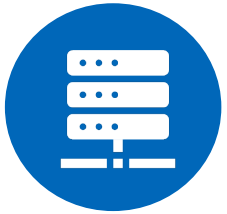
Canada's Drug and
Health Technology Agency

Support for the Strategy for Drugs for Rare Diseases

Disease Based Registries & Real-World Evidence - Description



Create a “Registry of Registries” for Rare Diseases and assess the quality and comprehensiveness of data within those registries.

Establish data standards for registries to be ‘fit for purpose’ for both HTA and regulatory evaluations

Test existing registries in HTA and identify barriers or limitations in data readiness, comprehensiveness, and/or ability to report on relevant outcomes.

Benefits

- Improve business intelligence to inform jurisdictional decisions
- Optimize RWE data sets for long-term analysis

Disease Based Registries & Real-World Evidence - Activities

- Work with registry owners to achieve data completeness and quality improvements so that registries can be 'fit for purpose' for both HTA and regulatory purposes
- Aligning with other DRD/RWE initiatives under the National DRD Strategy co-led by CIHI and Health Data Research Network (HDRN) Canada
- Convening an advisory panel to guide planning and implementation

Open funding call for registry improvements launching soon

- Request for Proposals (RFP): Rare Disease Registries are invited to submit proposals to receive funding in order to support quality improvement initiatives for their registries
- Hosting webinar on March 14th from 1:00-2:00 pm ET to provide additional information and answer questions
- Refer to CADTH Events Page or contact Registries@CADTH.ca for more information

Newborn Screening – Description

Provide jurisdictions with the best available information to support decision-making through:

Building on existing work to map current newborn screening coverage across the country

Facilitating engagement with interested parties to ensure the right voices are included

Convening experts to provide advice and guidance

Benefits

- Leverage lessons learned and address common challenges
- Identify solutions to meet the needs of the system

Newborn Screening – Activities

- Recruited participants and created an Advisory Panel
- Hosted kick-off hybrid meeting on January 11th, 2024
- Drafting principles which will guide the development of criteria for consistent newborn screening in Canada

DRD Pipeline Repository – Description

- New interactive self-service tool for decision makers
- Designed based on user needs in the Canadian context
- Consolidated path to access relevant information about DRDs

- Provides improved intelligence about which DRDs may enter Canada and when
- Supports customized reports on demand
- Informs planning for implementation decisions

Benefits

- Easy access to consistent and up to date information about DRDs
- Pro-active monitoring to signal new products early for decision-makers

DRD Pipeline Repository – Activities

- ✓ Conducted and validated a needs assessment with public drug program leaders to identify gaps and use cases
- ✓ Secured a data source for validation
- ↻ Developing a prototype for review and testing by Spring 2024

Customized Pharmaceutical Work on Request

- Advancing the DRD Strategy by leveraging existing processes and infrastructure and dedicated expertise; examples include:
  - Non-sponsored reviews and reassessments to support optimizing use of DRDs.
  - Implementation panels to address questions related to DRDs by FPT decision-makers.
  - Customized Health Technology Reviews on select DRD topics.

- 2 non-sponsored reviews for rare disease drugs are being conducted as part of the Formulary Management Expert Committee (FMEC) pilot
  - eltrombopag for severe aplastic anemia and everolimus for tuberous sclerosis complex)

- The FMEC pilot is an innovative deliberative committee which further incorporates patient and clinician engagement to consider their perspectives in addition to technical expertise

Benefit

- Jurisdictions receive timely and customized support
- Provides opportunities to innovate process (e.g., patient engagement)

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