Bulletin #05
Request for Proposal Clarifications

File #C-2401280 Rare Disease Registry Funding

Overview of Clarifications to Proposal Submissions

This bulletin is an amendment to the existing Request for Proposal (RFP) and outlines important changes to the proposal guidelines. It also introduces the 2 funding levels that will be available to applicants.

New Key Dates

Reference RFP Section 4 (Application and Funding Process)

Deadline to submit the full proposal: May 24, 2024, at 11:59 p.m. ET

Notification of the outcome of the proposal: By June 14, 2024, at 11:59 p.m. ET

Work commencement: June 21, 2024

Proposal Submission Guidelines

Reference RFP Section 3.2 (Proposal Preparation Instructions)

- Applicants are limited to a **single comprehensive proposal** per registry.
- **Proposal length**: Maximum of 8 pages, reduced from 12 pages with section limits. All other format requirements remain the same.
- **Section limits (refer to RFP document for full descriptions)**:
    - Registry information, overview of registry (including other funding sources and ongoing projects with those sources), team information, and disease(s) of focus: **maximum of 2 pages**
    - Proposed objectives and work plan: **maximum of 2 pages**
    - Alignment with RFP objectives (describing existing limitations of the registry for real-world evidence [RWE], health technology assessment [HTA], and health care decision-making; in particular, emphasizing specific examples of existing or emerging therapies for rare diseases of interest and how therapies could be better assessed with proposed registry enhancements): **maximum of 2 pages**
    - Risk and mitigation strategies (describing the team's capacity, capability, and experience — and, if applicable, institutional and operational supports — to ensure deliverables will be completed within the time frame): **maximum of 1 page**
    - Deliverables and information about performance measures: **maximum of 1 page**

- For proposals that exceed these limits, only the first 8 pages (including appendices, CVs, or other supporting documents) will be assessed, and the remaining pages will not be reviewed.

Funding Transparency and Eligibility

Applicants are required to detail other funding sources and articulate how the requested funds will be used to support the proposed objectives. We are committed to supporting a diverse range of registries while minimizing redundancy with research grants. This is especially important for ongoing projects that are being funded by other sources.

Funding will only be administered to Canadian organizations or international organizations with a Canadian site or account.

Proposal Evaluation

Refer to RFP Section 5 (Proposal Evaluation and Selection) for the 3 key evaluation criteria by which all proposals will be assessed. In particular, the following will be considered when evaluating the Proposed Objectives and Workplan and the Alignment of Proposal to RFP Objectives sections:

- **Data quality domains**: Does the proposal specify which domains of the data quality enhancement framework are being covered?
- **Registry gaps and enhancements**: Does the proposal identify and describe existing registry gaps or limitations and how they will be addressed? Does the proposal demonstrate how the enhancements align with historical regulatory and HTA data requirements in the same therapeutic area, ensuring updates contribute directly to meaningful use of the data? Is the registry equipped to make those proposed enhancements to registry data — including quality, linkages, and policies — within the project time period?
- **Therapies**: Has the proposal clearly specified how existing and/or emerging therapies could be evaluated by regulators and HTA bodies using data from the registry through the proposed enhancements?
- **Impact:** What are the unique impact and strategic value-adds of the registry enhancements to support health care decision-making?
- **Collaboration and awareness**: If applicable, do the applicants demonstrate awareness of other existing initiatives, registries, patient groups, or databases within the targeted disease area? Does the proposal describe the unique contributions of the registry and approaches to improve synergy and knowledge exchange within the pan-Canadian data landscape? Is the proposal a collaboration among registries in the same disease area?
- **Scalability**: Does the proposal describe and demonstrate the capability of enhancements to lead to sustainable long-term improvements, particularly with reference to the evolving landscape of innovative rare disease therapies and the RWE landscape?

RFP Funding Categories

We have established standardized funding categories to clarify the expectations and scope of proposal submissions. Although the evaluation criteria and process will be the same for both levels, proposals seeking level 2 funding are expected to include a detailed explanation of how they will deliver on the proposed work plan at scale by March 31, 2025.

Level 1 Funding (< $75,000)
Level 1 funding initiatives are designed to strengthen the foundation of registries and data collection efforts without requiring substantial financial investment.

Examples:

- Enhanced data collection procedures and policies: Implementing standardized data collection forms and procedures to improve the completeness and accuracy of registry data; improving registry policies and governance to become more equipped for HTA analyses.

- Collaboration: Strengthening patient engagement through education materials and outreach programs; consulting with health care stakeholders to ensure the registry is collecting valid, meaningful, and informative data elements that could support regulatory and HTA requirements.
- Pilot studies for data validation: Conducting pilot studies to validate registry data and assess the reliability and consistency of collected information.
- Training and capacity building: Developing or enhancing training for registry staff, data providers, and new users to improve expertise in data management, analysis, and reporting relevant to HTA.
- Registry website development: Creating or improving a registry's online presence to provide information, recruit participants, and disseminate findings.
- Technology upgrades: Investing in software to facilitate data entry, such as mobile apps for patient self-reporting or software for electronic health record integration.
- Revising and updating standard operating procedures, governance, and data-sharing policies.

Level 2 Funding ($75,000 to $200,000)

Level 2 funding initiatives involve more comprehensive projects that aim to broaden the scope and impact of patient registries. Due to their scale and complexity, these projects require a higher financial investment. Applicants at both funding levels should demonstrate a commitment to collaborating with other registries and relevant partners and provide a sustainability strategy for post funding operations. This strategy must ensure the long-term viability and impact of the registry enhancements, with a clear plan for maintaining these upgrades beyond the funding period of the current RFP. Proposals should explicitly address the project's feasibility within the funding period and detail how risks impacting delivery will be mitigated, ensuring that all enhancements can be sustained independently.

Examples:

- Expansion of registry coverage: Expanding registry coverage to include rare diseases (and the relevant patient populations) and additional sites; for each rare disease captured, identifying linkages to existing treatments or treatments expected to launch in Canada within the next 2 to 5 years for each rare disease captured.
- Data quality assurance plan implementation: Establishing a comprehensive data quality assurance plan to identify and address errors, inconsistencies, and missing data within the registry.
- Longitudinal data collection expansion: Expanding longitudinal data collection to capture long-term patient outcomes and treatment effectiveness over time.
- International data harmonization efforts: Participating in international data harmonization initiatives to align registry data standards and terminologies with global best practices, specifically with published registry standards.
- Advanced data analytics: Implementing advanced data analytics techniques to derive actionable insights from registry data.
- Enhancing interoperability to facilitate sharing and/or linkages to data from other sources (e.g., clinical trials, electronic health records, administrative databases) to generate comprehensive evidence for premarket and postmarket decision-making.

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