Canada's Drug Agency
L'Agence des médicaments du Canada

April 2025

Bulletin #02: RFP Questions and Answers

File #C-242506400: CDA-AMC Rare Disease Registry Request for Proposals 2025–2026

Note by CDA-AMC: Questions may have been edited for clarity or to maintain confidentiality.

Question #1 received April 3, 2025

Reference RFP 2.1 Eligibility Criteria

Question: Our organization is an international nonprofit based outside of Canada. We have established partnerships with researchers in Canada and our rare disease registry includes Canadian data. However, we do not have a physical office or Canadian bank accounts. Are we eligible to apply for this opportunity?

Answer: While international organizations may apply, the eligibility criteria requires that recipients be capable of entering into a contract with the contracting authority in Canada. This includes the ability to receive payments in Canadian dollars, which typically necessitates having a Canadian bank account. We encourage international applicants to consider partnering with organizations based in Canada to ensure compliance with administrative and contractual requirements.

Question #2 received April 4, 2025

Reference RFP Appendix 1: Fit-for-Purpose Initiatives

Question: In Appendix 1, there is a reference to potential initiatives such as “improving governance structures and policies to enable data acquisition from other data sources to identify additional patients.” Could you provide examples of the types of data sources that could be leveraged to identify additional patients for a registry?

Answer: Examples of other data sources that could support patient identification include:

- provincial or territorial administrative health data (e.g., hospital discharge abstracts, billing claims)
- laboratory and diagnostic data repositories
- newborn screening programs
- clinical databases specific to diseases, maintained by academic institutions or provincial health authorities
- international registry collaborations and data-sharing initiatives
- data from genetic testing laboratories, as appropriate and in compliance with privacy regulations.

Any use of these data sources must comply with ethical and privacy requirements, including necessary data-sharing agreements and patient consent where required.

Question #3 received April 7, 2025

Reference RFP Section 3. Scope of Work

Question: Our registry enrols a small number of patients annually with a rare, acute condition and currently focuses on immediate medical therapy. We are seeking funding to enhance the registry to enable longer-term follow-up after standard treatments in Canada and to compare outcomes, such as survival and morbidity, with patients receiving alternative therapies in Canada and internationally (we have formal

relationships with international registries). Previously, a treatment relevant to this condition was reviewed but not funded due to insufficient Canadian real-world data, particularly related to variations in clinical practice. Would it be within scope to apply for funding to extend our registry for longer-term follow-up and comparative analysis to better inform future funding decisions?

Answer: Expanding an existing registry to facilitate longer-term follow-up and conduct comparative analyses falls within the scope of this funding opportunity, especially if it addresses identified evidence gaps. Proposals should clearly describe how the expanded data collection will provide meaningful insights into real-world outcomes, such as morbidity and mortality, and how it will support clinical and policy decision-making in the context in Canada.

Question #4 received April 1, 2025

Reference RFP Section 2.1 Eligibility Criteria

Question: We are a private data management company with significant experience managing registry data. Could we apply as an independent applicant, or would we be required to partner with clinical investigators or other organizations?

Answer: Private data management companies may apply as independent applicants if they are the stewards of the registry and have full responsibility for its governance, use, and improvement. Eligibility is not based on the type of organization, but on whether applicants can clearly show how their proposed work will advance the objectives of the funding opportunity. We encourage all applicants to describe their governance model and explain how their activities will support the goals of this funding program.

Question #5 received April 7, 2025

Reference RFP Section 3. Scope of Work

Question: Can you please confirm whether initiatives focused on specific evidence gaps in pediatric cancers would be eligible for this funding opportunity? Additionally, for very rare conditions, is it acceptable to include data collected retrospectively, or is prospective data collection required?

Answer: Yes, pediatric cancer indications are eligible for this competition. Additionally, the inclusion of retrospective data collection is acceptable, especially in the context of very rare conditions where prospective enrolment may be challenging. Proposals should explain the rationale for using retrospective data, detail how data quality will be ensured, and describe how the approach will help address evidence gaps for decision-makers.

Question #6 received April 8, 2025

Reference RFP Section 3. Scope of Work

Question: Is there an opportunity in this funding to support rare disease registries that are not exclusively focused on drug therapies?

Answer: The primary focus of this funding opportunity is to support registries that can generate real-world data to inform decisions about drug therapies for rare diseases. This includes conventional pharmaceuticals as well as advanced therapies, such as gene or cell therapies, that are regulated as drugs.

Registries that are not focused on drug therapies may still be eligible if they can clearly demonstrate how their data will support decision-making related to drug products. For example, registries collecting natural history data or monitoring other interventions may still be relevant if they anticipate contributing to future drug evaluations, or if there is an expected therapy in development.

Proposals should clearly outline any anticipated drug products, expected regulatory timelines, and associated evidence gaps that the registry aims to address. Applications that do not include a drug component or a clear link to drug-related decision-making are unlikely to be competitive under this funding call.

Question #7 received April 2, 2025

Reference RFP Section 3. Scope of Work

Question: Can the funds be used to support enhanced data collection for data that will inform decision-making on the drug?

Answer: The funding can be used to support enhanced data collection, provided the activities are aligned with the purpose of this funding opportunity. Specifically, the data collection should aim to fill identified evidence gaps and produce information that will be relevant and useful for regulatory, reimbursement, or policy decisions concerning drugs for rare diseases. Proposals should clearly explain how the additional data will support decision-making processes. All data collection activities must also comply with applicable ethical, privacy, and data governance standards.

Question #8 received April 10, 2025

Reference RFP Section 2.1 Eligibility Criteria

Question: We understand that applicants to the CDA-AMC Rare Disease Registry Request for Proposal (RFP) 2025–2026 must have the capacity to manage a contract and carry out the proposed work. We are seeking clarification on the specific requirements for the funding recipient in situations where registry governance is shared.

In our case, the registry involves both a patient organization and an academic institution. The academic institution serves as the data steward and research ethics lead, while the patient organization provides leadership through participation on the registry steering committee and plays a key role in guiding patient-oriented activities.

Given this governance structure, could the funding recipient be the patient organization, or is it required that the data steward organization serve as the funding recipient?

Answer: As outlined in the RFP, all applicants must have the capacity to manage a contract with the contracting authority and deliver the proposed work. While there is no requirement that the funding recipient be the data steward of the registry, the applicant organization must be able to fulfill the contractual obligations, including financial management, reporting, and oversight of activities described in the proposal.

If the patient organization has this capacity, they may apply as the funding recipient. Applicants are encouraged to clearly describe in their proposal the governance structure of the registry and how the roles and responsibilities of the data steward and patient organization will support the successful delivery of the project.

For example, in the previous funding cycle, recipients included a range of organization types, such as academic and research institutions (e.g., McMaster University, Hospital for Sick Children, and the University of British Columbia) as well as national patient organizations and health-focused nonprofits (e.g., Fighting Blindness Canada, Cystic Fibrosis Canada, and the World Federation of Hemophilia).

These examples reflect the flexibility of the funding model, provided the applicant organization can manage the contract and carry out the work described in the proposal.

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