NICE National Institute for Health and Care Excellence

IDERHA

RWD69

Acceptability Standards for Real-World Evidence – A Review of the HTA and Regulatory Policy Landscape

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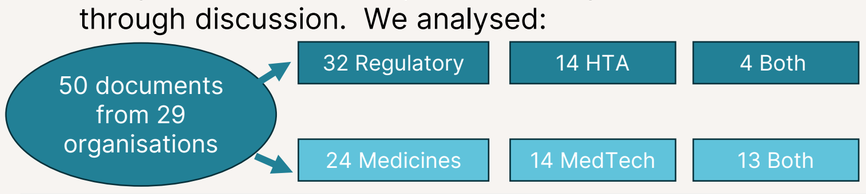
Background

IDERHA is a European Innovative Health Initiative (IHI) project launched in 2023, aiming to enable the safe, secure, and streamlined sharing of heterogeneous healthcare data. The goal of IDERHA's policy-focused activities is to accelerate policy development by building consensus on the use of real-world data (RWD) and real-world evidence (RWE) in regulatory and Health Technology Assessment (HTA) decision-making for medicines and medical technologies. With the increasing number of policy and guidance documents on the use and acceptability of RWE, IDERHA has undertaken a global landscape review to understand areas of consensus, divergence, and the need for further policy development.

What we did

This review employed the JBI scoping review methodology. NICE information specialists conducted literature searches through MEDLINE ALL and Embase, as well as key organisational websites such as EMA, US FDA, and NICE. The review included English-language policies, standards, frameworks, and guidance documents published between 2017 and January 2024 that addressed acceptable RWE data use. Documents specifically addressing RWD/RWE by regulatory and HTA agencies were prioritised.

Documents superseded by updated versions were excluded. Data extraction followed a standardised template, refined after piloting on ten documents. Key extraction criteria included guidance on RWE use, study design, data quality, and transparency. At least 25% of extractions were compared by two independent reviewers, with discrepancies resolved through discussion. We analysed:



What we found

To our knowledge, this is the first review to look across policies for regulatory and HTA, covering both medicines and medical devices and having a global scope (Figure 1).

Though there has been a significant growth in the number of RWE policies, there remains considerable variability in the scope and depth of these documents. The documents reflect both acknowledgement of the benefits of RWE, but also concerns around the potential for poor quality research and data misuse. Continued work is needed to build trust in RWE through ensuring high quality and transparent research.

Encouragingly, we found evidence of emerging best practices for RWE research, which could serve as a foundation for 'good RWE research practices'. This could be taken forward further through actions such as identifying key lessons and tools from pilot programmes of RWE use (Figure 2). Additionally, we identified several policy gaps requiring consensus-building and further development (Figure 3).

Next steps

A public consultation was conducted between June and July 2024 to help prioritise IDERHA's focus areas in developing best practices and addressing policy gaps. The consultation process combined an open online survey, targeted one-to-one sessions with key stakeholders and an internal workshop.

The consultation indicated that there was particular interest in:

- Continuing to further emerging best practice – particularly drawing out lessons learnt, and case studies of RWE use in Regulatory and HTA decision-making
- Exploring the intersection of RWE and artificial intelligence (AI)

IDERHA will address these policy areas through collaborative stakeholder engagement to develop consensus-based policy recommendations. An initial set of policy recommendations is due to be published in November 2025.

Figure 1. Locations of documents included in the review

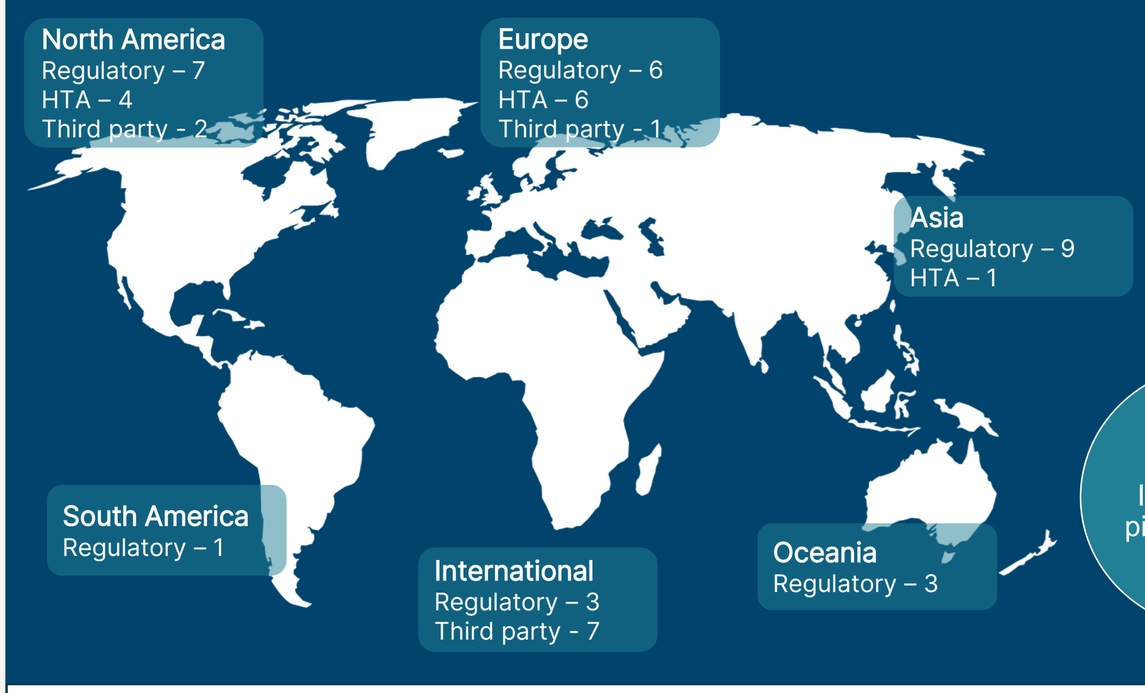


Figure 2. Emerging best practices

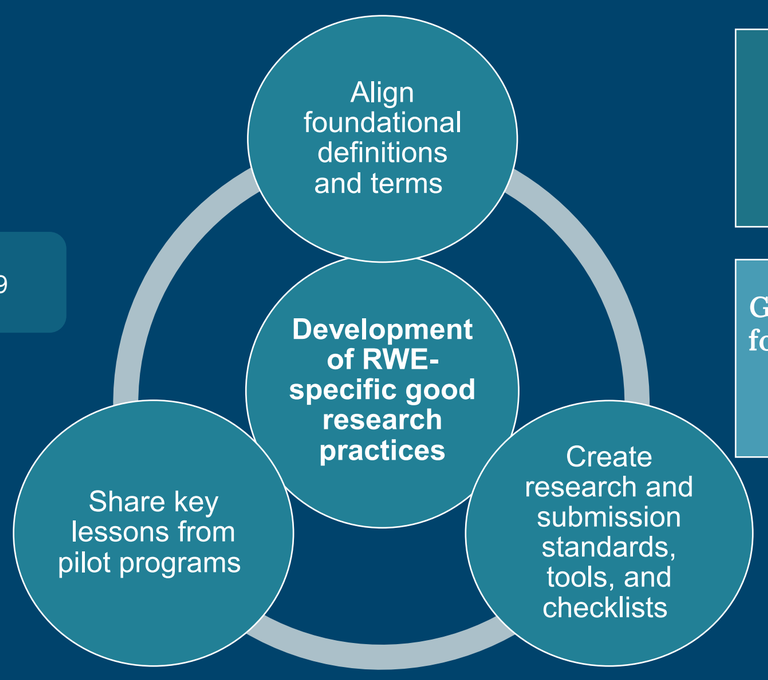
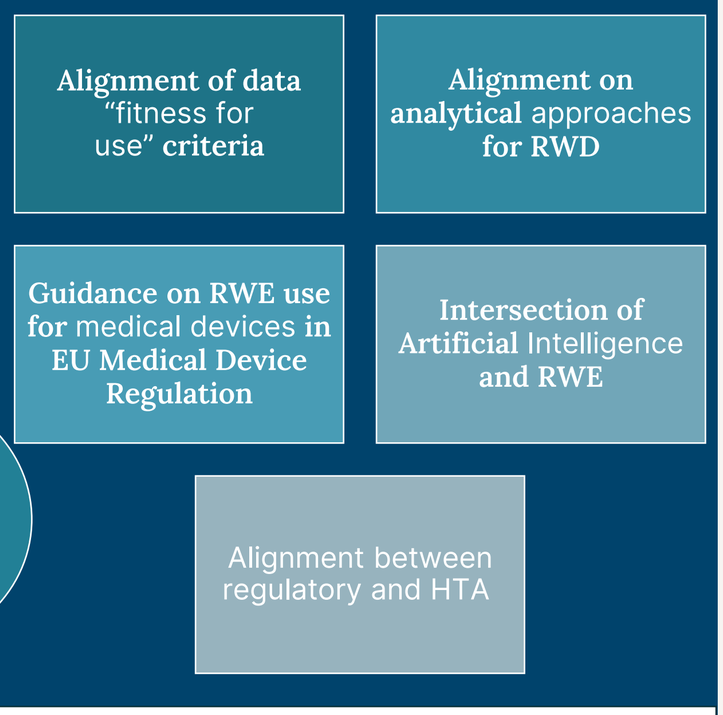


Figure 3. Key policy gaps



Conclusion: The review demonstrated advances in technical and methodological approaches to the use of RWE in HTA and Regulatory settings. However, establishing greater confidence in RWE applications requires more transparent, high-quality research. Key methodological gaps remain, particularly in standardising analytical approaches, determining data suitability criteria, understanding AI integration, and harmonising evidence requirements between regulatory and HTA bodies. Future work within the IDERHA project, will prioritise advancing emerging best practices and begin exploring the policy requirements around the intersection of AI and RWE.

About IDERHA
Find out more about IDERHA and the work we are doing by scanning the QR code.

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