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Drugs. Health Technologies and Systems. Médicaments, technologies de la santé et systèmes.

2025–2026 Rare Disease Registry Funding Opportunity Information Session

April 2, 2025

Land Acknowledgement

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Disclosure

- The organization is funded by contributions from the Canadian federal, provincial, and territorial ministries of health.
- We receive application fees from the pharmaceutical industry for:
    - Our Reimbursement Review processes, including those used for:
        - oncology drugs
        - non-oncology drugs
        - plasma protein and related products reviewed through the interim process
    - Scientific Advice

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Housekeeping

Slides from today will be posted on our website

Questions will be taken after the presentation

Bulletins will also be posted during the application window

Full details about the funding opportunity in the **2025-2026 RFP Document**

Overview for Today's Webinar

1. Canada's Drug Agency and the National Strategy for Drugs for Rare Diseases
2. Overview of 2025–2026 Funding Opportunity
3. Application Components
4. Evaluation & Timelines
5. Questions

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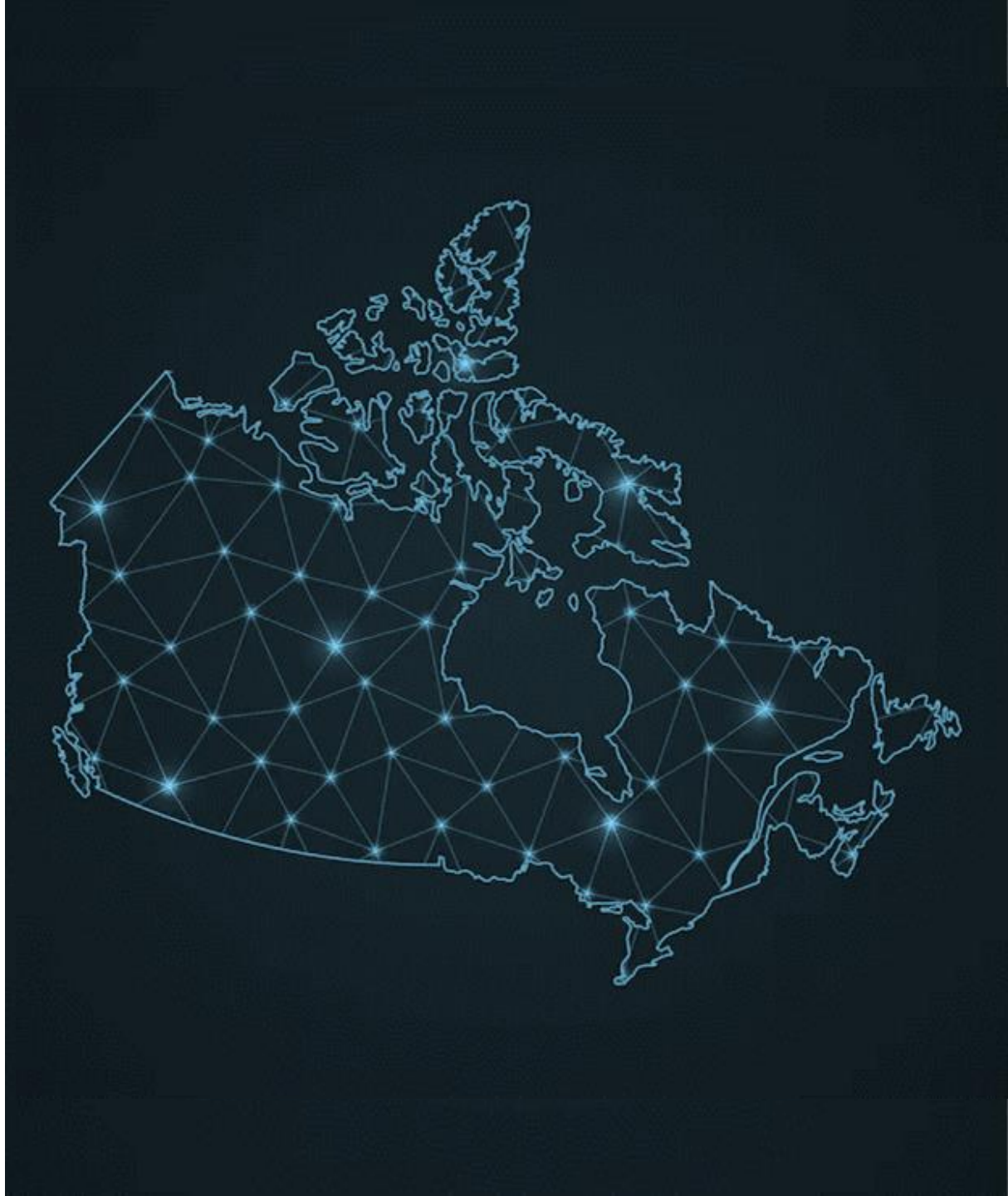
Committed to Helping Broaden the Evidence Landscape

Key Areas of Work Related to Registries

- Improving awareness about the landscape of rare disease registries
- Providing guidance about improving rare disease registries
- Investing in rare disease registries to enhance capabilities and be fit-for-purpose



Developments Since Inaugural Funding Opportunity

- **Registry funding**
  - **18 rare disease registries (RDR)** funded through a competitive RFP in 2024-2025.
  - Supporting initiatives to improve infrastructure, data quality, and capabilities.

- **Bilateral agreements**
  - **All Provinces/territories** have signed agreements with Federal Govt to improve access to drugs for rare diseases (DRD)
  - Funding to be provided from 2024 to 2027.

- Several new and emerging DRD for rare diseases have evidence uncertainties that could inform decision-making related to reimbursement, coverage, and implementation across jurisdictions.

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2025–2026 Funding Opportunity Overview

Aims of 2025–2026 Funding

- **To enhance** the quality of registries' data collection, **infrastructure**, and capabilities to generate high-quality real-world data for DRDs.
- **To support** initiatives that strengthen registries' ability to **address questions** and uncertainties related to rare disease treatment patterns and outcomes.
- **To equip** registries to produce **decision-relevant evidence** for informing policy and reimbursement decisions in Canada.
- **Total Funding Available: up to $3 million**

Limits of Scope: This funding opportunity is **not** conducting independent analyses or making new reimbursement recommendations outside of existing evidence-review and decision-support functions at Canada's Drug Agency.

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Feedback from Inaugural Funding	Changes Within 2025–2026 Funding Opportunity
This is an innovative funding opportunity to support registries	• Overall scope remains to bolster registries' capabilities and improve data quality • Higher funding amount can be requested
Is there opportunity to streamline & simplify the application process?	• Incorporates standard form for **letters of intent (LOI)** • Provides specific guidance about sections and tables to include in **detailed proposals**
Can you clarify all requirements at RFP launch and limit post-launch bulletins?	• Includes full evaluation criteria at RFP launch • Provides transparency about prioritization approach • Details total funding amounts available

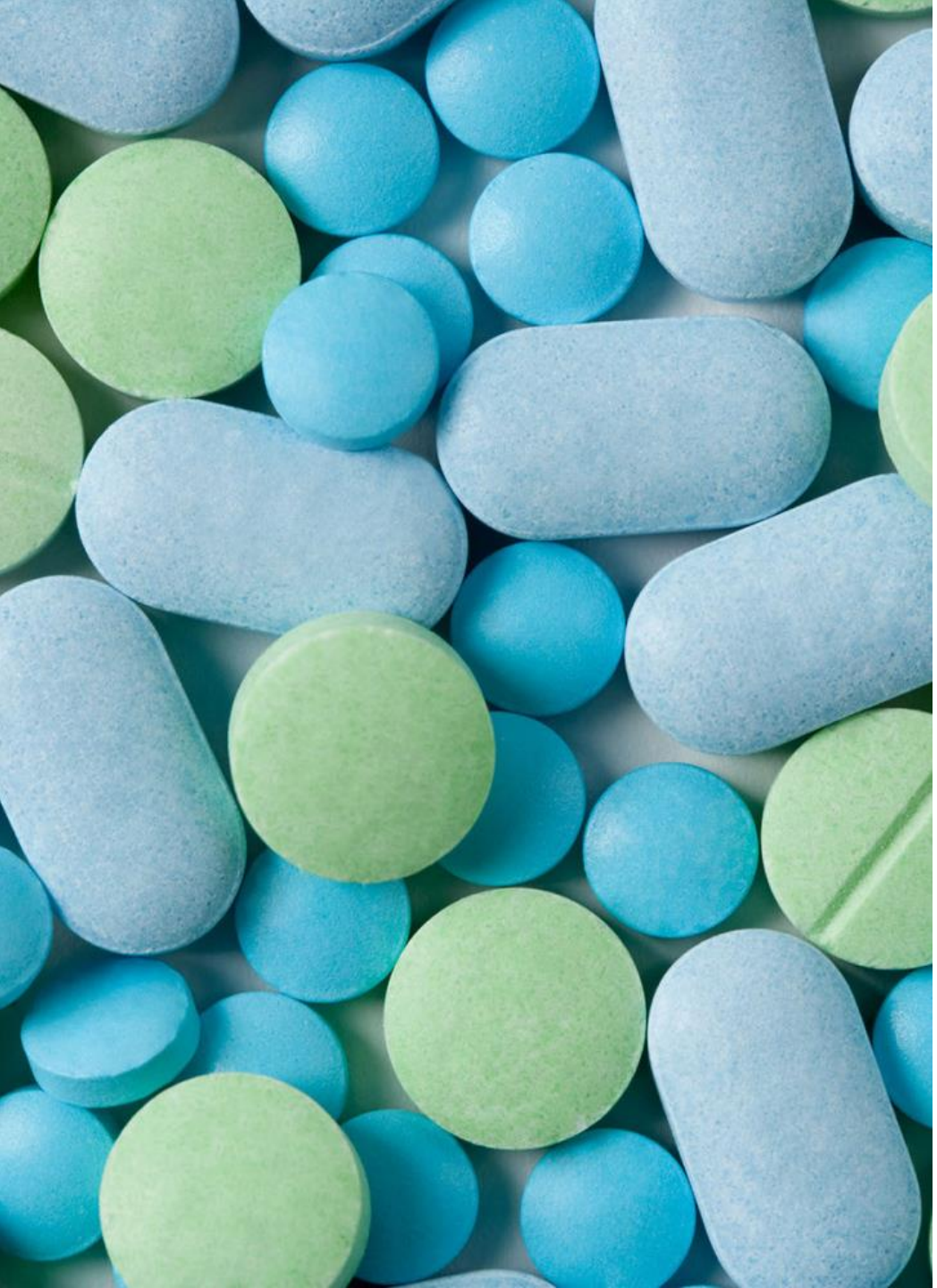
Eligibility

Rare Disease

- Meets classification of the National Organization for Rare Disorders (NORD), Food and Drug Administration (FDA), or the European Medicines Agency (EMA)
- **May include other diseases** based on unmet needs in clinical care, access to medicines, etc.

Registry

- Is capable of adding new patients
- Collects observational data about specific disease(s) or condition(s)
- Collects data that can be used to answer clinical, scientific, and policy questions (patient contact database alone is not sufficient)
- Has the potential to inform health care decision making



Targeted Towards Fit-For-Purpose Initiatives

Applicants must demonstrate how proposed initiatives address specific uncertainties or data gaps with DRDs

- DRDs have unique and diverse evidence uncertainties related to:
  - Clinical effectiveness
  - Safety
  - Health system impact
  - Implementation
  - Other domains

- Applicants to list the specific DRD poised to benefit from initiatives and their **‘Priority Level’**

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Priority Levels Correspond to Reimbursement & Regulatory Stage

Level 1

- DRD has **completed** negotiations at pCPA, since 2022

Level 2

- DRD is in **active** negotiation or **under consideration** for negotiation at pCPA

Level 3

- DRD is in **phase III** development or **pre-market**; OR
- DRD cannot be classified in L1 or L2, but has evidence-uncertainties that could be addressed by registry data

Consult CDA-AMC reimbursement reviews for evidence uncertainties

Example 1: Aligning Initiatives to Decision-Making

Drug Name: Hypothetical
DRD Level: 2, active negotiation at pCPA

CDA-AMC Review: N/A

Evidence Context	Decision-Making Question	Proposed Initiatives
There is uncertainty about the number of **potential patients who may be eligible** for a new hypothetical DRD, that is currently being negotiated at pCPA	What is the anticipated **number of patients in Canada** who could benefit from the new DRD?	• Improving registry coverage (prevalence) • Improving data collection to identify eligible patients • Improving governance policies to enhance data sharing and patient identification

Example 2: Von Hippel-Lindau disease

Drug Name: Welireg (belzutifan)
DRD Level: 1, completed pCPA negotiations in 2024

CDA-AMC Review: Completed, Sept 2023

Evidence Context from CDA-AMC Review	Decision-Making Question	Proposed Initiatives
Evidence shows high proportion of patients discontinue treatment. There is uncertainty about the **discontinuation rate** and characteristics associated with discontinuation in clinical practice.	What is the **number of patients and factors associated** with discontinuing treatment?	• Enhancing longitudinal treatment data collection • Improving data collection of patient characteristics (potentially re-consent)

Example 3: Spinal Muscular Atrophy

Drug Name: Zolgensma (onasemnogene abeparvovec)
DRD Level: 3, completed pCPA negotiations in 2021

CDA-AMC Review: Completed, May 2020

Evidence Context (CDA-AMC Review)	Decision-Making Question	Proposed Initiatives
There is uncertainty about the number of patients who received other existing therapies for SMA before treatment with Zolgensma, as well as after treatment with Zolgensma	What is the **number of patients in Canada** who receive other therapies for SMA pre- and post- treatment with Zolgensma?	• Enhancing the collection of longitudinal treatment data • E.g., start dates, adherence, event dates for discontinuation or switching, reasons for discontinuation

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Example 4: Acute myeloid leukemia (AML) or myelodysplastic syndromes (MDS)

Drug Name: Trecondyv (treosulfan)
DRD Level: 1, completed pCPA negotiations in 2024

CDA-AMC Review: Completed, June 2024

Evidence Context (CDA-AMC Review)	Decision-Making Question	Proposed Initiatives
There is uncertainty about the impact of treosulfan on health-related quality of life in patients with AML or MDS	What is the health-related **quality of life** in patients with AML or MDS treated with treosulfan?	• Improving data collection related to patient reported outcomes • Engaging with patient partners to improve the completeness of patient reported outcome data

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RFP Application Components & Process

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2 Stage Application

Letter of Intent

LOI Form

- Registry information
- List of DRDs that are poised to benefit from initiatives
- Abstract of proposed initiatives (300 words)

Detailed Proposal

Technical

- Evidence uncertainties for specific DRDs
- Proposed objectives and alignment to evidence gaps
- Risks and mitigation
- Table of expected deliverables by milestones

Financial

- Expenses and resources required for project completion
- Excel Template
- Max: $300,000

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Application Stages

Refer to RFP Document for Detailed Instructions

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Detailed Proposal Evaluation

Review panel will independently conduct scoring and collectively deliberate rankings

Technical Evaluation Domains

1. **Strategic Impact:** Alignment to evidence-uncertainties about specific DRD
2. **Methodology:** Initiatives are well designed to address evidence gaps, appropriate, and comprehensive
3. **Feasibility:** Organization has resources and is equipped to deliver results by contract end-date
4. **Risk Mitigation:** Proposed initiatives do not face procedural challenges that could affect project completion, and if so, have adequate mitigation plans

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Application Deadlines

Letter of Intent

RFP launch: March 26, 2025

LOI submission date: **April 16, 2025**

Detailed Proposal

Applicants notified: May 2, 2025

Proposal submission date: **May 30, 2025**

Notification of results: Early July 2025

All contracts funded until March 31, 2026

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Bulletin Publication Timelines

Letter of Intent Stage

Deadline to submit questions: **April 8, 2025**

Bulletin publication dates:

1) April 7, 2025
2) April 14, 2025

Detailed Proposal Stage

Deadline to submit questions: **May 21, 2025**

Bulletin publication dates:

1) May 12, 2025
2) May 20, 2025
3) May 26, 2025

Additional Points for Consideration

Rare disease registries at **any maturity level**, including newer registries may apply, however, fit-for-purpose initiatives aligned to evidence needs will be most competitive

Overall competitiveness is based on the **quality of proposals and their alignment to RFP objectives**

Consideration for inclusion, diversity, equity, and accessibility (IDEA) as part of proposed engagement approach

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Questions

Monitoring Website for Updates

- Continue to visit: https://www.cda-amc.ca/drugs-rare-diseases
- Send questions to contracts@cda-amc.ca
- All responses will be provided via posted bulletins

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