Medicines Australia | Better health through research and innovation

Real world evidence

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Australia needs standards for accepting real world evidence in regulatory and reimbursement evaluations

- Real world evidence (RWE), or observational data, is fundamentally changing healthcare by providing a more complete picture of the safety and effectiveness of medical technologies in "real-world" patient populations
- Australia currently lacks clear guidance on how RWE will be considered in regulatory and reimbursement evaluations, leading to inconsistent and unclear processes
- Lack of access and poor linkage of healthcare data sources prevents sponsors (usually pharmaceutical companies) from generating robust Australian RWE that could present better evidence for HTA and enhance decision making

Possible policy solutions

1. Adopt a high level, principles-based framework for accepting and assessing RWE. This would be a single standard that would be used by the Therapeutic Goods Association (TGA), Pharmaceutical Benefits Advisory Committee (PBAC), Medical Services Advisory Committee (MSAC) and other Australian decision makers. This could be based on the UK NICE Framework[1]. Guidance is needed on when and where RWE is appropriate to use, how to demonstrate its relevance and develop standards for data integrity.
2. Develop standards for the utilisation of RWE for post-marketing monitoring in the reimbursement context. This would cover provisional listings, managed entry, or other interim funding mechanisms.
3. Enhance system infrastructure to centralise linked health data and provide appropriate access to stakeholders, including industry. A proposed model of linked health data would require:

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1 The NICE Strategy 2021-2026 has recognised that RWD is essential to enabling rapid, robust, and responsive technology evaluations and dynamic, living guidelines. NICE has developed an RWE framework which provides in-depth guidance and tools to support the implementation of these core principles across different uses. There is early engagement with NICE Scientific Advice if sponsors plan to use real-world data in their submissions as part of their evidence-generation plans. To make this easier, the UK's regulatory agency (MHRA) has guidelines on using real-world data to support regulatory decisions. National Institute for Health and Care Excellence, NICE strategy 2021 to 2026, UK, 2021 https://static.nice.org.uk/NICE%20strategy%202021%20to%202026%20-%20Dynamic,%20Collaborative,%20Excellent.pdf

- Independent entity or entities that is aligned to other best practice approaches internationally
- Government investment to implement capability, with a user-pays pricing model
- Common data model that transforms data into common formats using standardised terminologies and vocabularies
- Governance structure that is single, independent, scientific and allows for ethical review of projects. This would remove duplication of ethical and scientific review of projects by multiple jurisdictional entities.

These options would include evolving the existing Australian Institute of Health and Welfare (AIHW) datasets[2] to be utilised for approved purposes, including generating evidence for reimbursement and conducting post-marketing studies.

The importance of RWE

RWE provides evidence of the usage and potential benefits or risks of a medical product[3]. Common sources include electronic health records (EHRs), hospital episode data, claims data (PBS and MBS) and patient registry data (product and disease), chart reviews, clinical audits, and observational cohorts. RWE is an evidence base usually made available from clinical trials that provides a more complete picture of treatment effectiveness and safety within a real-world patient population.

RWE is important both in supporting ethical study design and overcoming design limitations of randomised controlled trials (RCTs). High quality evidence may be generated where there are clear frameworks that detail the data elements, characteristics, and the internal validation processes to be used.

Utilisation of RWE in patient access decisions

RWE can be used to support claims of efficacy or safety in reimbursement applications, regulatory approvals or monitor outcome in the post-marketing setting, in addition to clinical trial data. It is often used in situations where the data is scarce or where RCTs are not feasible or ethical (e.g., rare diseases and paediatric populations).

The use of RWE is under active consideration by the TGA.[4] This provides an opportunity to achieve consistency and efficiency between registration and reimbursement. It is noted that because Australia is a small market in the global context, any bespoke Australian requirements will be problematic.

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2 Australian Insitute of Health and Welfare, Data linkage: accessing data, Australian Government, 2021 https://www.aihw.gov.au/our-services/data-linkage

3 U.S. Food and Drug Administration, *Real-World Evidence*, United States Government, 2022 https://www.fda.gov/science-research/science-and-research-special-topics/real-world-evidence

4 In May 2021, the TGA commissioned a rapid review including around 50 targeted stakeholder interviews on their understanding, and use, of RWE and patient-reported outcomes (PROs).
Therapeutic Goods Administration, Real world evidence and patient reported outcomes in the regulatory context, Australian Government, 2021
https://www.tga.gov.au/sites/default/files/real-world-evidence-and-patient-reported-outcomes-in-the-regulatory-context.pdf

While RWE is currently accepted in HTA submissions in Australia, the extent to which it is actually taken into account varies. Examples where RWE has been accepted include treatment pattern analysis, estimating the size of patient populations and financial impact, and informing Real World comparator data[5]. In other scenarios, the role of RWE is less clear and its acceptance can be inconsistent.

Addressing the Australian patient access gap with RWE

IQVIA's HTA Accelerator database shows that the UK and France are clear leaders in terms of number of decisions that reference RWE (55% and 47% of all submissions). Germany, Canada and Australia show much more limited use of RWE, with 10%, 6% and 5% of decisions referencing RWE, respectively.[6]

Comparable countries provide examples of how to improve the utility of healthcare data. Denmark, Sweden and the US Sentinel System[7] have established linked data infrastructure across the healthcare system. England has made substantial investments in linking data across NHS-funded services, and both England and Canada have frameworks for the incorporation of RWE into HTA.

The Strategic Agreement between the Australian Government and Medicines Australia makes a commitment to review HTA policy and methods, which is the first comprehensive review of Australian HTA in 30 years. This is an important opportunity to introduce bold reforms that will speed up Australians' access to new, innovative medicines by enhancing the adoption of RWE in HTA decisions.

Feedback

Do you have any thoughts on the policy ideas in these papers? We'd love to hear your feedback! Please let us know at this email address: HTA-Reform@medicinesaustralia.com.au.

5 Medicines Australia - Oncology Industry Taskforce, THE EVOLVING ROLE OF REAL-WORLD EVIDENCE IN AUSTRALIA, Medicines Australia, 2020 https://www.medicinesaustralia.com.au/wp-content/uploads/sites/65/2020/11/Oncology-Industry-Taskforce_-Real-World-Evidence-in-Australia-Report-NOV-2020.pdf

6 *Ibid.*

7 U.S. Food and Drug Administration, FDA's Sentinel Initiative - Background, United States Government, 2022 https://www.fda.gov/safety/fdas-sentinel-initiative/fdas-sentinel-initiative-background

NOTE: This Discussion Paper is not a final position paper. It has been developed as a conversation starter and to support discussion and feedback