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Assessing Canadian Rare Disease Patient Registries for Real-World Evidence Using REQueST

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Introduction

Real-world evidence (RWE) can provide invaluable insights that aid patients, clinicians, researchers, federal health agencies, and others when making decisions about care for disease. RWE is especially relevant for rare diseases (RD) for which there may be efficacy and safety uncertainty from clinical trials because of small patient populations, disease complexity, and dissimilar standards of care across Canadian and international jurisdictions. Optimal use of RWE to inform decision-making requires access to fit-for-purpose data from registries as well as administrative data.

To date, no inventoried resource exists for Canadian RD registries, making it difficult to identify potential sources of real-world data (RWD). Our aim is to establish a localized resource of RWD for health technology assessments (HTA), regulatory purposes, and research by compiling and describing pan-Canadian RD registries.

Objectives

- To identify pan-Canadian RD registries
- To describe these registries using the Registry Evaluation and Quality Standards Tool (REQueST)
- To conduct a preliminary analysis on the REQueST results to guide discussions with stewards of new and existing Canadian RD registries about collaborative initiatives to generate RWE to support decision-making.

Methods

RD registries were identified using specific Google search terms and an existing list of registries provided by CADTH. Registries were included if they were currently active, pan-Canadian in scope, and captured pan-Canadian data concerning patients with an RD.

Registries were described using publicly available information and REQueST (www.eunethta.eu), a 23-item tool developed to facilitate consistent registry evaluation by both registry custodians and regulatory and HTA decision-making agencies.

Assessment of 3 areas via consecutive steps:

- methodological information (8 elements)
- essential standards (12 elements)
- additional requirements (3 elements).

REQueST items were categorized for each registry as satisfactory, unclear or incomplete, or unknown based on publicly available information. Aggregate scores were calculated; no individual registry was identified.

Results

In June 2022, the mean REQueST assessment score of RD registries based exclusively on publicly available information was 13.8 (range, 8 to 19) out of a maximum of 23.

Methodological Information Area

The most complete information about Canadian RD registries was identified for the area of REQueST concerning methodological information.

All 25 RD registries publicly provided clear information on element:

1. Type of registry and aims.

Little information was disclosed publicly about methods to deal with confounders — these practices were deemed unknown in 24 of 25 registries.

Essential Standards Area

The greatest variability across Canadian RD registries was observed in the Essential Standards area of REQueST concerning universal and essential elements of good practice and evidence quality for registries:

In publicly available resources, most information gaps were related to data quality processes, such as:

17. Data cleaning: practices unknown in 23 of 25 registries
18. Missing data: practices unknown in 23 of 25 registries

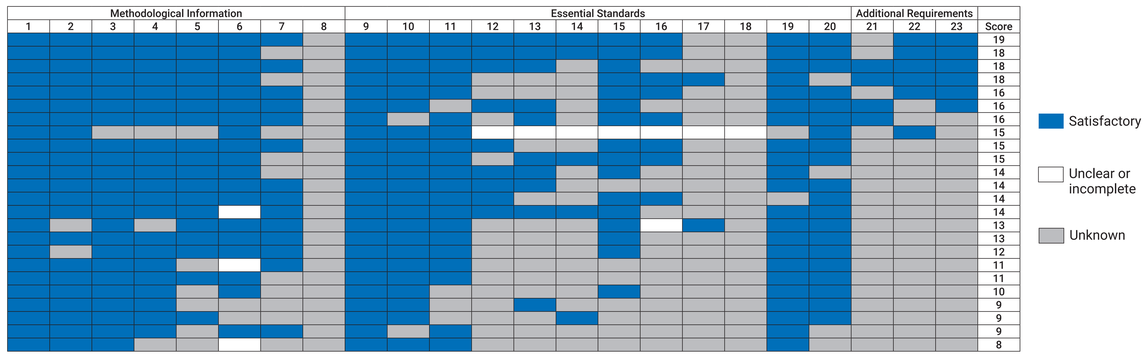
Additional Information Area

The assessment criteria provided by REQueST within this area are designed to be interpreted in relation to a specific evaluation or research question; therefore, making an assessment based solely on publicly available information is challenging.

There were limited publicly available data concerning the 3 elements included in the Additional Information area:

21. Interoperability and readiness for data linkage
22. Identification of data sources
23. Consideration of ethical requirements

Figure 1: Assessment of Canadian Rare Disease Registries Based on 23 REQueST Items



Item: 1 = type of registry and aims; 2 = use of registry and previous publications; 3 = geographical and organizational setting; 4 = duration of data collection; 5 = size and number of patients; 6 = inclusion and exclusion criteria; 7 = follow up methodology; 8 = methods to measure and control confounding; 9 = objectives and methodology; 10 = governance; 11 = informed consent; 12 = data dictionary; 13 = defined minimum data set; 14 = standard terminology, terminology, and specifications; 15 = description of data collection; 16 = quality assurance plan; 17 = data cleaning plan; 18 = missing data plan; 19 = financing; 20 = protection, security and safeguards; 21 = interoperability and readiness for data linkage; 22 = identification of data sources; 23 = consideration of ethics requirements.

Limitations

The application of REQueST was completed on publicly available information without the participation of RD registry holders or custodians. We acknowledge that REQueST is designed as a framework for registry holders or custodians to demonstrate the quality of data collection and for regulators and HTA agencies to assess quality in an objective and transparent way.

Discussion

This preliminary work sets the stage for an ongoing and more in-depth assessment of RD registries in Canada. It provides information to guide multistakeholder dialogue on the suitability of REQueST for appraisal of Canadian RD registries for the purpose of informing regulatory, HTA, reimbursement, and clinical decision-making.

Ideally, next steps will include collaborative initiatives with registry holders or custodians that include self-assessments using REQueST.

Another potential area for further exploration is identifying examples of how Canadian RD registry data have contributed information to Canadian and international regulators and/or HTA agencies.

Conclusions

The results of this preliminary assessment suggest that Canadian RD registries are collecting patient-level health care data that can potentially support excellence in clinical care, continued research, and inform decision-making. This also highlights the importance of establishing clear standards at the pan-Canadian and international level for RD registries.

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