HSD81

Identifying and appraising fit-for-purpose data sources for real-world evidence studies: a structured approach to conducting data source feasibility assessments

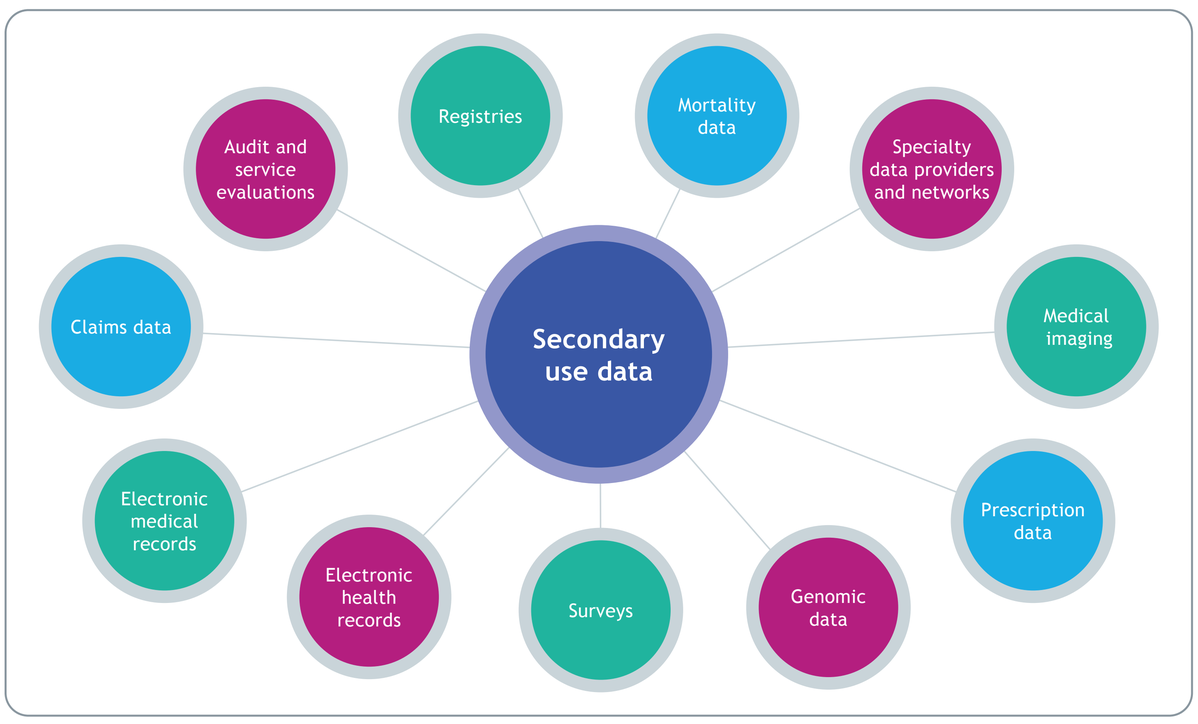
Hannah Matthews, Myriam Alexander, Fiona Glen

OPEN Health HEOR & Market Access, London, UK

INTRODUCTION

- Researchers, healthcare providers, payers, and regulators are becoming increasingly receptive to evidence generated from real-world data (RWD) sources alongside randomised controlled trials to support the understanding of new therapies and their clinical and economic impact.
- Due to the growing use of RWD, there is an appropriate scrutiny around the applicability and quality of this data, which may be used to support decision-making.
- Typically, real-world evidence (RWE) studies are derived from primary or secondary RWD sources. Primary data collection studies collect RWD specifically for RWE studies, whereas secondary studies use data primarily collected for another purpose.[1]
- Data generated from RWE studies must be methodologically robust. Therefore, this research focuses on providing a framework to assess the robustness, suitability, and quality of secondary RWD sources.

Figure 1. Types of secondary use data sources



OBJECTIVES

- To develop a simple methodological framework for identifying and appraising RWE data sources to achieve meaningful and reliable results.
- It is hoped that a simple and clear structured approach will allow stakeholders to confidently identify and appraise RWE data sources to support informed decision-making.

METHODS

- We synthesised our expert insights and experience from implementing secondary use RWE studies globally from a focus group-based discussion to generate key components of the framework.
- Alongside this, we also undertook a targeted literature review of peer-reviewed scientific literature and guidance documents and tools which explored data suitability.
- For the targeted literature review, we searched Embase and MEDLINE (ProQuest) for recently published literature identifying or appraising secondary use data. We also carried out extensive desk research into grey literature sources to identify RWE guidance and tools.

RESULTS

- We developed a framework using our expert insights within the secondary use data space and the targeted literature review of scientific literature.
- The targeted literature review found a dearth of published peer-reviewed evidence around identifying and appraising secondary data sources. However, key guidance documents were retrieved, including *The Good Practice Guide for the Use of the Metadata Catalogue for RWD Sources*[2] and *The NICE RWE Framework*.[3]
- Our research clearly highlighted the need to implement a **feasibility assessment** prior to implementing an RWE study to ensure the most appropriate data source for the research question.

FRAMEWORK

The following steps were identified as key to implementing a robust data source feasibility assessment:

1. **Establishing research scope:** Clearly identifying the research question, objectives, disease indication, and eligibility criteria, such as countries of interest.
2. **Data source identification:** Conducting a targeted literature review of peer-reviewed scientific literature, databases registries, and networks in line with a pre-defined search strategy, to identify any existing RWE data sources.
3. **Data source screening:** Conducting a comparison against pre-specified eligibility criteria to generate a database listing of the most appropriate data sources for further examination.
4. **Data extraction:** Collating key information and outcomes about each short-listed data source according to a standardised grid.
5. **Assessment and appraisal:** Consolidating and grading information to select the potentially most appropriate data sources for addressing the research question and objectives.

Figure 2. Example data extraction parameters

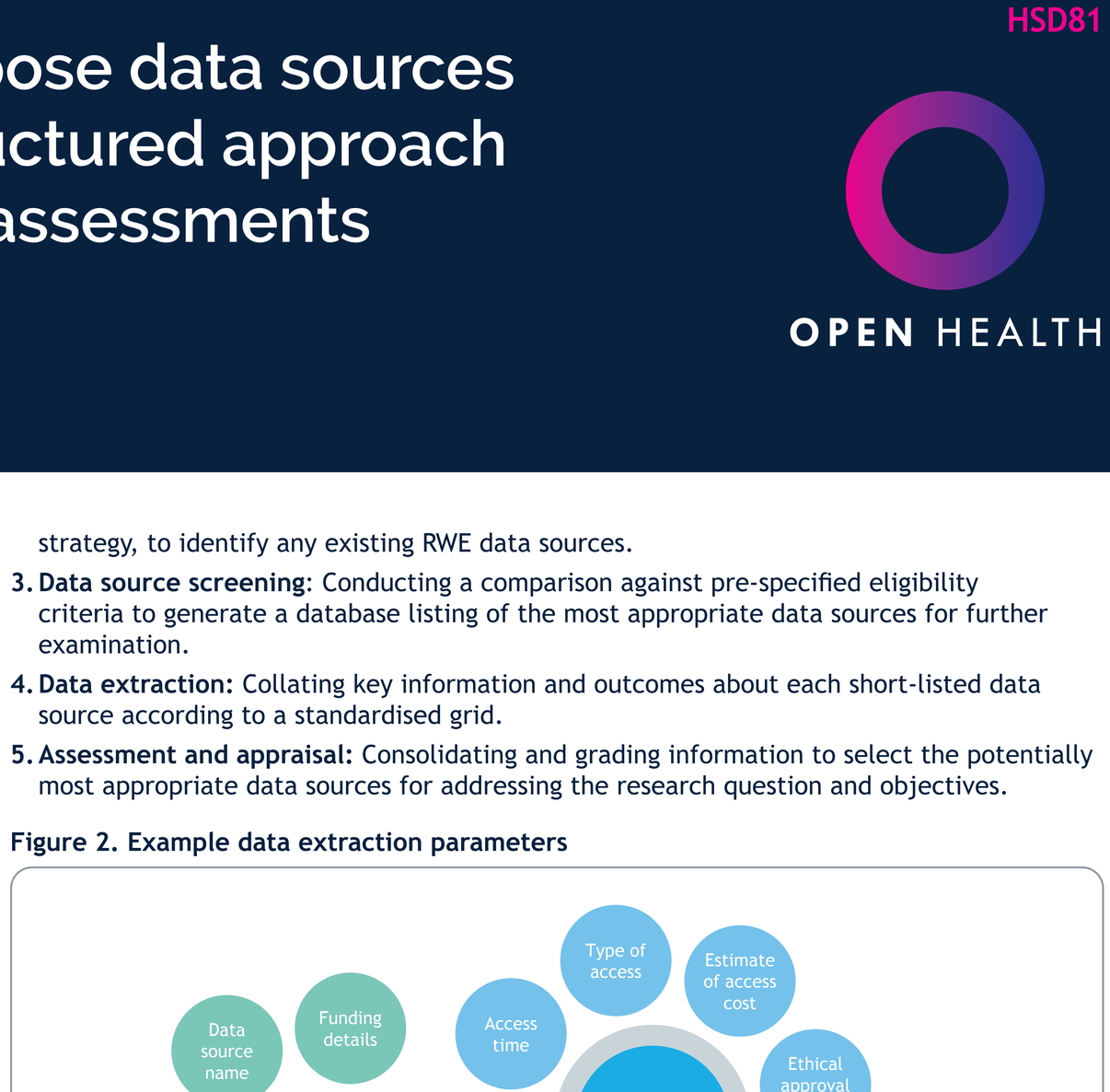
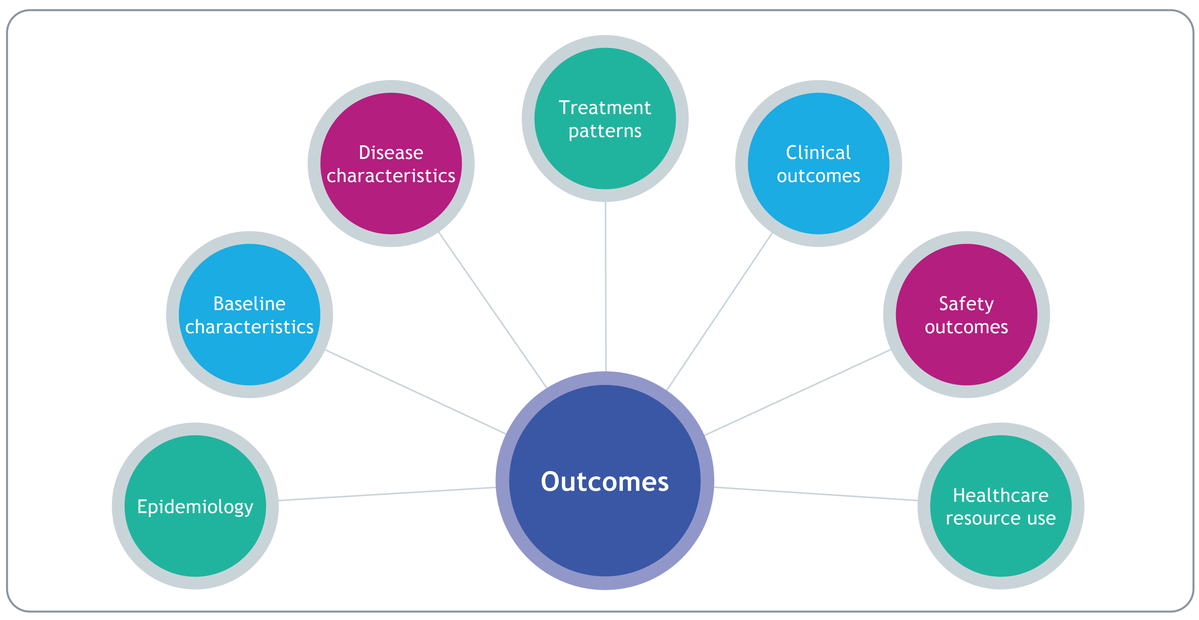


Figure 3. Example data extraction outcomes



CONCLUSIONS

- This simple framework can be applied and adapted across multiple indications to facilitate the identification of the most appropriate and relevant data sources for the generation of robust RWE studies and their appraisal, potentially allowing for RWE to inform healthcare decision-making.
- Nevertheless, further peer-reviewed literature and standardised guidance will be required if RWE is to support decision-making for regulatory and reimbursement submissions.
- Moreover, RWD sources and the environment in which they operate are dynamic and will require continual updates to ensure relevance and suitability. Therefore, we would suggest that feasibility assessments and our simple framework is either applied on a project-by-project basis or updated annually to ensure the relevance and continued robustness of RWE generation.

REFERENCES

1. de Lusignan, S., Crawford, L., & Munro, N. (2015). Creating and using real-world evidence to answer questions about clinical effectiveness. *J Innov Health Inform*, 22(3), 368–373. https://doi.org/10.14236/jhi.v22i3.177.
2. European Medicines Agency. (2022). *The Good Practice Guide for the Use of the Metadata Catalogue for RWD Sources*. www.ema.europa.eu/en/documents/regulatory-procedural-guideline/good-practice-guide-use-metadata-catalogue-real-world-data-sources_en.pdf.
3. National Institute for Health and Care Excellence. (2022). The NICE RWE Framework [Corporate document ECD9]. www.nice.org.uk/corporate/ecd9/chapter/overview.

Presented at ISPOR Europe 2023