The Maze of RWE Frameworks: An Environmental Scanning and Comparison Across Regulatory and HTA Bodies

RWD106

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Background

- The use of real-world evidence (RWE) can provide valuable insights and information on the impact and outcomes of healthcare technologies in the real world (e.g., effectiveness, safety, drug usage/adherence).
- Much has been documented about the potential uses of RWE in healthcare decision-making, as well as the related challenges (Figure 1).
- Differences in guidance for the use of RWE across different phases of the drug development lifecycle, however, creates complexity in preparing for regulatory and reimbursement submissions. The available regulatory and reimbursement guidance is presented in Figure 2.
- As the number of RWE frameworks and guidance continues to rapidly grow, the maze of information becomes more difficult to navigate.

Figure 1. Overview of the use of RWE for healthcare decision-making

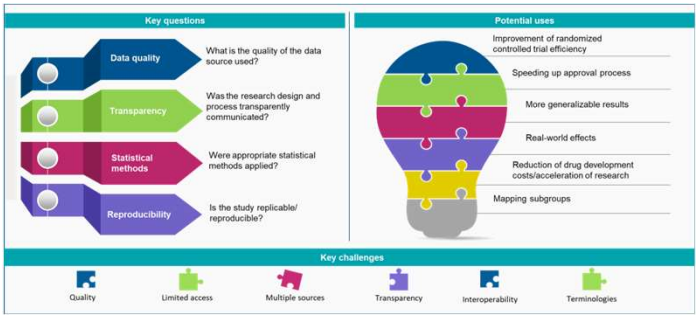
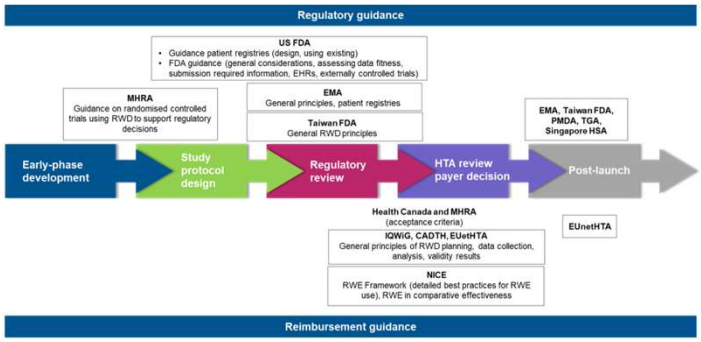


Figure 2. General RWE guidance across the phases of the drug development lifecycle



Abbreviations: CADTH, Canadian Agency for Drugs and Technologies in Health; EHR, electronic health record; EMA, European Medicines Agency; EUnetHTA, European Network for Health Technology Assessment; FDA, Food and Drug Administration; HAS, Health Sciences Authority; HTA, health technology assessment; IQWiG, Institute for Quality and Efficiency in Health Care; MHRA, Medicines and Healthcare products Regulatory Agency; NICE, National Institute for Health and Care Excellence; PDMA, Pharmaceuticals and Medical Devices Agency; RWD, real-world data; TGA, Therapeutic Goods Administration; US, United States

Objectives

- This study aimed to conduct an environmental scan (review) of RWE frameworks and guidance documents across regulatory and health technology assessment (HTA) agencies.

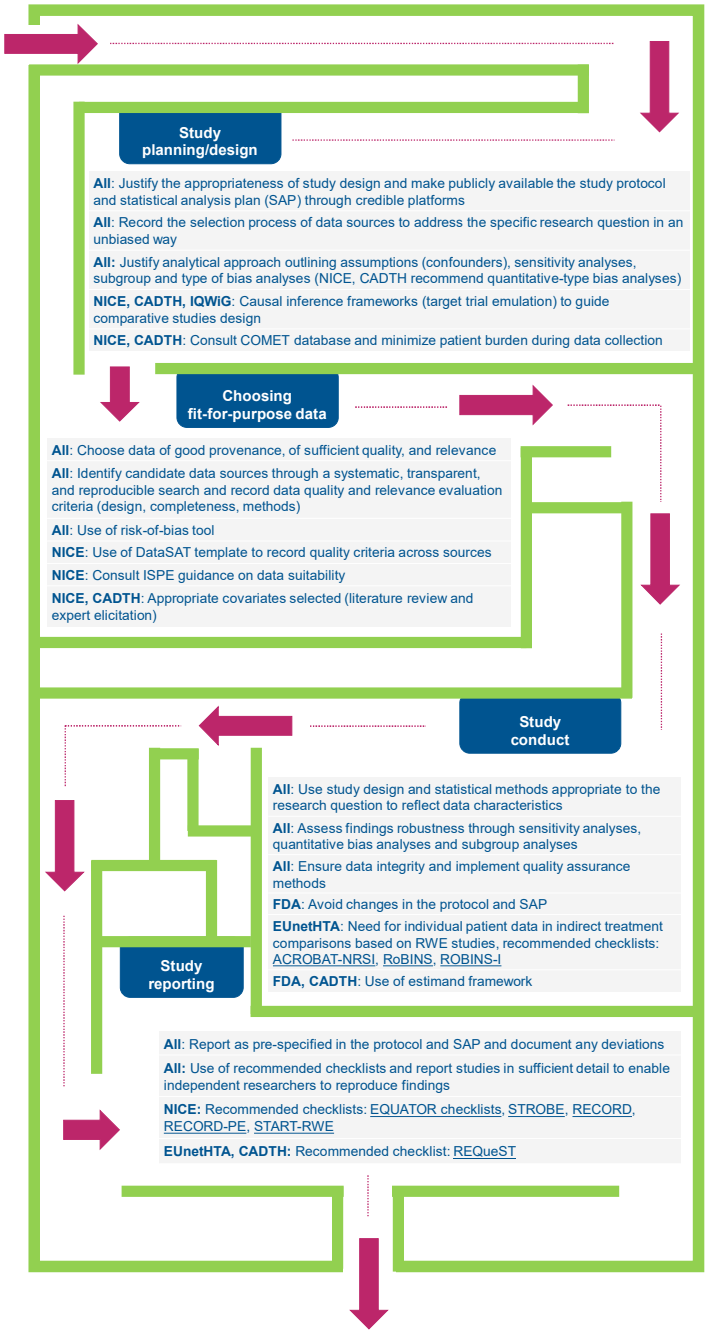
Methods

- The websites of the United States (US) Food and Drug Administration (FDA), European Medicines Agency (EMA), United Kingdom's Medicines and Healthcare products Regulatory Agency (MHRA), Taiwan FDA, Canadian Agency for Drugs and Technologies in Health (CADTH), Institute for Clinical and Economic Review, Institute for Quality and Efficiency in Health Care (IQWiG), and European Network for HTA (EUnetHTA) were systematically searched in June 2023 for RWE guidance, including white papers.
- Using a pre-designed form, data on scope and recommendations were extracted from each document.
- Guidance on artificial intelligence or digital technologies were excluded.
- Results were synthesized by four topics (study planning, choosing fit-for-purpose data, study conduct, and reporting) to identify similarities and differences.

Results

- Forty-two documents were identified, of which four were from HTA bodies or assessment groups supporting these decisions (NICE, CADTH, EUnetHTA, IQWiG).
- Except for two, all documents were published after 2018. FDA and EMA documents covered all four topics. NICE and CADTH frameworks provided a centralized document with all RWE submission requirements.
- EMA, NICE, and IQWiG guidance included specific analytical suggestions.
- The NICE framework included the most extensive list of RWE quality tools by study design and discussed specific methodological topics by RWE use.
- Similarities and differences of RWE requirements are shown in Figure 3.

Figure 3. Overview of RWE guidance across EMA, FDA, MHRA, NICE, CADTH, IQWiG, EUnetHTA



Conclusions

- Keeping updated with the rapid publication of RWE frameworks and guidance across organizations remains a challenge for all stakeholders.
- Closer collaboration among organizations to standardize RWE minimum requirements for regulatory and reimbursement submissions can contribute to higher quality in submissions using RWE, and timely medicines assessment and access for patients.
- There is an urgent need for regular updates of frameworks and their guidance, recommendations and requirements in a living mode.

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