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BEST PRACTICES AND RECOMMENDATIONS

How Can We Enhance the Use of RWE in Reimbursement Decisions?

CONTRACT RESEARCH

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Background

- Innovative medicines are increasingly challenging our standard paradigms of evidence generation.
- While randomised control trials (RCTs) remain the gold standard for establishing efficacy,[1,2] they often fail to capture broader questions of long-term effectiveness, safety in routine practice, and relevance to diverse patient populations.
- Real-world evidence (RWE) fills these gaps by drawing on real-world data (RWD) sources such as registries, claims databases, and electronic health records.
- Globally, health systems are increasingly acknowledging the value of RWE to inform health technology assessment (HTA), reimbursement decisions, and policies that directly impact patient access to medicines.
- RWE can better inform reimbursement decisions in the presence of uncertainty, but is currently underutilised.
- This research focused on key considerations for the use of RWE to assess clinical and comparative effectiveness and thereby inform reimbursement decisions made by HTA bodies

Aim

This research aimed to explore best practices for the use of RWE by HTA bodies. We examined the challenges that hinder the routine use of RWE, and make recommendations for increasing its credibility, transparency, and ultimately its use in decision-making.

Methods

1. We conducted a targeted literature review, focusing on guidance from established HTA bodies and highly cited academic publications.
2. We identified common themes and actionable recommendations for improving the generation and use of RWE in HTA.
3. We used Taiwan as a case study to explore how these insights apply in a real-world context.

Key challenges for including RWE in reimbursement decision-making

Data Quality Concerns	• Lower internal validity of RWD studies[3] • RWD studies are prone to measurement and publication bias and unmeasured confounding[4,5]
Lack of trust in RWE	• Lack of transparency in study design • Absence of data sharing
Incorporating into Decision-making	• Timeliness of RWE generation[2] • What questions do HTA bodies think can be answered by RWE?[6] • What data do HTA bodies believe is appropriate for answering different research questions?[2]
Knowledge of HTA Bodies	• Expertise to critically appraise RWE[6]

Taiwan Case Study

Taiwan's National Health Insurance Administration (NHIA) has pioneered the use of RWE in the Asia-Pacific, leveraging comprehensive health data systems.

- RWE is formally used to support reimbursement and outcome-based managed entry agreements, providing real-world insights into treatment performance.
- The National Health Insurance Research Database (NHIRD)[7] and disease-specific registries[8,9,10,11] supply rich, linked datasets that enable RWE generation
- Renewing managed entry agreements, such as those for hepatitis C treatments[8,9] and immune checkpoint inhibitors,[10,11] link real-world outcomes to informed decision-making.
- Challenges persist, including ensuring data quality, aligning outcome measures with decision-making needs, improving transparency, and involving stakeholders more directly in data use.

KEY LEARNING:
Even with good access to de-identified data and established registries — opportunities remain to improve data quality and scientific rigor. The case study underscored the need for better infrastructure, stronger governance, and more systematic approaches to enable robust RWE generation that can inform local reimbursement decisions.

Recommendations

ESTABLISHING TRUST IN RWE
Transparency in data collection and methods used to transform RWD to RWE would allow decision-makers — including HTA bodies — to better assess the internal validity, quality, and suitability of RWE,[5, 12] which is essential to establishing trust in the results. This can be achieved through:

- Pre-registration of studies — Researchers should pre-register RWD study protocols in the public domain, but wider adoption will require incentives such as requirements from data owners, journal editors and HTA bodies.[5]
- Publication of results — Results of all RWD studies, whether favourable/unfavourable or significant/insignificant, should be published. In reporting results, authors should explore and identify any methodological issues and potential sources of bias and discuss how these may impact the presented results.[5]
- Information governance — Robust processes for capturing the raw data, cleaning and managing the data, linkage and aggregation, and finally, access/use need to be defined and agreed.[13]

INCLUSION OF RWE IN REIMBURSEMENT DECISION-MAKING
To fully integrate RWE into reimbursement frameworks, decision-makers must establish clear processes and collaborative practices. This requires:

- Process infrastructure — HTA bodies need to ensure they have capacity and capability to assess RWE.
- Collaboration — Manufacturers, HTA bodies and other stakeholders should work together to develop a shared view of the role that RWE should play in reimbursement decision-making. They should also work together to produce data analysis and process standards.[2] Standards should be set by decision-makers and communicated to generators of RWE to ensure that the RWE generated is robust and provides meaningful information to decision-makers.[3]

Discussion and Conclusion

- **Significant work remains to standardize and strengthen the role of RWE, ultimately supporting better informed and more responsive access decisions for medicines.**
- **To fully realize the potential of RWE in reimbursement decision-making, we urge stakeholders internationally to work together to advance the use of high-quality real-world data, thereby promoting trust in its use for reimbursement decision-making.**

Read the full report here

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