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

April 2025

Bulletin #01: 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 March 26, 2025

Reference RFP Table 1: Timeline of Key Events Related to the RFP

Question: I'm not free to attend the webinar. Will a recording be available?

Answer: The webinar recording and the slides have been posted on our website: https://www.cda-amc.ca/drugs-rare-diseases-funding-opportunity.

Question #2 received March 26, 2025

Reference RFP Section 2.1 Eligibility Criteria

Question: Are international rare disease registries based outside of Canada, but include patients in Canada, eligible for funding?

Answer: Funding will be provided only to Canadian or international organizations with a Canadian site or account and are actively collecting data on patients in Canada. If there is no Canadian organizational presence, it will not be possible to proceed with a contract, as the applicant must be able to receive and administer funds through a Canadian institution.

Question #3 received April 1, 2025

Reference RFP Section 2.1 Eligibility Criteria

Question: We received combined funding last year for 2 of our registries. At this stage, the goals of the funding for the 2 registries will start to become divergent. Can we go ahead and submit 2 applications outlining the individual needs of the registries at this stage?

Answer: An individual registry can submit only 1 application. If an organization has 2 distinct registries, each registry should submit their own independent application and clearly outline the unique objectives and scope of that registry's proposed work.

Question #4 received April 1, 2025

Reference RFP Section 2.1 Eligibility Criteria

Question: Our registry focuses on a rare disease for which there are currently no drugs available. However, there are advances in technologies and devices for which we could use the registry data to advance the regulatory approval and reimbursement. Would this type of initiative be eligible for funding?

Answer: The focus of this funding is guided by the National Strategy for Drugs for Rare Diseases and aims to enhance the capability of registries to evaluate drug products. This includes both conventional drugs and advanced therapies such as gene or cell therapies, that are regulated as drugs.

We recognize that in some rare disease areas, devices or other health technologies may be the most relevant emerging interventions. While the primary focus is on drug products, applicants may choose to

submit a proposal involving a nondrug technology if they can clearly demonstrate a relevant evidence gap and articulate how the real-world data generated would support decision-making.

Applicants should use their proposal to identify any anticipated therapies, their expected regulatory timelines, and the associated evidence uncertainties.

Question #5 received April 2, 2025

Reference RFP Section 2.2 Focus On Fit-For-Purpose Enhancements

Question: Is funding available to establish a new registry, even if it's intended to support future clinical trials and doesn't yet have a specific drug target?

Answer: Registries at any stage of development are eligible to apply for funding. This includes registries that are in the initial stages of development or have been established for many years. While registries in earlier stages of development are eligible, Canada's Drug Agency (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. Please note that all proposed initiatives must be completed by March 31, 2026, therefore early-stage registries must ensure their proposed timelines and deliverables are achievable within that period.

Question #6 received April 2, 2025

Reference RFP Section 2.2. Focus On Fit-for-Purpose Enhancements

Question: Could you clarify how proposals involving multiple medications for rare diseases fit within the funding call? Is this more of an observational study of how the different medications are used (usually off label) and their efficacy in our rare diseases?

Answer: This request for proposal (RFP) supports proposals focused on specific drugs for rare diseases (DRDs) with identified evidence gaps. While registries may include data on multiple therapies, the proposal should clearly identify which DRDs and associated initiatives addressing evidence uncertainties will be the focus of initiatives, and how this work aligns with regulatory, health technology assessment (HTA), and payer decision-making.

This funding is intended to support the development of registries in Canada to bolster their infrastructure and ability to generate high-quality data related to specific therapies. This initiative is not intended to fund research studies or observational studies, even if they include off-label use. Proposals must clearly demonstrate how the work aligns with the purpose and priorities of the RFP.

Question #7 received April 2, 2025

Reference RFP Section Not Applicable

Question: We submitted a full proposal last year that was ultimately unfunded. Would it be possible to see the reviewers' feedback so we can address identified weaknesses in the proposal we plan to submit this year?

Answer: Feedback about the previous year's applications is not available. We encourage applicants to ensure their submission aligns with the 2025 to 2026 RFP requirements. As both the requirements and evaluation approach have been updated this year, focusing on the current RFP will be the most helpful in preparing a strong proposal.

Question #8 received April 2, 2025

Reference RFP Section 2.3. Prioritization of Initiatives

Question: Given the fact that registries' greatest value is in identifying therapies that are being used off label effectively, can you clarify why Level 1 and 2 DRDs are being prioritized?

Answer: The priority levels do not reflect a judgment about the *potential value* of a registry, nor do they preclude funding for important real-world insights, including off-label use. Rather, the levels are designed to reflect how closely a proposal aligns with *known and time-sensitive evidence uncertainties* identified by regulatory, HTA, and payer bodies in Canada.

Therapies across all levels will be considered and may be competitive if they align with the aims and objectives of the RFP. Level 1 and 2 DRDs are more likely to have identified evidence uncertainties that can inform specific and time-sensitive decision-making needs (within the 1 to 2 years time horizon), especially related to reimbursement decisions. Applicants proposing work focused on Level 3 DRDs are encouraged to clearly demonstrate the relevance of the drug, the associated evidence uncertainties, and how enhancing the registry's capacity could support future decision-making needs.

Question #9 received April 2, 2025

Reference RFP Section 3.1 Letter of Intent

Question: If our registry includes multiple rare diseases, each with its own therapy, can we apply for the funding with more than 1 targeted drug or disease?

Answer: Applicants may propose initiatives that address multiple rare diseases and associated therapies, if the proposal clearly identifies the specific DRDs and the evidence uncertainties within their registry.

In the Letter of Intent (LOI), please list the most pertinent and relevant DRDs that align with the proposed initiatives and evidence uncertainties and how the work contributes to the objectives of the RFP.

Question #10 received April 2, 2025

Reference RFP Section 2.3. Prioritization of Initiatives

Question: If a drug has received a do not list recommendation and was not subject to negotiations at the pan-Canadian Pharmaceutical Alliance **(**pCPA), can a registry still apply for funding to address the evidence gaps identified for a particular product?

Answer: A DRD that may have received a do not list recommendation will be classified as a Level 3 drug under this RFP. An applicant can propose initiatives to address evidence uncertainties related to that drug.

However, as Level 3 DRDs are not currently undergoing reimbursement negotiations, proposals must clearly demonstrate the relevance of the evidence gap, how the data generated could inform future decision-making, and why addressing these uncertainties is important in the current context. Proposals involving Level 3 DRDs will be assessed alongside broader strategic considerations.

Question #11 received April 2, 2025

Reference RFP Section 2.4 Funding Amount

Question: Can registries that were awarded funding last year reapply this year?

Answer: Registries that were awarded contracts from CDA-AMC in 2024 to 2025 are eligible to apply again. Applicants may propose new initiatives or a continuation of previously funded work, if the proposal aligns 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. 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 to 2026 fiscal year are required to declare their funding.

Question #12 received April 2, 2025

Reference RFP Section 2.2. Focus On Fit-for-Purpose Enhancements

Question: What do you recommend for identifying uncertainties for Level 2 DRDs that are in active negotiation but do not yet have publicly available CDA-AMC or l'Institut national d'excellence en santé et en services sociaux (INESSS) reviews? Is published literature the best source in that case?

Answer: For DRDs without completed or published CDA-AMC or INESSS reimbursement reviews at the time of application, applicants may list evidence uncertainties described in published literature, provided the source is mentioned.

Note that while CDA-AMC reviews represent a source of information about evidence uncertainties, they may not list all evidentiary uncertainties or reflect emerging gaps. Applicants are encouraged to identify uncertainties that are most relevant to informing regulatory, HTA, and payer decision-making.

Question #13 received April 2, 2025

Reference Letter of Intent Application Form

Question: Can a single registry submit 2 different LOIs if there are multiple questions that could be addressed?

Answer: An individual registry can submit only 1 application. If an organization has 2 distinct registries, each registry should make their own independent application. If there are multiple questions that can be addressed within a single registry, funding will still be limited to a maximum of $300,000 for that 1 registry.

Question #14 received April 2, 2025

Reference RFP Section Appendix 3 Definitions of Key Terms

Question: Could you please explain what is meant by the term “fit for service?”

Answer: Fit-for-purpose (fit for service is not a term used in the RFP) refers to targeted improvements to registry infrastructure, data collection, or methodologies that enhance its ability to generate decision-grade data for HTA, regulatory, and reimbursement decision-making.

Question #15 received April 2, 2025

Reference RFP Section 3. Submission Guidelines

Question: We are developing a registry for children with anorectal malformations and Hirschsprung disease, but there are currently no drugs or medications available. Would this disqualify us from applying?

Answer: The focus of this funding is driven by the National Strategy for Drugs for Rare Diseases and is intended to bolster the capability of registries to assess drug products. This includes drugs with regulatory approval or anticipated to have regulatory approval.

If there are no drug therapies currently available or expected in the near future for your disease area, the application may be less competitive. However, if your registry could support future drug-related decision-making, we encourage you to clearly describe any anticipated drug products, relevant timelines, and associated evidence uncertainties in your proposal.

Question #16 received April 2, 2025

Reference RFP Section 2.1. Eligibility Criteria and 3.4. CDA-AMC Requirements

Question: Can funding be spent outside of Canada, and if so, is there a limit?

Answer: All funds must be spent in Canada and support initiatives in Canada.

Question #17 received April 2, 2025

Reference RFP Section 2.1.1. Additional Requirements

Question: Can the DRD be an already approved drug that is now seeking a label extension?

Answer: A lack of data about off-label drug use would be an example of an identified uncertainty. A registry may propose specific initiatives to bolster its capacity to examine off-label drug use.

This funding is not intended to fund observational studies or research. This funding is separate from existing reimbursement review processes, and the outcomes of this funding do not influence reimbursement recommendations. It will be important to demonstrate how the proposed registry initiative will directly impact decision-making in the areas outlined in the RFP.

Question #18 received April 2, 2025

Reference RFP Section 2.1. Eligibility Criteria

Question: For an established registry that collects data globally, would the grant only apply to work applied in Canada? In addition, what is the guidance for international registries that would like to include data from all participants to answer the project questions?

Answer: This funding is provided through a contract, not a grant, and is available only to Canadian organizations or international organizations with a Canadian site or account that are collecting data on patients in Canada.

Although registries may have data from international patients, the funded contract will be provided only to focus on improving the quality, completeness, and use of patient data in Canada. Any use of international data must be clearly justified as supporting decision-making in the Canadian context.

Importantly, this funding is not intended to support the conduct of observational studies, pooled analyses, or research projects. It is intended to support enhancements to registry infrastructure and processes that address clearly identified evidence gaps relevant to regulatory, HTA, or payer decision-making.

Question #19 received April 2, 2025

Reference RFP Section 2.3. Prioritization of Initiatives

Question: If a drug is entering phase III trial in the US or Canada, is that drug eligible as the DRD associated with the real-world evidence that we are collecting?

Answer: DRDs that are in active or completed phase III trials will be classified as Level 3. They may be considered for funding; however, the application must demonstrate specific evidence uncertainties or evidence gaps not addressed in clinical trials.

Question #20 received April 2, 2025

Reference RFP Section 2.3. Prioritization of Initiatives

Question: What level of priority would be assigned to a DRD that was approved before 2022? Would an application involving such a drug still be considered?

Answer: The 3 levels are based on the pCPA negotiation status and not the drugs' regulatory approval date. DRDs that completed pCPA negotiations before 2022 will be classified as Level 3. In that case, to be competitive, applications must clearly describe the relevant evidence uncertainties related to that specific DRD and explain how the proposed initiatives may bolster the registry's capabilities to address decision-making needs.

Question #21 received April 2, 2025

Reference RFP Section 2.2. Focus On Fit-for-Purpose Enhancements

Question: Can we apply for funding to improve our registry to evaluate the outcomes of a current therapy not approved by CDA-AMC, by comparing patients in Canada to others around the world who receive the drug? We need to get Canadian data to support its use.

Answer: Yes, you may apply for funding to support initiatives focused on evaluating outcomes related to a therapy that has not been assessed or has received a do not list recommendation. Such therapies would be considered Level 3 DRDs under the RFP prioritization framework.

Applicants can propose initiatives that aim to address evidence uncertainties related to these therapies — provided the registry is actively collecting data on patients in Canada. However, please note that this funding is not intended to support standalone observational studies or research projects. The purpose of the funding is to enhance registry infrastructure and data quality to generate real-world evidence that is fit-for-purpose for decision-making.

Importantly, this funding process is separate from CDA-AMC's reimbursement review activities. Participation in this initiative does not influence or replace formal drug assessment or listing decisions.

Question #22 received April 2, 2025

Reference RFP Section 2.3. Prioritization of Initiatives

Question: Do the DRD levels apply to drugs that have been approved for other indications, or only for their indication for the rare disease?

Answer: The DRD levels apply specifically to the rare disease indication and based on the drug's status listed on the pCPA website for the specific indication, and not other conditions for which the drug may also have received a listing. A drug may be approved or reimbursed for other conditions, but its classification for this funding initiative is determined solely by its status in the rare disease context.

Please note that a single drug may have multiple rare disease indications, each with its own pCPA status, CDA-AMC recommendation, and associated evidence uncertainties. Applications should identify and describe the specific indication they aim to target in their proposal as well as the evidence uncertainties that could be specifically addressed by the data improvements.

Question #23 received April 2, 2025

Reference RFP Section 2.2. Focus On Fit-for-Purpose Enhancements

Question: Could this funding opportunity be used to support a postmarketing surveillance study for an approved drug?

Answer: This funding call is not intended to support observational studies or research projects. However, enhancing a registry's capability for postmarket surveillance and analysis may be considered for funding, provided applicants can demonstrate specific decision-making needs. All applications should describe

evidence uncertainties related to specific DRDs and how that information may be used for informing decision-making needs.

Question #24 received April 2, 2025

Reference RFP Section 2.3. Prioritization of Initiatives

Question: To clarify, could Level 3 potentially receive funding?

Answer: Registries focusing on DRDs at all levels are eligible for funding, provided the application describes evidence uncertainties related to specific DRDs and how that information may be used for informing decision-making needs. It is entirely possible that initiatives proposing data improvements for Level 3 products will be funded and will be dependent on the number and quality of proposals received overall.

Question #25 received April 2, 2025

Reference RFP Section 2.1. Eligibility Criteria

Question: Are recipients limited to academic applicants?

Answer: No, there is no requirement for applicants to be affiliated with academic institutions. Eligible applicants may include a wide range of organizations, such as academic institutions, health care organizations or hospital networks, nonprofit or charitable organizations, patient organizations, and international organizations with a Canadian site or institutional account. All applicants must have the capacity to manage a contract and carry out the proposed work.

While a registry does not need to be owned or governed by an academic institution, the overarching goal of this funding initiative is to strengthen the generation of decision-grade evidence. This often involves enabling access by researchers — particularly those in academic settings — and contributing to broader evidence ecosystems. Registries should be prepared to support this by making their governance models and data collection methodologies transparent and accessible.

Question #26 received April 2, 2025

Reference RFP Section 1.2. Alignment With National Strategy for Drugs for Rare Diseases

Question: Considering that rare disease treatment involves various interventions other than pharmaceutical treatments, can rare disease registries on surgically correctable congenital malformations apply?

Answer: The focus of this funding is driven by the National Strategy for Drugs for Rare Diseases and is specifically intended to bolster the capability of registries to assess drug products. These drugs may be drugs with regulatory approval or anticipated to have regulatory approval.

Although other interventions, such as surgeries, may play an important role in managing certain rare diseases, applications that do not include a drug component will likely not be competitive. If applicable, please demonstrate in your application what specific drug products may be anticipated for regulatory approval, their timelines, and associated evidence uncertainties.

Question #27 received April 2, 2025

Reference RFP Section 1.2. Alignment With National Strategy for Drugs for Rare Diseases and 2.2. Focus On Fit-for-Purpose Enhancements

Question: In cases where there are currently no available drugs for a group of rare diseases, but there is emerging evidence, would a registry still be eligible to address uncertainties and evidence gaps?

Answer: The focus of this funding is driven by the National Strategy for Drugs for Rare Diseases and is intended to bolster the capability of registries to assess drug products and their associated evidence uncertainties. The scope includes drugs with regulatory approval or anticipated to have regulatory approval.

Registries focused on conditions without a specific drug in the pipeline (phase III or later) will likely not be competitive. This funding is not intended to support basic science research, clinical trials, or drug development. To be eligible and competitive, applicants must clearly identify a target drug and describe the associated evidence uncertainties the registry will address

Question #28 received April 2, 2025

Reference RFP Section 3.2.1 Risks and Mitigation Plan

Question: In many centres, it takes many months to get Research Ethics Board (REB) approval for any type of project, including registries. Given the short delivery timeline (less than 1 year), should we assume that this initiative prioritizes registries that already have all these regulatory issues settled as of July for all participating centres?

Answer: Applicants are not required to have all approvals finalized at the time of application; however, they should indicate whether the registry will have 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. The feasibility of successfully completing proposed initiatives within the contract timeline is an important scoring domain that will affect the competitiveness of the applications.

Question #29 received April 2, 2025

Reference RFP Section Not Applicable

Question: Can you share what the application pool looked like last year or what DRD levels ended up being funded (proportion of Level 1 to 3)?

Answer: The 2025 to 2026 RFP is distinct from the previous year's funding opportunity, with updated requirements, evaluation criteria, and objectives. Therefore, past funding outcomes may not be indicative of how proposals will be assessed this year. We encourage applicants to ensure their submission aligns with the 2025 to 2026 RFP requirements.

Question #30 received April 2, 2025

Reference RFP Section Not Applicable

Question: To better understand the landscape of rare diseases in Canada, is there a listing of existing rare disease registries on the CDA-AMC website? If so, could you provide the link and is there a process to include our registry?

Answer: CDA-AMC maintains a living Rare Disease Registries Inventory, which provides information about existing registries across Canada and internationally. The inventory is available at https://www.cda-amc.ca/rare-disease-registries-inventory.

If your registry is not currently listed, you will find information on the website about how to submit your registry for inclusion in the inventory.

Question #31 received April 2, 2025

Reference RFP Section 1.2. Alignment With National Strategy for Drugs for Rare Diseases and 2.2. Focus On Fit-for-Purpose Enhancements

Question: In registries where currently no drugs are available, is there available funding for basic science work (e.g., in vivo, in vitro, animal models, or translational studies) to get closer to a drug target?

Answer: The focus of this funding is driven by the National Strategy for Drugs for Rare Diseases and is to bolster the capability of registries to assess drug products and their associated evidence uncertainties. The scope includes drugs with regulatory approval or anticipated to have regulatory approval.

Applications without any drugs in the pipeline (phase III or later) will likely not be competitive. The funding is not designed for conducting basic science research or to inform the natural history of rare diseases.

Question #32 received April 2, 2025

Reference RFP Section 1.2. Alignment With National Strategy for Drugs for Rare Diseases and 2.2. Focus On Fit-for-Purpose Enhancements

Question: What if I need a better diagnostic test rather than a drug for a disease that does not meet rare disease criteria?

Answer: The focus of this funding is driven by the National Strategy for Drugs for Rare Diseases and is to bolster the capability of registries to assess drug products and their associated evidence uncertainties. This includes drugs with regulatory approval or anticipated to have regulatory approval.

This specific funding initiative is not intended to support initiatives related to diagnostics, screening, or diseases that do not meet the rare disease criteria. Applicants should ensure that their proposal clearly aligns with the scope and objectives outlined in the RFP

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