Canada's
Drug and Health
Technology Agency

Real-World Data and Real-World Evidence at CADTH

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Real world data (RWD) is an umbrella term for data collected outside of the randomized clinical trial (RCTs) paradigm.

Real world evidence (RWE) is derived from the analysis of data collected outside of randomized controlled trials.

It is considered complimentary to RCTs, not a replacement.

It includes data from medico-administrative databases, registries, observations from clinical practice, and patient-reported information.

CADTH already uses and accepts RWE

Best Brains Exchange (October 2021)

- Successful collaboration between Canadian Institutes for Health Research (CIHR), CADTH, Canadian Organization for Rare Disorders (CORD) and Health Canada
  - And members of the **RWE4Decisions international** initiative
- ~150 attendees on day 1 and 2
- Representatives of 13 different types of stakeholders
- Participants from 9 provinces and 1 territory
- 5 multi-stakeholder breakout groups discussed optimization of use of RWE in the Canadian context over 2 days
- Knowledge foundation of Canadian stakeholders in the domain of drugs for rare disease
- Learnings: Data Access and Multistakeholder

Learning Period Launch (November 2021)

Key Learnings from First Year of Consultations and Collaborations

We have a **Coordination**, **Awareness**, and **Data** problem.

"It is critical that a multistakeholder team comes together…to say what kind of data is needed and what are going to be the caveats around the quality of that data."

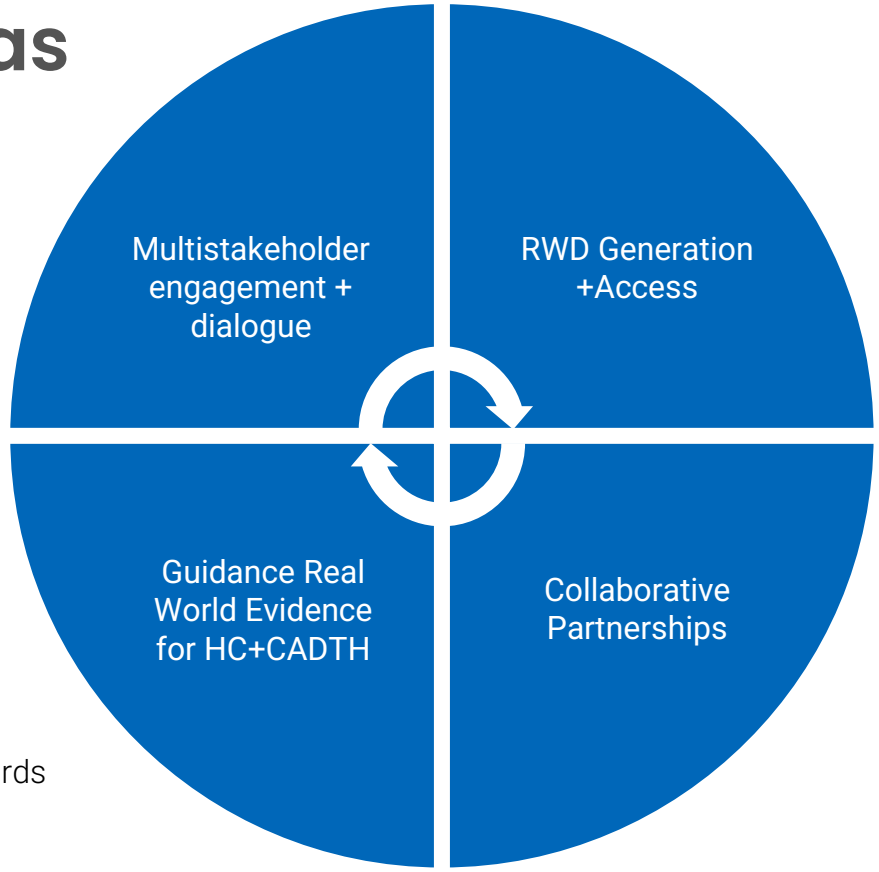
RWE Goals

1. Provide learnings to Health Canada to support rare disease strategy (**ANTICIPATE**)

2. Develop methods and standards for CADTH for appraisals and reviews of drugs for rare disease or complex therapies (**INNOVATE** and **TRANSFORM**)

Develop Strategies Within 4 Key Areas

- Best Brains Exchange
- Literature review
- Expert support and advice
- Learning by doing
- Stakeholder feedback
- Inform development of process



- DSEN Data Access WG
- Literature review
- Expert support and advice
- Data holder network
- Learning by doing
- Inform development of process and standards

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- Partnership with Health Canada
- Partnerships with data holders and facilitators
- RWE Steering Committee with pan Canadian Health Organizations
- International partners

Develop infrastructure, governance, guidance and tools within the 4 pillars

Multistakeholder Decision-Making Focus through the Lifecycle (TRANSFORM)

1. **Early Dialogue**
   Identify needs of patients, clinicians, HTA, payers, regulator

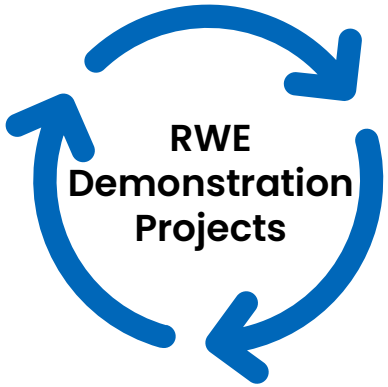
2. **Second Dialogue After Pivotal Trial, but Before Official HTA Submission**
   Identify and plan response to remaining uncertainties

3. **Post HTA Dialogue**
   Discuss evidence generation after launch in the real world setting and its consequences

4. **Postmarket Dialogue**
   Reassessment of new evidence

Vision: Iterative dialogue for complex health technologies at all 4 timepoints

CADTH RWE Steering Committee (TRANSFORM)



Oversight

CADTH (Chair)

Health Canada (co-Chair)

Members

INESSS **pCPA** **CIHR** **CORD** **IMC/BIOTECanada**

Statistics Canada **CIHI** **HDRN** Invited Experts (ad hoc)

INESSS: Institut National d'Excellence en Sante et Services Sociaux
pCPA: Pan-Canadian Pharmaceutical Alliance
CIHR: Canadian Institute of Health Research
CORD: Canadian Organization for Rare Disorders
IMC: Innovative Medicines Canada
CIHI: Canadian Institute of Health Information
HDRN: Health Data Research Institute

RWE Steering Committee Oversight

Oncology Working Group
Notice of Compliance
with conditions

Non-Oncology Working Group
Notice of Compliance
with conditions

RWE Guidance
Working Group

Data Access for Safety and Effectiveness of Drugs
Working Group

Rare Disease RWD Generation
Working Group

CADTH RWE and rare disease initiatives (INNOVATE)

- Developing a Canadian inventory of databases for **rare disease** registries
- Developing standards and processes to enhance the quality of data in Canada's **rare disease** registries in collaboration with a **registry network**
- Developing partnerships with Canadian administrative data holders and data facilitators
  - CIHI ; Statistics Canada ; HDRN Canada
- Developing partnerships with RWE methodological experts
- Exploring the utility, feasibility of data requests to **real world data** holders
- Developing a **multistakeholder** approach to determining outcomes for **rare disease**
- Developing partnerships with international partners
  - RWE4Decisions ; ISPOR ; ISPE ; CIOMS
- Enhancing CADTH internal processes and procedures to optimize use of **RWE** for decision-making

Learning by Doing Projects: Collaboration and RWD to Support Decision-Making

Projects	Anticipate
Amyotrophic Lateral Sclerosis	Using registry and administrative data to describe the portrait of care
Pediatric Glioma	Multistakeholder dialogue on RWD generation to describe the portrait of care
Pediatric Spinal Muscular Atrophy	Multistakeholder dialogue on RWD generation from a registry to describe the portrait of care
Cystic Fibrosis	Multistakeholder dialogue on RWD generation about care for different sub-populations of patients
Rare disease in pediatric patients	Participation with NICE in post-phase III scientific advice
Plaque Psoriasis	Use of private and public payer data to inform decisions about optimal pharmacotherapy
Multiple Myeloma	Collaboration with a Canadian registry data and an international partner to inform decisions about optimal pharmacotherapy
Mild /Moderate Dementia	Use of published RWD and different stakeholder perspectives to develop an evidence bundle concerning Canadian health system readiness for new potential therapies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