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A TARGETED LITERATURE REVIEW COMPARING GLOBAL GUIDELINES FOR THE USE OF REAL-WORLD EVIDENCE IN HEALTH TECHNOLOGY ASSESSMENT AND REGULATORY DECISION-MAKING

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INTRODUCTION

- Digitised health records, AI, and scalable computing power position Real-World Evidence (RWE) as a transformative force in research and market access.
- RWE offers a robust, less costly, and inclusive approach to healthcare research and a sustainable life sciences ecosystem.
- RWE already plays a key role in drug development, regulatory approval, and post-authorisation monitoring of medicines.
- By driving research and fostering a sustainable life sciences ecosystem, real-world evidence and ongoing monitoring of medicines.

OBJECTIVE

To systematically identify, compare, and synthesise key recommendations from major global guidelines on the generation and use of RWE in regulatory and HTA decision-making.

METHODOLOGY

1. Search — Targeted literature review of PubMed and Google Scholar for RWE guidance documents; manual review of HTA/regulatory agency websites.

2. Terms — "Real-world evidence," "real-world data," "RWE," "RWD," and "guidelines."

3. Eligibility — Formal RWE guidance issued by regulatory bodies or HTA agencies.

4. Synthesis — Data qualitatively synthesised to compare recommendations across data sources, study design, methodological rigour, and decision-making applications.

Figure 1. Methodology process

- Comprehensive searches were conducted in publication platforms like PubMed and Google Scholar to identify studies comparing existing RWE guidelines.
- The key search terms used included "Guideline*", "Real world evidence", "RWE", "Real world data", and "RWD", etc. (Figure 1)
- No specific inclusion and exclusion criteria were applied, and no restrictions in timeframe, language, or geographical scope were imposed.
- The searches were not expanded to include academic, non-academic articles, and conference abstracts.

RESULTS

- 24 hits identified; 5 RWE guidelines included: REALISE group, NICE, HAS, NMPA, and CADTH.
- RWD refers to administrative data from routine care usable for research purposes.
- Guidelines recommend RWE for characterising health conditions, estimating economic burden, and supporting drug regulatory decisions.
- RWE can be generated from randomised trials, large simple trials, pragmatic trials, and observational studies.

Table 1. Recommendations on When to Use RWD/RWE

REALISE group	NICE
• When RCTs are of poor quality, impossible for ethical reasons, or infeasible due to small numbers (e.g., rare diseases). • To contextualise/localize economic models, extrapolate RCT data beyond trials, and support price negotiations.	• Characterizing health conditions, interventions, care pathways, patient outcomes/experiences, including natural history. • Estimating, designing, populating, and validating economic models; measuring patient experience.
HAS	**NMPA**
• Describing intervention use conditions; estimating utility scores, long-term effects, and risks of health products. • Measuring resource consumption, efficacy, risks, and organizational impact of disease/drug.	• RWE in support of Drug Development and Review, with specific requirements. • Guidance on RWD definition, source, evaluation, governance, standards, safety, quality assurance, applicability.

Figure 2. Recommendations on What RWE to Collect

REALISE group	Patient variables on medical history/condition and practice variation for comparability across settings; person-level RWE to assess whether trial data translate to meaningful patient improvements; PRO collection where possible.
NICE	Demographics, dose, route/duration, health behaviors, medical history, diagnosis, admission/discharge, clinical & patient-reported outcomes, resource use, costs, labs, imaging, free text, patient-generated data. National/international data considered.
HAS	Structured and unstructured digital/non-digital patient records: demographics, clinical characteristics, diagnosis, treatment, labs, safety, and clinical outcomes.
NMPA	Demographic, dose, route, duration, health behaviors, medical history, diagnosis, admission, discharge, clinical outcomes (incl. PROs), patient experience, resource use, costs, economics, labs, imaging, free text, test results, patient-generated data; national/international data considered.

- Regulatory agencies are advancing RWE use in submissions, raising the importance of high-quality RWD collection.
- Pharma applies RWE for trial design/recruitment, payer/reimbursement analysis, and post-market safety monitoring.
- Key quality parameters: good/known data provenance, relevance, sufficient quality, and transparent, consistent evidence generation from planning to reporting.

CONCLUSION

- Leveraging RWE can significantly enhance the efficiency and effectiveness of healthcare research and market access.
- By utilizing digitized health data, AI technology, and scalable computational power, RWE offers a promising solution to the challenges of drug development. Even so, additional research is required to examine how applying the current RWE standards can enhance patient outcomes in everyday practice.

CONFLICT OF INTEREST

- Manupati R, Kumar A, Vishnubhotla M, and Rai MK are employees of Trinity Life Sciences, India.
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