CDA-AMC Rare Disease Registry Request for Proposals 2025-2026

Issued: March 26, 2025
Contract Number: C-242506400
Important Dates: Refer to Section 3.3 for Request for Proposal Timelines
All inquiries and submissions by email: contracts@cda-amc.ca

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Overview of RFP Timeline and Schedule

Table 1: Timeline of Key Events Related to the RFP

Description	Date	Reference in this document
1. RFP release	March 26, 2025	Published
2. RFP information webinar	April 2, 2025	Section 3.1
3. Deadline to submit inquires about LOI	April 8, 2025	
4. CDA-AMC to post final bulletin for LOI	April 11, 2025	
5. Submission deadline for LOI; send to contracts@cda-amc.ca	April 16, 2025	
6. Notification of LOI results	May 2, 2025	Section 3.2
7. Deadline to submit inquires about proposals	May 21, 2025	
8. CDA-AMC to post final bulletin for proposals	May 26, 2025	
9. Submission deadline for detailed proposals	May 30, 2025	
10. Notification to contract awardees	Early July 2025	Section 3.4
11. Work commencement	July 2025	

CDA-AMC = Canada's Drug Agency; LOI = Letter of Intent; RFP = Request for Proposal.

1. Introduction

Canada's Drug Agency (CDA-AMC) is seeking proposals from rare disease registries to enhance their capability and implement improvement initiatives to support decision-making in Canada related to drugs for rare diseases (DRDs).

This funding is designed as a short-term contract opportunity rather than a long-term grant program. The timelines are structured to ensure that funds are awarded and fully used within the 2025–2026 fiscal year. As a result, all projects funded through this Request for Proposal (RFP) must be completed by **March 31, 2026.**

1.1. CDA-AMC Overview

CDA-AMC is an independent, not-for-profit organization responsible for providing health care decision-makers in Canada with objective evidence to help make informed decisions about the optimal use of drugs and medical devices in health care systems in Canada.

CDA-AMC receives funding from Canada's federal, provincial, and territorial governments, except Quebec.

1.2. Alignment With National Strategy for Drugs for Rare Diseases

The National Strategy for Drugs for Rare Diseases was launched by the federal government in 2023 with a directive to help improve the access and affordability of DRDs in Canada. As part of the national strategy, most of the $1.5 billion in funding is for provinces and territories to improve access to new DRDs, enhance coverage of existing DRDs, improve screening and diagnostics, and support decision-making related to

DRDs. Since the launch of the strategy, funding is distributed to provincial and territorial jurisdictions via bilateral agreements with the federal government designed around these goals.

CDA-AMC is funded as part of the national strategy to support the collection and use of evidence, including real-world evidence, to guide decision-making related to DRDs. This RFP is a component of that funding as it aims to improve data quality, infrastructure, and capabilities of rare disease registries to generate evidence for decision-making.

2. Funding Overview

2.1. Eligibility Criteria

This funding opportunity is limited to rare disease patient registries. CDA-AMC will evaluate eligibility based on the following criteria:

- **Rare diseases:** Defined as 'rare' or holding 'orphan' designation by the European Medicines Agency (EMA), FDA, the National Organization for Rare Disorders (NORD), or diseases with significant unmet needs may also be considered even if they are not included in the international definitions, as long as the application provides a clear rationale.
- **Patient or disease registries:** The term 'patient registry' is often used interchangeably with 'disease registry.' In this RFP, both patient registries and disease registries refer to organizations that collect observational data to evaluate specified outcomes for a population defined by a particular disease, condition, or exposure for various purposes, including research, clinical care, and informing policy.
  - Registries that cannot recruit new patients or that rely exclusively on retrospective datasets will not be considered active and therefore, will be ineligible.
  - Registries should have the capacity to share information about their structure, data governance, and methodologies with decision-makers within publicly funded health systems, ensuring transparency and the ability to assess their relevance for decision-making. This does not imply the sharing of line-level or identifiable patient data; rather, the focus is on ensuring that registry design and data collection processes can be reviewed and understood.

2.1.1. Additional Requirements

Funding will be provided only to Canadian or international organizations with a Canadian site or account and are collecting data on patients in Canada.

Registries receiving federal funds or research grants from another component of federal rare disease funding (e.g., Canadian Institutes of Health Research [CIHR] Rare Disease Research Initiative) in the 2025–2026 fiscal year are required to declare their funding.

CDA-AMC's role in this initiative is limited to providing funding and support for registries to enhance the quality of their data collection and infrastructure.

CDA-AMC is not conducting independent analyses or making new reimbursement recommendations outside of existing evidence-review and decision-support functions. The objective of this funding is to ensure that registries can produce high-quality real-world data (RWD) that may be used by decision-makers within the health system in Canada.

2.2. Focus On Fit-for-Purpose Enhancements

CDA-AMC is committed to supporting the development of registries in Canada to help broaden the evidence landscape and generate high-quality RWD. As part of this broader goal, CDA-AMC has developed the Best Practices and Standards to Enhance the Quality of Rare Disease Registries in Canada to provide guidance for enhancing overall registry infrastructure, data quality, governance, and other aspects of registry design (there is no obligation or prerequisite to meet these standards).

We also recognize that the landscape of rare diseases is highly diverse. Rare diseases differ in treatment options, clinical outcomes, and organizational structures of their registries. Some registries have been well established for many years, while others are in the initial stages of development. For this funding opportunity, regardless of their maturity, size, or history of development, any rare disease patient registry is eligible to apply for funding.

Nonetheless, this funding opportunity is designed for applicants proposing **targeted, fit-for-purpose initiatives** that strengthen the registries' ability to address **specific questions and uncertainties** related to rare disease treatment patterns and outcomes. Proposals that focus solely on broad registry improvements or the natural history of rare diseases — without a clear connection to addressing therapeutic uncertainties, including comparative treatment outcomes — will be less competitive.

Given high demand and limited funding, registries with a well-defined plan to bolster their ability to generate decision-grade data will be most competitive. While registries in earlier stages of development are eligible, CDA-AMC will prioritize those with a clear strategy to enhance their capacity to meet current and emerging evidence needs for decision-makers at the federal, provincial, and territorial levels.

The primary objective of this funding is to enhance registry **infrastructure, data collection processes, and overall data quality** to ensure they can generate **decision-relevant evidence**. CDA-AMC's role is not to conduct independent analyses but to support registries in becoming **fit-for-purpose** in order that decision-makers can access high-quality RWD to inform policy and reimbursement decisions.

CDA-AMC encourages applications that target specific gaps in improving **data quality and/or relevance** to enable the generation of fit-for-purpose analyses.

- **Data quality** refers to reliability, accuracy, completeness, traceability, extensiveness, coherence, consistency, and timeliness dimensions of registry data. Improving data quality can entail a range of

improvements that relate to registry infrastructure, policies, governance, and specific data collection, data processing, or reporting components. Applicants may refer to international frameworks and guidance by the FDA, EMA, and the National Institute for Health and Care Excellence (NICE).

- **Relevance** refers to how well both the specific data elements captured in the registry and the overall dataset are aligned to, and capable of, answering decision-making questions. Relevance ensures that the registry dataset includes the population of interest, and has the appropriate coverage, treatment data, outcomes, and other characteristics that can be analyzed to help inform evidence needs.

Proposed initiatives must clearly demonstrate how their proposed data and infrastructure improvements will enhance data quality and relevance, as previously mentioned. Specifically, applicants should articulate how their initiatives will improve reliability, accuracy, completeness, and timeliness of data, as well as ensure alignment with decision-making needs. Addressing specific evidence gaps or uncertainties should be directly tied to these foundational improvements in registry infrastructure and data collection.

Applicants should propose targeted initiatives that align with the principles of data quality and relevance, ensuring that registries can generate decision-grade evidence. High-priority initiatives include those that strengthen data on prevalence, usage, implementation, clinical and comparative effectiveness, safety, costs, and economic evaluation. Applicants are encouraged to refer to Appendix 1 detailing examples of DRDs (hypothetical) and how to align proposed initiatives to specific DRDs and their associated evidence needs. There are also examples of relevant initiatives, including infrastructure-focused investments for emerging registries.

2.3. Prioritization of Initiatives

CDA-AMC will prioritize initiatives that align with DRDs that have clearly defined evidence needs. Evidence needs refers to regulatory, reimbursement, health technology assessment, or payer questions from federal, provincial, or territorial decision-makers about specific DRDs. This prioritization focuses on new and emerging DRDs that are likely to have anticipatory evidence needs within the next 1 to 2 years in Canada based on their status within Canada's regulatory and reimbursement pathway. **Registries are highly encouraged to refer to published CDA-AMC reimbursement reviews that identify evidence uncertainties and gaps for new and emerging DRDs.**

Applicants will be required to provide the priority designation of specific DRDs as per the priority levels described in Table 2. These levels reflect the current stage of DRDs within the regulatory, health technology assessment, and reimbursement pathways in Canada. DRDs further along in the pathway will be assigned a higher priority level.

Table 2: Prioritization Levels of DRDs for This RFP

Priority	Description
Level 1	• DRDs with recently completed (since 2022) negotiations at pCPA. These DRDs will also have completed CDA-AMC or INESSS reimbursement reviews describing evidence uncertainties related to clinical effectiveness, safety, cost-effectiveness, access, implementation, or other aspects related to the therapy.
Level 2	• DRDs in active negotiation or under consideration for negotiations at the pCPA These DRDs may be in active review or have completed reviews (since 2022) at CDA-AMC or INESSS, describing evidence uncertainties related to clinical effectiveness, safety, cost-effectiveness, access, implementation, or other aspects related to the therapy.
Level 3	• DRDs in the development pipeline (phase III trial or premarket) or with market authorization in other countries and expected to become available to patients in Canada within the next 1 to 2 years. **OR** • DRDs not classified in either level 1 or 2 but have evidence uncertainties that could be addressed using real-world data generated by registries.

CDA-AMC = Canada's Drug Agency; DRD = drugs for rare diseases; INESSS = Institut national d'excellence en santé et en services sociaux; pCPA = pan-Canadian Pharmaceutical Alliance.

Note: The pCPA plays a key role in the drug approval and reimbursement process in Canada. It publishes a publicly available list of drug products that are under active negotiation, have completed negotiations, or are under consideration for negotiation on the pCPA website. Applicants are encouraged to review the status of relevant DRDs associated with their registry on the pCPA website.

This prioritization is 1 component of the evaluation process, and registries with initiatives at any priority level are encouraged to apply. The complete evaluation criteria are described in Section 4. CDA-AMC reserves the right to contact individual registries to direct them toward initiatives that support specific decision-making needs.

2.4. Funding Amount

The total amount of funding available through this RFP is **$3 million**, of which, applicants may request a maximum of **$300,000 per contract**, inclusive of all costs, expenses, and fees. Registries that were awarded contracts from CDA-AMC in 2024 to 2025 are eligible to apply again, provided they submit newly proposed initiatives that align with the objectives and priorities outlined in this RFP. All registries must propose work that is not currently funded by other organizations, grants, or research projects. CDA-AMC reserves the right to fund individual registries more than the individual maximum amount if identified to meet critical decision-making needs of provincial or territorial partners. For additional guidance about the Financial Proposal, refer to Section 3.2.2 and Appendix 2 for a list of eligible expenses.

3. Submission Guidelines

The RFP process consists of **2 stages.**

1. **Letter of Intent:** Applicants must first submit an initial Letter of Intent (LOI), providing an overview of their registry, identify the relevant DRDs of focus, and a high-level description of the proposed initiatives. The first stage is open to all applicants. Refer to Section 3.1 for detailed guidance for submitting an LOI. Meeting the eligibility criteria or identifying DRDs at any priority level does not guarantee advancement to the next stage.

2. **Full proposal:** Only applicants invited from the LOI stage will proceed to the second stage. This stage requires a submission including a Technical Proposal, Financial Proposal, and accompanying documentation. Applicants must describe in greater detail the proposed initiatives, their alignment with decision-making needs or evidence uncertainties, and their capability to deliver the intended outcomes. Refer to Section 3.2 for detailed guidance about submitting a proposal.

Due to the competitive nature of funding, only a limited number of applicants will be awarded contracts. All contracts will be funded until **March 31, 2026.**

3.1. Letter of Intent

Applicants will be required to complete the LOI template and submit their application by **April 16, 2025.** No additional documents or accompanying information is required. The template requires applicants to provide the following information:

- **Registry name and authorized legal contracting organization**: Clearly state the registry name and the organization legally responsible for the contract.
- **Applicant information:** Provide the names and contact information for up to 3 lead applicant(s), along with a brief summary of their expertise and experience.
- **Overview of registry:** Describe the registry, including:
  - disease(s) covered
  - number of patients captured within registry
  - jurisdictional coverage in Canada
  - any affiliations or connections with other registries (in Canada or international) and clinical sites.
- **Relevant DRDs**: Applicants are required to list specific DRDs that the registry will focus on as part of the proposed initiatives. If the registry has drug information for multiple DRDs, please list the most pertinent and relevant DRDs. Applicants should submit the information listed in Table 3.
- **Overview of proposed initiatives** (300 words): Provide a description of the proposed project, including its objectives and aims.

Table 3: Sample Table Identifying DRDs and Their Priority Designations

DRD name	Indication	Priority designation
Drug name(s)	Disease name	**Level 1, level 2, or level 3** With a short description of status within drug life cycle, for example: • DRD is at pCPA in active negotiation • DRD has Health Canada approval and is under consideration at pCPA.

DRD = drugs for rare diseases; pCPA = pan-Canadian Pharmaceutical Alliance.

3.2. Detailed Proposal Submission

Applicants selected from the LOI stage will be invited to submit detailed proposals. Due to the competitive nature of the RFP and limited funding, only a limited number of applicants will be invited to submit full

proposals. Full proposals will consist of 3 main documents and additional forms confirming compliance with CDA-AMC's rules and regulations. Detailed proposals must be submitted by **May 30, 2025.**

3.2.1. Technical Proposal

Each applicant will be required to submit a maximum **6-page** proposal with the following sections:

- **Detailed registry information (maximum 1 page)**: Provide a detailed description of the registry, including:
  - disease(s) covered
  - patient numbers
  - jurisdictional coverage in Canada
  - affiliations or connections with other registries (in Canada or internationally)
  - structure (e.g., oversight by clinical association or link to clinical site)
  - where applicable, include the registry and lead applicants' experience in generating evidence to inform decision-making in Canada.
- **Relevant DRDs and evidence uncertainties (maximum 2 pages)**: Building on the information provided in the LOI, applicants should describe the registry's capability (i.e., current capacity or infrastructure) to assess specific DRDs and their associated evidence uncertainties. Applicants should provide a high-level summary of the information in Table 4 (1 table per DRD).

Table 4: Information to Include in Proposals Describing the Capacity or Capability of the Registry to Assess Specific DRDs

Domain	Description or detailed information
DRD name	Drug name
Indication	Disease name or therapeutic area
Evidence uncertainties	(Bullet points)
Existing capability of registry to assess DRD	(Bullet points)
Summary of proposed initiatives	(Bullet points)

DRD = drugs for rare diseases.

- **Proposed initiatives (maximum 2 pages)**: Describe, in detail, areas for improving the registry in order that it is better equipped to help generate fit-for-purpose data. Provide a detailed workplan that describes tasks, resources, or other components to deliver on the improvement initiatives and how they would help address corresponding evidence uncertainties for relevant DRDs. Include a list of deliverables and/or expected results that will be completed by the end of the funding contract.
- **Engagement and consultation approach (maximum 0.5 pages)**: Describe the proposed approach to engaging with relevant parties, including:
  - decision-makers, researchers, health system leaders, and/or industry, such as sponsors of specific DRDs

    - patient partners to guide improvements in the registry's design.
- **Risks and mitigation plan (maximum 0.5 pages):** Describe the registry's resources and operational support to manage potential risks that could affect project delivery. Indicate whether the registry will require dependencies such as ethics applications, data sharing agreements, other administrative permissions, and/or the hiring of new staff to complete the proposed work. For each dependency, describe the approach to managing any delays and their potential impact on the overall project completion.

Relevant references may be included in footnotes. Any pages beyond the 6-page limit will not be reviewed.

3.2.2. Financial Proposal

Applicants must complete the Financial Proposal template in Excel (will be provided to applicants who move on to the proposal stage). Scanned copies or other document formats will not be accepted. The proposed budget must not exceed **$300,000.00**.

Costs incurred in preparing or submitting the proposal cannot be included in the budget. For further guidance, refer to the list of eligible expenses in Appendix 2.

3.2.3. Table of Proposed Deliverables and Milestones

Awarded applicants will receive funds based on the portion of work completed at each milestone. Payments will be made upon submitted progress reports demonstrating completed deliverables or expected progress at 4 milestones set for specific dates. If the proposed activities or deliverables are not completed by the milestone date, registries may be permitted to report on the remaining progress at the next milestone or a later date and receive payment for the outstanding portion accordingly. Such requests will be reviewed on an individual basis. No milestone dates can be extended beyond the maximum contract duration of March 31, 2026.

For initiatives or activities proposed throughout the contract period, applicants must provide the expected percentage of completion at each milestone. For example, if an initiative involves collecting new data for 100 patients, the progress toward reaching that goal should be reported at each relevant milestone.

The proposed deliverables and milestones table submitted by applicants will form part of the final contract agreement between CDA-AMC and the registry, should it be awarded a contract. Refer to Table 5 for the format; this table will not count toward the 6-page limit of the Technical Proposal.

Table 5: Sample Table of Contract Milestones to Be Submitted as Part of Detailed Proposals

Milestone	Deliverables or activities completed or expected progress	Payment, %
1 (Month 0)	At contract signing	30%
2 (September 30, 2025)	Details	20%
3 (January 30, 2026)	Details	20%
4 (March 31, 2026)	Details	30%

3.3. Key Dates

Letter of Intent:

- **Launch of RFP:** March 26, 2025
- **Last date to submit questions about LOIs:** April 8, 2025
- **Submission date for LOIs:** Wednesday, April 16, 2025, by 11:59 P.M. (ET)
- **Notification of results for LOIs:** Friday, May 2, 2025, by 5:00 P.M. (ET)

Detailed proposal submission:

- **Last date to submit inquiries about proposals:** May 21, 2025
- **Submission date for proposals:** Friday, May 30, 2025, by 11:59 P.M. (ET)
- **Notification of results for proposals:** Estimated by early July 2025
- **Contract development with awardees:** July 2025 to August 2025

Prospective applicants are encouraged to monitor the CDA-AMC Drugs for Rare Disease website for updates and bulletins. Responses will not be emailed and instead will be posted online for all applicants to review.

3.4. CDA-AMC Requirements

3.4.1. Contracts

Registries awarded through this RFP will be required to execute a CDA-AMC contract for this work. Successful applicants will be required to agree to follow CDA-AMC procedures and use CDA-AMC forms and templates, when provided.

All properly submitted invoices will be paid 30 calendar days after receipt.

3.4.2. Language and Location of Work

Unless stated otherwise, all submitted reports detailing the work are to be prepared in the English language and CDA-AMC will be responsible should any translation be required.

The majority of the work is expected to be performed at the contractor's facilities or operations in Canada.

3.4.3. Funding Period

It is expected that the successful registries selected from this process will commence work as soon as possible, following contract execution. All work must be completed by **March 31, 2026.** No work completed beyond the end date or expenses incurred after the date will be reimbursed. There will be no provision for any no-cost extension for awarded applicants.

4. Application Evaluation and Selection

CDA-AMC will conduct a 2-stage evaluation process to award applications:

Stage 1: Letter of Intent Evaluation

This stage serves as the initial screening to identify registries that meet the eligibility criteria and align with the priorities outlined in this RFP. CDA-AMC will evaluate the LOIs based on:

- **Eligibility:** LOIs will be assessed for alignment with the following criteria:
  - The registry focuses on **rare diseases** as defined by international frameworks (EMA, FDA, and NORD) or diseases with significant unmet needs supported by a clear rationale.
  - The registry is an **active patient registry** that collects observational data and is capable of adding new patients.
  - The LOI identifies relevant **DRDs** and classifies them by priority level as described in Section 2.3.
- **Alignment with RFP objectives:** A high-level assessment of the registry, its proposed initiatives, and potential for informing decision-making needs. This includes an evaluation of how well the registry's goals and capabilities align with the priorities outlined in the RFP.

Only registries that meet the eligibility criteria and align with the objectives in the RFP will be invited to submit a full proposal. However, meeting the eligibility criteria does not guarantee advancement to the next stage.

Stage 2: Full Proposal Evaluation

CDA-AMC will conduct an initial administrative review to ensure each submission meets the requirements outlined in the RFP and described in Section 3. Only submissions that pass this review will proceed to evaluation by a multidisciplinary panel.

The panel will include internal and external experts in rare diseases, real-world evidence, registries, and a person affected by a rare disease. The evaluation will assess the **technical merits** of each application and how well the proposal aligns with the priorities and technical criteria outlined in this RFP.

Submissions will be reviewed and scored based on the evaluation domains described in Table 6. Scores will be compiled, and the panel will deliberate to determine final recommendations. The financial scoring will occur in parallel but independent of the Technical Proposal.

CDA-AMC reserves the right to prioritize funding decisions at its sole discretion, based on alignment with the priorities outlined in this RFP. While submissions will be evaluated and ranked through the established evaluation process, CDA-AMC reserves the right to consider emerging priorities or needs when making the final funding decisions.

Submissions with the lowest cost are not guaranteed to be accepted. CDA-AMC reserves the right not to award funding to any applicants if none sufficiently aligns with the stated priorities or meet the evaluation criteria. All decisions are final.

4.1. Evaluation Domains

Proposals will be evaluated based on specific criteria, which may be subject to change. Applicants may be requested to present specific components of their proposal via a video conference meeting with a subset or full review panel as part of the selection process.

Table 6 describes the evaluation domains and some guiding questions the review panel will be considering. The specific questions and weighting of each domain may be subject to change.

Table 6: Framework for Evaluating Detailed Proposals

Domain	Guiding questions	Weight %
Strategic impact	• Does the proposal demonstrate awareness and alignment with international and Canadian regulatory, HTA, and payer needs to enable meaningful use of registry data? • Does the proposal describe how the proposed initiatives could inform specific decision-making needs about DRDs? • What is the priority level of the identified DRDs? • What is the anticipated impact of the proposed initiatives to address specific decision-making needs? • Are there organizations working in the same or similar disease area, and if so, have there been efforts to coordinate or collaborate?	35%
Methodology and scope	• Does the applicant demonstrate the registry's capabilities to inform existing gaps to better generate fit-for-purpose data? • Are the proposed initiatives for addressing identified gaps well-defined, scoped appropriately, and methodologically sound? • Is the proposed engagement plan comprehensive and impactful? • Are the engagement methods designed to help guide improvements, especially with regards to patient engagement?	30%
Feasibility	• Are the proposed deliverables and milestones clearly described and aligned with the initiatives? • Is the registry equipped with sufficient resources, infrastructure, and personnel to meet the expected timeline?	20%
Sustainability and risk mitigation	• Does the proposal include initiatives that are likely to have sustainable impact beyond the funding period? • Does the proposal identify potential risks or dependencies that could affect project success? • Are appropriate mitigation strategies in place to address specified risks?	5%
Financials (reviewed independently)	• Are the costs and fees appropriate for the proposed work? • Does the proposal meet all financial requirements?	10%

DRD = drugs for rare diseases; HTA = health technology assessment.

4.2. Additional Considerations

While the preceding domains represent the primary focus areas and evaluation criteria, CDA-AMC recognizes the dynamic nature of the disease evidence landscape. Therefore, CDA-AMC remains open to

considering innovative proposals that may fall outside these specified areas but demonstrate strong potential for impact.

The final prioritization of funding will depend on the quality of the submissions received. Applicants should articulate how their project aligns with the RFP's objectives and address the evaluation domains, providing specifics where possible.

Appendix 1: Examples of Fit-for-Purpose Initiatives to Address Evidence Uncertainties and Decision-Making Needs

Table 7: Hypothetical Examples of DRDs and Fit-for-Purpose Initiatives Could Help Inform Evidence of Uncertainties

Context of evidence uncertainty for DRDs	Potential fit-for-purpose initiative(s)	Evidence domain
A new DRD shows promising results in clinical trials and is under negotiation at pCPA. There is uncertainty about the size of the patient population (across jurisdictions in Canada) that could benefit from the new or emerging DRD. **Question of interest:** What is the anticipated number of patients in Canada who could benefit from the new DRD?	• Expanding registry coverage to include additional provinces and territories or pan-Canadian clinical sites. • Improving data collection within the registry for demographic and clinical characteristics to better identify the number of patients that are eligible for the DRD. • Improving governance structures and policies to enable data acquisition from other data sources to identify additional patients.	Utilization and implementation Health system impact
A new DRD shows promising results in clinical trials for a targeted group of patients. There is uncertainty about the clinical effectiveness of the DRD among additional patient groups for which trial data were limited. **Question(s) of interest:** Jurisdictions in Canada are assessing the feasibility and appropriateness of expanding coverage. Based on emerging RWD, what is the clinical effectiveness of the DRD for additional patient groups underrepresented in the trials?	• Expanding registry coverage to include additional patient populations of interest. • Improving registry policies to enhance consent protocols. • Aligning outcomes data with clinical trial data and outcomes of interest to decision-makers. • Engaging with patient partners to ensure data collection is person-centred and minimizes administrative burden.	Clinical effectiveness Safety Health system impact
The clinical evidence about a specific DRD reviewed by CDA-AMC showed that a high proportion of patients discontinued treatment. The review identified uncertainty about whether the discontinuation rate would be similar in routine clinical practice. **Question(s) of interest:** How many patients treated with the DRD discontinue treatment in Canada? Based on RWD, what patient characteristics are associated with discontinuation?	• Enhancing the collection of longitudinal treatment data (e.g., start dates, adherence, event dates for switching or discontinuation, and reasons for discontinuation). • Improving data collection related to patient reported outcomes and patient characteristics. • Engaging with patient partners to improve the completeness and relevance of patient reported outcomes data.	Utilization and implementation Health system impact
The clinical evidence about a specific DRD reviewed by CDA-AMC included an indirect treatment comparison where patients in an observational study were reweighted to match patient characteristics in the pivotal trial. The review identified that there were important effect-modifying or prognostic factors that were not included in the matching procedure. **Question(s) of interest:** Is there RWD available to assess the effectiveness of	• Conducting a registry data review to assess the availability of relevant effect-modifying or prognostic factors. • Improving the ability of the registry to collect important effect-modifying or prognostic factors, including considerations for how information could be collected retrospectively and/or prospectively.	Clinical effectiveness Safety

Context of evidence uncertainty for DRDs	Potential fit-for-purpose initiative(s)	Evidence domain
the DRD with appropriate consideration of important effect-modifying or prognostic factors?		

CDA-AMC = Canada's Drug Agency; DRD = drugs for rare diseases; HTA = health technology assessment; pCPA = pan-Canadian Pharmaceutical Alliance; RWD = real-world data.

Note: These hypothetical examples illustrate how applicants may propose initiatives to align with specific or anticipated decision-making needs. These examples are intended for guidance only and do not represent any specific DRDs.

Appendix 2: Guide to Eligible Expenses

Eligible

1. Stipends, honorariums, salaries, and benefits:
    - Salaries for project leads, co-leads, and principal investigators are eligible, provided they are justified in the Financial Proposal, including the amount of time dedicated to the project and how their involvement supports project objectives.
2. General office and administration, including institution overhead, to a maximum of 25%; anything more than 25% will not be honoured. This expense category includes, but is not limited to:
    - equipment, IT, support services, data retention, and software licences beyond what is typically provided by the host institution
    - costs for equipment maintenance and service contracts, training of staff operating equipment and/or software, and extended warranty for equipment limited to the life of the agreement
    - computers, essential hardware, and/or software required for project operations. These should be listed under the “Tangible Items” section in the Financial Proposal, specifying unit costs and quantities.
3. Consulting costs (third party), including platform or database transitions:
    - If switching to a new platform or database, the quoted cost should be included under “Consulting Costs (Third Party).” Internal staff time spent implementing or learning the platform should be categorized under “Salaries, Stipends, and Benefits.”
4. Engagement activities:
    - Costs related to engaging and/or consulting with patient partners, industry experts, or other relevant parties for the development of the registry.

Ineligible

1. Discretionary severance or separation packages.
2. Parental leave and leave of absence.
3. Benefits for trainees.
4. Any project costs that are funded, will be funded, or will be reimbursed by any third party, ministry agency, or organization of a federal, provincial, or territorial government.
5. Travel costs other than on an exception basis and with preapproval by CDA-AMC.
6. Legal costs for general policy development or consenting procedures, unless specifically incurred as professional fees for legal services performed by a lawyer.
7. Indirect costs beyond the allowable institutional overhead percentage (25%).
8. Flat-fee consulting costs must be itemized in the Financial Proposal and cannot be estimated as an hourly rate unless explicitly structured that way in the contract.

Appendix 3: Definitions of Key Terms

The following definitions are applicable to this RFP.

Table 8: Definitions of Key Terms Used in This RFP

Term	Definition
Application	All documentation submitted by an applicant in response to the RFP.
Applicant	Refers to the entity, respondent team, or joint venture submitting a response to this RFP.
Contract	Refers to the aggregate of: (a) the Agreement, including any schedules attached at the time of execution; (b) any Statements of Work executed during the term of the contract; (c) the RFP, including any addenda; (d) the Application; and (e) any amendments executed in accordance with the terms of the Agreement.
Decision-grade evidence	High-quality, relevant, and reliable evidence capable of informing regulatory, health technology assessment, or reimbursement decisions.
Deliverable(s)	Refers to everything developed for, or provided to, the agency in the course of performing under the contract or agreed to be provided to the agency under the contract by the registry or the registry's personnel, as further defined, but not limited by Schedule 1, including, but not limited to, any goods or services or any and all intellectual property and any and all concepts, techniques, ideas, information, documentation, and other materials, however recorded, developed, or provided.
Drugs for rare diseases (DRDs)	Refers to pharmaceuticals used to prevent, diagnose, or treat rare diseases. These drugs may be identified by 'rare' or 'orphan' drug designations from regulatory bodies such as the European Medicines Agency, FDA, or the National Organization for Rare Disorders. DRDs often address high unmet medical needs and may be eligible for regulatory incentives to support development and access.
Fit-for-purpose registry enhancements	Targeted improvements to registry infrastructure, data collection, or methodologies that enhance its ability to generate decision-grade evidence for health technology assessment, regulatory, and reimbursement decision-making.
Funded registry	Refers to an applicant selected by CDA-AMC to receive funding and enter into an agreement.
Health technology assessment (HTA)	Includes independent assessments of drugs and other health technologies. Assessments consist of one, or a combination, of the following: an environmental scan, a rapid review, a clinical (or systematic) review, an economic review, or a review of patient perspectives. They may also include an assessment of the legal, ethical, social, implementation, environmental, and policy implications of a specific health technology. Recommendations on the appropriate use of health technologies are provided where applicable.
Milestones	Defined stages in the contract where specific deliverables must be completed for funding disbursement.
Patient or disease registry	An organization that collects observational data to evaluate specified outcomes for a population defined by a particular disease, condition, or exposure. These registries serve multiple purposes, including research, clinical care, and informing policy. To be considered active and eligible, registries must be capable of enrolling new patients and should not rely exclusively on retrospective datasets. Registries should aim to collect data that can be shared and used to inform decision-making within publicly funded health systems.
Patient engagement	The involvement of patients, caregivers, or patient organizations in registry activities, ensuring their perspectives contribute to data collection, governance, and decision-making.

Term	Definition
Rare diseases	A rare disease is a condition that affects a small percentage of the population. Definitions vary by jurisdiction, but rare diseases are generally identified based on prevalence thresholds set by regulatory bodies. For example, the European Medicines Agency defines a rare disease as affecting fewer than 1 in 2,000 people. The US FDA defines it as affecting fewer than 200,000 people in the US, a definition also recognized by the National Organization for Rare Disorders. Many rare diseases are serious, chronic, and life-threatening, often with limited treatment options.
Real-world data **(RWD)**	Data related to patient status and/or the delivery of health care collected from a variety of sources, and can include electronic medical records, clinical and disease registries, and administrative databases.
Real-world evidence **(RWE)**	Evidence about the use, safety, effectiveness, and costs of health technologies that are derived from real-world data.
Reimbursement reviews	Refers to drug funding recommendations to Canada's federal, provincial, and territorial public drug programs. CDA-AMC's reimbursement recommendations are based on comprehensive assessments of clinical and pharmacoeconomic evidence, as well as input from pharmaceutical companies, clinicians, and patient groups.
RFP	Request for Proposal

CDA-AMC = Canada's Drug Agency.

Canada's Drug Agency
L'Agence des médicaments du Canada
Drugs. Health Technologies and Systems. Médicaments, technologies de la santé et systèmes.

Canada's Drug Agency (CDA-AMC) is a pan-Canadian health organization. Created and funded by Canada's federal, provincial, and territorial governments, we're responsible for driving better coordination, alignment, and public value within Canada's drug and health technology landscape. We provide Canada's health system leaders with independent evidence and advice so they can make informed drug, health technology, and health system decisions, and we collaborate with national and international partners to enhance our collective impact.

Questions or requests for information about this report can be directed to Requests@CDA-AMC.ca.