and clinical trials
- The Strategy, through CIHR, is providing various funding options for projects focused on enabling more clinical trials related to rare diseases with a goal of drawing more drug submissions to the Canadian market:
  - CIHR is supporting the creation of a network for pediatric rare disease clinical trials and treatments, including RareKids-CAN, which is now up and running and moving forward with multi-faceted efforts across a number of areas
  - CIHR is increasing readiness for gene therapy clinical trials for rare diseases (in partnership with the National Research Council Canada) – three team grants have been awarded
- The Strategy is also supporting tools to build information on emerging drugs for rare diseases (DRD)
  - CDA-AMC is building a pipeline repository to better inform drug plans of future products and to help the system prepare for their introduction – a needs assessment has informed the building and testing of a prototype that resulted in an active tool to be launched in 2025

Supporting Decision-Making Processes

- The Strategy is supporting building an evidence and knowledge base to inform decision-making through various activities that support advancing data, analytics, and monitoring
- This includes a focus on building better data infrastructure and information systems:
  - CDA-AMC has published an inventory of Canadian rare disease registries, established guidance around registry standards, is funding improvements to 18 rare disease registries to enhance and expand their holdings to better support reimbursement decision-making, and has released guidance on linking health data
  - The Canadian Institute of Health Information (CIHI) has launched an interactive tool for users to compare public prescription coverage across Canada as well as reports on the existing prescription drug data landscape and considerations to support the use of data for analyses of DRD
  - CIHI also built on this work to explore preliminary insights on the impact of Trikafta on Cystic Fibrosis patients and medication use and care among Huntington Disease patients

Supporting Decision-Making Processes (2)

- The Strategy is also supporting and exploring activities that can turn data into actionable information to aid in decision-making
  - CDA-AMC is building capacity for reassessment through the Formulary Management Expert Committee – Strategy funds have supported 4 customized reviews of DRD to advise decision-makers on new recommendations based on recent data
  - CIHR is funding projects focused on improving insights from administrative data and monitoring for rare diseases to better understand health system impacts – two team grants have been awarded
  - Through the bilateral agreements, Health Canada is working with provinces and territories to explore real-world evidence projects – the goal is to explore how to help decision-makers gather and evaluate real world data relevant for drug listing and reimbursement
    - This work serves as a baseline for improving, collecting, and using data and will involve assessing the useability and transferability of approaches to inform future phases of the Strategy

National Consistency in Access

- Ensuring patients can consistently access DRD across Canada by sharing the costs associated with reimbursement of these drugs and working to enable public coverage
  - Through the bilateral agreements, the Strategy is directly helping public drug plans to support drug coverage and sharing the costs of DRD, while exploring how to improve availability
  - PTs may use bilateral agreement funds to cover drugs on a common list AND to improve coverage for any other new or existing DRD
  - PTs can therefore cover drugs based on the circumstances of their jurisdiction, while also working toward pan-Canadian consistency
- The Government of Canada has signed bilateral agreements with British Columbia and Newfoundland and Labrador
- Negotiations are ongoing with other jurisdictions

Coordination

- Strategy activities are supporting better governance and convening leaders around common goals
  - Through the Pharmaceutical Executive Group (PEG) for public plans, Health Canada is working with provinces and territories to establish a Federal-Provincial-Territorial governance structure to enable collaborative discussion and collective work around public drug coverage, screening and diagnostics, and real-world evidence
  - An Implementation Advisory Group, composed of stakeholders in patient groups, clinicians, industry and others, is providing advice to Health Canada on Strategy projects and assessing progress while ensuring that stakeholders are provided with a platform to inform ongoing work
- To support collaboration and reinforce other activities that are supporting patients, partners are working to integrate Strategy activities with other initiatives across the country
  - Health Canada and CDA-AMC have been meeting with the Canadian Rare Disease Network (CRDN) to explore how best to align various activities specific to patient registries, diagnostic services, and other infrastructure
  - RareKids-CAN is deeply integrated in the CRDN work to support innovative therapies, and are working with CRDN to establish a national coordination group that feeds into broader international efforts working to align actions in rare diseases (European Rare Disease Research Alliance – National Mirror Group)