What Does "Good" Look Like in Real World Evidence (RWE) Research?

- Authors: Omnia Bilal, Hanane Omichessan, Katharine Cresswell, Ravinder Claire, Bethany Shinkins, Rita Peeters, Heather Colvin & IDERHA Work Package 6.4 Contributors

FACTS & FIGURES

Start date:	01/04/2023
End date:	31/03/2028
IHI funding:	€23 M
Industry contribution:	€19.6 M
Project website:	www.iderha.org
LinkedIn:	www.linkedin.com/company/iderha
Email:	communications@iderha.org

CHALLENGE

Real world evidence (RWE) has an increasingly important role in regulatory and health technology assessment (HTA) decision-making. But it only supports good decisions when it is generated and assessed in ways that inspire confidence.

Traditional clinical studies have real limitations. They can underrepresent diverse patient populations and in some circumstances, such as rare diseases or ethical recruitment challenges, randomised trials are simply not feasible. RWE can help fill these gaps.

The problem is there are no widely accepted standards for RWE research equivalent to Good Clinical Practice. Almost every policy document reviewed acknowledged RWE's potential but also raised concerns about study design, data quality, and when RWE can reliably inform decisions within the research lifecycle. Without shared expectations, decision-makers cannot consistently assess the credibility of the evidence they receive.

APPROACH & METHODOLOGY

Three sequential phases were used: identifying policy gaps through a global landscape review; developing recommendations through multi-stakeholder, public consultation and targeted expert engagement on the RWE research lifecycle; and disseminating findings to drive adoption across regulatory and HTA environments globally (see Figure 1 below).

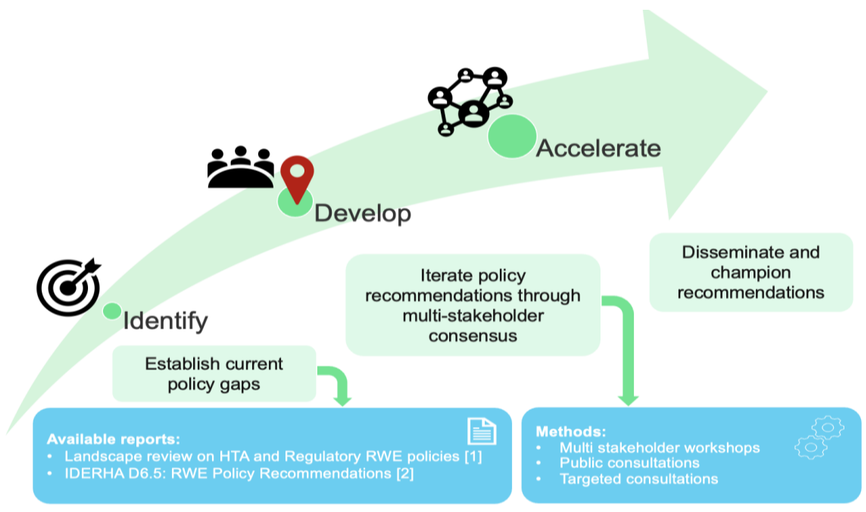


Figure 1: IDERHA stepwise approach for recommendation development

[1] Landscape review (D6.2):
https://www.iderha.org/sites/iderha/files/2024-05/D6.2%20Report%20on%20Global%20Regulatory%20Best%20Practices%20Analysis_v2.0.pdf
[2] D6.5 RWE policy recommendations:
https://zenodo.org/records/18455384

CAREER IMPACT

IDERHA gave me the chance to work across internation health data policy, regulatory science, and HTA in a single project. That combination is rare.

Working with 33 partners across academia, industry, patient organisations, and regulatory bodies pushed me to develop skills I would not have developed in a more conventional role: multi-stakeholder facilitation, European policy analysis, and translating complex technical evidence into recommendations that people from very different backgrounds can act on.

It also expanded my network significantly and connected strands of my work across RWE methodology, health data governance, and HTA in ways that continue to open new directions for me.

RESULTS

Two headline recommendations apply across the RWE research lifecycle, each with prioritised sub-recommendations: Essential, Important, and Optional.

Recommendation 1: Research Transparency to Strengthen Trust in RWE
Transparency is essential to build confidence among regulators and HTA bodies. Key essential actions: register study protocols before data access; document all analytical decisions and deviations; be transparent about data sources and provenance; report null findings alongside positive results. Important actions include sharing analysis code and reporting to standards such as RECORD or STROBE.

Recommendation 2: Establishing the Fundamentals for Data Integrity and Governance
A standardised framework for documenting data quality and fitness for purpose, across three elements:

- Data Extraction: pre-specify algorithms; document coding decisions; maintain audit trails.
- Data Curation: document cleaning and transformation steps; apply standardised coding (e.g. ICD, ATC).
- Data Characterisation: describe the study population; assess confounders; report generalisability and representativeness.

The RWE Research Process Map
The recommendations are mapped to the seven stages of a generalised RWE research workflow (see Figure 2 below).

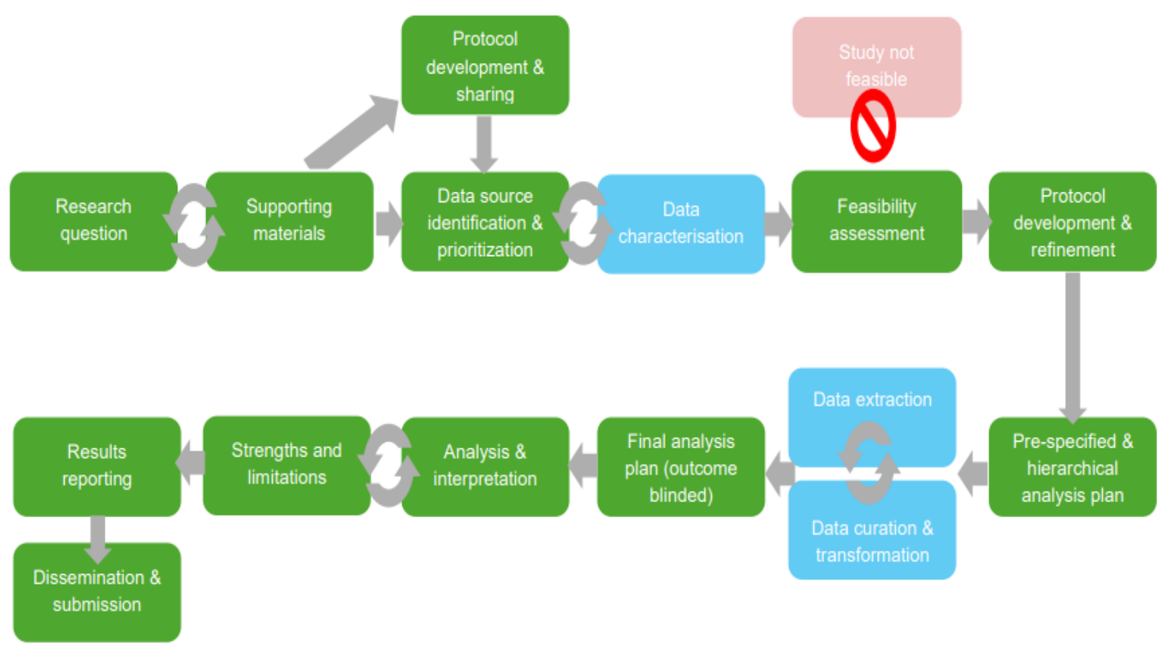


Figure 2: General RWE process map developed by IDERHA Task 6.4

VALUE OF IHI COLLABORATION

IDERHA brought together regulators, HTA bodies, pharmaceutical and MedTech companies, patient advocates, and academic researchers. Each partner brought something the others could not: industry understood the practical realities of data generation; regulatory and HTA partners defined what decision-makers need to trust evidence; academic researchers contributed methodological rigour; and patient advocates ensured the framework reflects the populations these recommendations are designed to serve.

Built through genuine cross-sector consensus, these policy recommendations and framework are grounded in operational reality, scientific rigour, and decision-maker expectations simultaneously - something that would not have been possible without the IHI public-private partnership model.

DISCLAIMER & ACKNOWLEDGEMENTS

Views are my own and not that of my employer, the National Institute for Health and Care Excellence, nor any of the funding partners of the Innovative Health Initiative, or any of its affiliated entities.

The IDERHA project is supported by the Innovative Health Initiative Joint Undertaking (JU) under grant agreement No 101112135. The JU receives support from the European Union's Horizon Europe research and innovation programme and life science industries represented by COCIR, EFPIA / Vaccines Europe, EuropaBio and MedTech Europe. The UK participant is supported by UKRI grant No 10079453 (National Institute for Health and Care Excellence).

This project is supported by the Innovative Health Initiative Joint Undertaking and its members.