Rare Disease Registries (RDR) at CADTH

The Drug Data Services and Analytics Team

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Overview

1 Introduction to the rare disease registry portfolio at CADTH

2 Request for Proposal: funding opportunity for RDR quality improvement initiatives

3 Application process

4 Proposal evaluation and selection approach

5 Q & A

Commitment to improve rare disease registries

- Drugs for Rare Diseases is a health care priority, with substantial unmet need and potential for growth

- Canada is health **data rich**, **but information poor**

- CADTH funded by Health Canada to lay the foundation for improving real-world evidence generation for rare diseases

- Three Key Activities
  - **Understand** the landscape of rare disease registries (RDR)
  - **Develop** standards & **identify** best practices
  - **Support** quality improvement

Transform

Funding opportunity for RDR quality improvement

- CADTH is providing a new stream of funding through the National Strategy for Drugs for Rare Diseases

This time-limited funding (until March 2025) supports RDR to:

Improve infrastructure and data quality

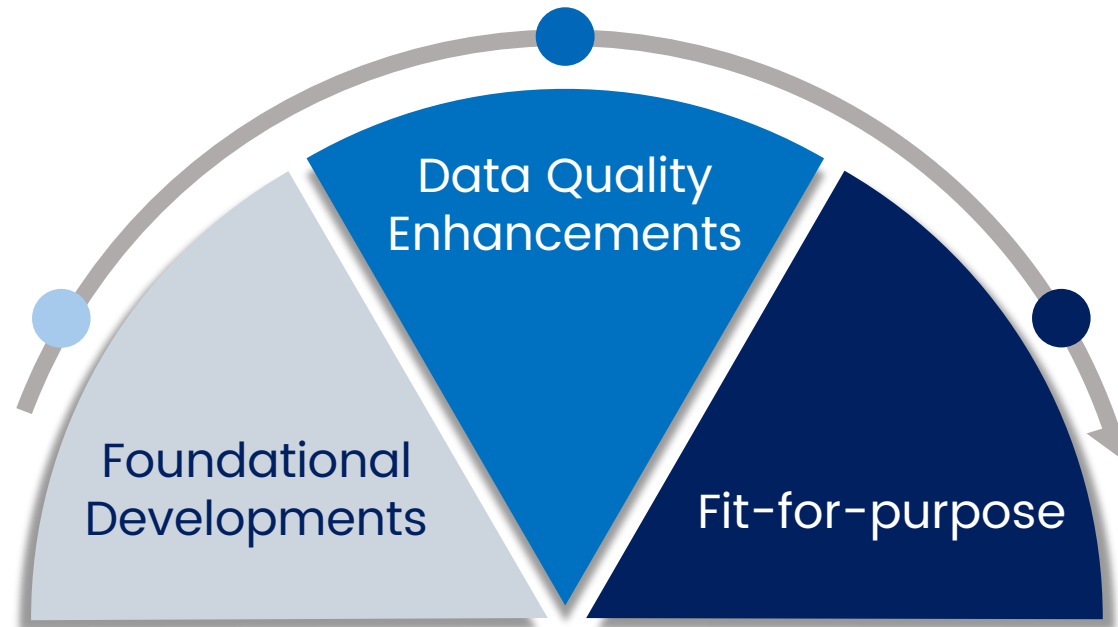Increase capacity to produce decision-grade real-world evidence

Enhance ability to answer regulatory, HTA, and payer evidence questions

Funding opportunity for RDR quality improvement (Maturity)

- Funding is available across three key improvement domains

Rare Disease Registry Maturity

Foundational Developments

Data Quality Enhancements

Fit-for-purpose

Potential initiatives for registry funding

Foundational Developments

- Enhance registry governance structure

- Refine the data quality assurance plan

- Improve data sharing and access policies

Data Quality Enhancements

- Enhance adherence to standardized coding

- Improve completeness of data

- Increase coherence of data

Fit-for-purpose

- Improve longitudinal data collection of treatment outcomes

- Enhance relevance of data elements to align with data collection from clinical trials

Who is eligible for funding?

Rare Disease

- Informed by the National Organization for Rare Disorders (NORD), Food and Drug Administration (FDA), and the European Medicines Agency (EMA)

- **May include additional considerations** for unmet needs in clinical care, access to medicines, etc.

Registry

- Active registry (does not only use retrospective patient data)

- 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 wider health care decision making

Unsure if you meet eligibility criteria? Please reach out to us at Registries@cadth.ca

Application and proposal components

For invited applications

1 **Letter of Intent**

- Notify CADTH of the applicant's intention to submit a proposal

- Include a brief overview of planned quality improvement activities

- Maximum 2 pages

2 **Technical Proposal**

- Overview of registry and team

- Proposed work plan, including risk and mitigation strategies

- Work plan must align with RFP objectives and include performance measures

- Maximum 12 pages

Financial Proposal

- CADTH will provide a Financial Proposal Form

- Submitted as an Excel

Send all documents to contracts@cadth.ca

If you are unsure about the application process or components, reach out to us at registries@cadth.ca

Application process

RFP release date: March 27, 2024

Submit a Letter of Intent (LOI):
Deadline: April 10, 2024
Decision: April 17, 2024

Full proposal:
Deadline: May 17, 2024, for invited applications
Decision: May 31, 2024

Submission:
All documents to contracts@cadth.ca;
include **Registries RFP** in the subject line

Review process: assessment for compliance, relevance and merit. Includes confirmation receipt and review outcome.

After approval: Implementation and monitoring, including specified deliverables, and performance measures.

Proposal evaluation and selection

Evaluation process: review by a multidisciplinary panel

Selection methodology: evaluation based on feasibility, immediate impact, and strategic importance
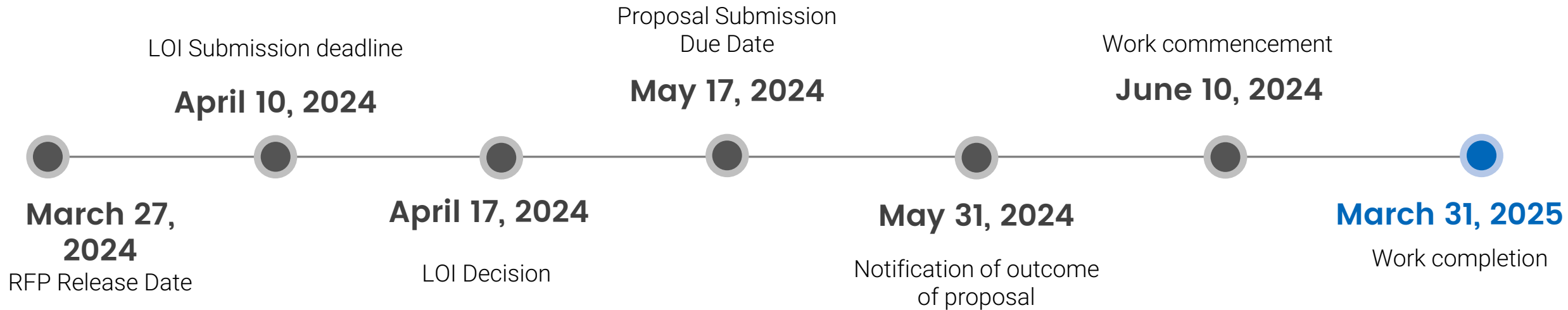
Open to innovation: while focusing on specified areas, we remain open to innovative proposals with strong potential for impact

Alignment with objectives: applicants are encouraged to demonstrate project alignment with priority areas and evaluation criteria, providing specific examples

Registry Funding Timeline

LOI Submission deadline

April 10, 2024

Proposal Submission
Due Date

May 17, 2024

Work commencement

June 10, 2024

**March 27,
2024**

RFP Release Date

April 17, 2024

LOI Decision

May 31, 2024

Notification of outcome
of proposal

March 31, 2025

Work completion

Thank you