



Bulletin 1

RFP Questions and Answers

File #C-2401280 Rare Disease Registry Funding

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Note: Questions may have been edited for clarity or to maintain confidentiality. Answers will be provided in the language of the original submission.

Question 1 received March 28, 2024

Reference RFP Section 2.2: Applicant Eligibility

Question: Je viens de tomber sur l'Appel à Projet "financement de l'ACMTS destiné aux registres de maladies rares." Avant de me lancer tête baissée dans la préparation d'une éventuelle lettre d'intention, je voulais savoir si les registres français étaient éligibles?

English translation:

Question: I have just come across the CADTH Request for Proposal (RFP) for rare disease registry funding. Before jumping into the process of preparing a Letter of Intent, I wanted to know whether French registries were eligible?

Answer: *French translation:*

Le financement des registres à l'international, comme ceux qui sont gérés à l'étranger, est évalué au cas par cas. Les critères déterminants à prendre en compte sont notamment la capacité du registre à saisir des données sur les personnes vivant au Canada, la rareté de la maladie ou un besoin non comblé important, de même que l'incidence que le registre pourrait avoir, à court terme, sur la prise de décisions relatives à la santé au Canada.

Answer:

International registries, including those operated from countries outside of Canada, are considered for funding on a case-by-case basis. The critical criteria for consideration include the registry's ability to capture data on participants living in Canada, the disease's rarity or significant unmet need, and the registry's potential to impact Canadian health care decisions within a short time frame.

Question 2 received March 28, 2024

Reference RFP Section 2.2: Applicant Eligibility

Question: If a national registry with detailed clinical annotations for a rare disease faces funding gaps for desired improvements and additional testing, is it eligible for this funding opportunity?

Answer: A national registry focused on a rare disease seeking funding for improvements or additional testing to enhance data quality is eligible for this funding opportunity. The funding is aimed at registries that can demonstrate a significant impact, especially those related to diseases with emerging therapies expected in the next 2 to 3 years.



Question 3 received March 27, 2024

Reference RFP Section 2.2: Applicant Eligibility

Question: Would enhancements to a general registry that introduces a rare disease component, such as the inclusion of rare cancers for more focused targeting, qualify for this funding?

Answer: Because there is no formal definition of “rare” in Canada, if a disease is considered rare by the FDA and/ or European Medicines Agency (EMA), we will also consider it rare. Beyond the FDA and EMA rare disease definitions, the disease may be considered rare if it has a substantial unmet need. Cancers that are rare may fall within that definition.

Question 4 received March 28, 2024

Reference RFP Section 3.2.2: Financial Proposal Instructions

Question: What is the range of grant sizes being offered, and is there a maximum budget limit for projects concluding by March 2025?

Answer: The funding aims to support a diverse set of rare disease registries in Canada, with allocations based on each project’s scope, potential impact, and specific improvements proposed. The goal is to distribute funds across many registries to maximize their impact on rare diseases within the given time frame until March 2025.

Question 5 received March 27, 2024

Reference RFP Section 3.1: Proposal Components

Question: Could you provide guidance on the expectations for the Letter of Intent (LOI), including the page limit and key content required, and clarify if project costing should be included in the LOI or is it only required in the final submission?

Answer: The Letter of Intent (LOI) should be no more than 2 pages long and describe how the organization meets the eligibility requirements, the proposal’s overall objectives, and the organization’s capacity to achieve specific goals within the time frame. The LOI serves as an initial dialogue to understand the project’s alignment with the funding goals. Project costing details are not required at this stage and should be included only in the final submission. Please refer to the RFP for full details.

Question 6 received April 2, 2024

Reference RFP Section 3.2.1: Technical Proposal Preparation Instructions

Question: For a registry operated without an academic lead site, and where the data governance is managed by a steering committee rather than a legal entity with funding and data sharing agreements managed by the custodian organization, who should be listed as the primary applicant or team lead for the funding proposal?



Answer: For a registry without an academic lead site that is managed by a steering committee without legal entity status, the primary applicant or team lead should be the organization or individual that can provide oversight and accountability for the use of funds and the project's execution. This could be the custodian organization managing the data sharing agreements.

Question 7 received April 3, 2024

Reference RFP Section 2.2: Applicant Eligibility

Question: Could you clarify if a biomedical technology organization that develops diagnostic algorithms for rare diseases, and whose technology is used in hospitals for patient screening and as a decision support tool for doctors, is eligible to apply for the funding?

Answer: Based on the specifics provided, a biomedical technology organization or information platform that is not focused on a particular rare disease(s) and does not have a direct link to Canadian health care may not meet the eligibility criteria for funding. This funding prioritizes projects with a clear impact on Canadian health care, specifically targeting rare diseases and therapies.

End of file.