Existing practices for the identification, selection or assessment of registries and real-word data for regulatory/HTA purposes: a More-EUROPA project systematic search

MSR30

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Introduction

- Real-world data (RWD), particularly patient registries, represent a potentially useful source of information.
- RWD may be required at various stages of the drug lifecycle for the regulatory and/or HTA decision-making process.
- The selection of an appropriate registry is critical in RWD-based research.
- Registry features impact the quality of statistical analyses and the resulting evidence.
- **Objective**: Explore the different standards 'fit-for-purpose" to identify, select, and assess registry data for regulatory and HTA purposes.
- **Project:** The More-EUROPA project, involving 15 public and private organizations from 7 EU countries, evaluates registry data's effective and ethical use to support patient-centered decisions by drug regulators and Health Technology Assessment agencies in Europe.

Methods

- Conduct a **systematic literature search** using MeSH terms (Figure 1).
- **Retrieve articles published between 2013-2023** in MEDLINE, Web of Science and Scopus.
- Triangulate the results with the stakeholders' survey and interviews (poster MSR91), and the second literature review (poster MSR61).

Figure 1. Selected search terms during the systematic search

("registr"[Title/Abstract] OR "observational"[Title/Abstract] OR "database"[Title/Abstract] OR "dataset"[Title/Abstract] OR "fit-for-purpose"[Title/Abstract] OR "fit-for-use"[Title/Abstract]) AND ("real-world"[Title/Abstract] OR "real world"[Title/Abstract] OR "RWE" [Title/Abstract] OR "RWD" [Title/Abstract]) AND ("regulat"[Title/Abstract]) AND ("2013"[Date - Publication] : "2023"[Date - Publication])

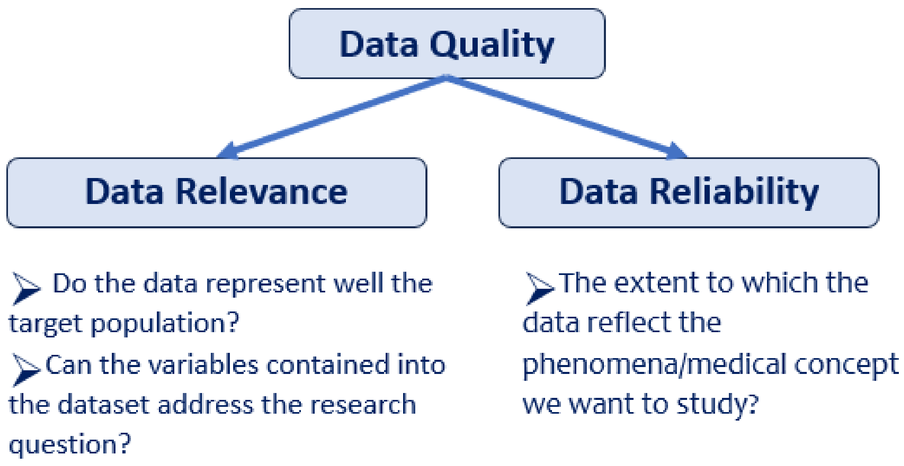
Results

- **45 articles** selected after abstract and full text screening.
- **10 frameworks** compliant with the **FAIR principles**: Findability, Accessibility, Interoperability & Reusability.
- Frameworks assessing if **a dataset is fit-for-purpose:**
  - MVET principles: Meaningful, Valid, Expedited and Transparent evidence
  - PICOTS: Population, Intervention, Comparator, Outcome(s), Timing, and Setting
  - SPACE: Structured Pre-Approval and Post- Approval Comparative Study Design
  - SPIFD/SPIFD2: Structured Process to Identify Fit-for-purpose Data
  - RWD-Cockpit
  - Maturity
  - The Duke Margolis Center for Health Policy framework
  - The Health Data Research United Kingdom 'data utility' framework

Table 1. Data quality criteria among identified frameworks

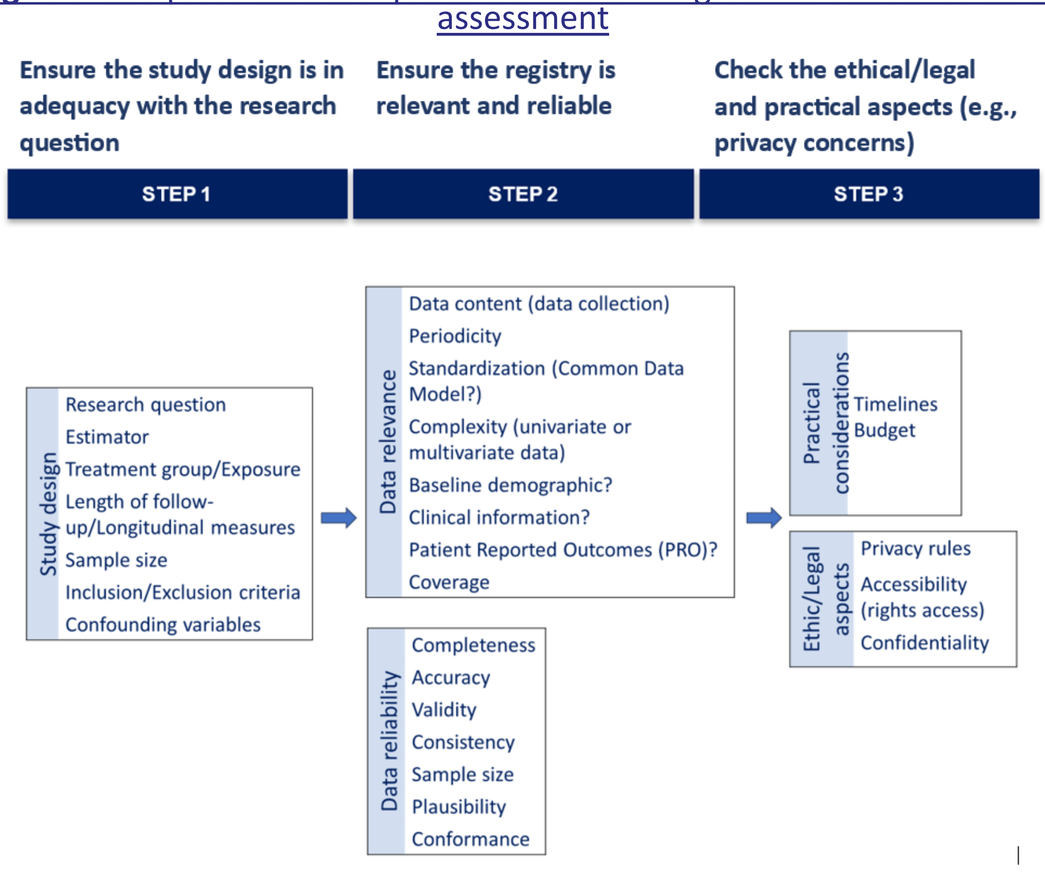
Data quality criteria	Data checks
Consistency	Uniformity of data over time
Accuracy	Data curation ensures that there are no errors, contradictions, or duplicates
Completeness	Are data values present? The registry should contain all the core variables needed and a low proportion of missing data (data integrity)
Plausibility	Do the observed values belong to a plausible range of values? Do variables have a context-dependent relationship?
Conformance	Do data meet the standard format requirements?
Validity	Are the measures of outcomes valid?
Transparency	Where does the data come from? How were they transformed?

Figure 2. Research questions on data relevance and reliability



- **Common core set of criteria** for all frameworks:
  - Research question
  - Population
  - Exposure
  - Outcome(s)
  - Sample size
  - Follow-up length
- Different ways to **assess the adequacy of the fitness-for-purpose of the data:**
  - Qualitative assessment: PICOTS
  - Colour-coded assessment: REQueST, HDR data utility
  - Scoring system: SPIFD/SPIFD2, RWD-cockpit

Figure 3. Proposed main steps and criteria for registries identification and assessment



Conclusion

- The existing tools primarily concentrate on assessing data sources after their identification and when research questions are already established or in progress.
- There is no tool that enables stakeholders to seamlessly combine both the identification and assessment processes on a fit-for-purpose.
- Next steps are to develop a screening tool to timely identify suitable registries on a 'fit-for-purpose' approach.

References include:

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