A review of the recent history of the use of RWE in Australian PBAC decision making

RWD265

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INTRODUCTION

The utilisation of real-world evidence (RWE) is becoming increasingly important in the decision making of reimbursement authorities. Traditionally, randomised controlled trials (RCTs) have been considered the gold standard for establishing clinical efficacy; however, they often face limitations in terms of generalisability, cost, and feasibility - especially in rare or severe disease contexts (Makady et al., 2017; Sherman et al., 2016). RWE, derived from observational data sources such as electronic health records, registries, and insurance claims, offers an opportunity to complement or, in some cases, substitute RCT data in reimbursement decisions (Franklin et al., 2019; Makady et al., 2018). However, the extent and manner of its incorporation into health technology assessment (HTA) submissions remain unclear.

The Pharmaceutical Benefits Advisory Committee (PBAC), which evaluates evidence to inform public subsidy of medicines under the Pharmaceutical Benefits Scheme (PBS) in Australia, must balance uncertainty in clinical data with cost-effectiveness and equity considerations (George et al., 2011). While RWE has been used in PBAC submissions, particularly in cases where RCTs are not feasible, the frequency, contexts, and outcomes of such submissions have not been systematically analysed. This study therefore investigates the use of RWE in PBAC submissions between March 2020 and March 2025, assessing its impact on decision-making and recommendation outcomes.

OBJECTIVE

To analyse Pharmaceutical Benefits Advisory Committee (PBAC) decision making to understand the history of, and value placed on RWE in the decision making for reimbursement of pharmaceuticals in Australia.

METHODS

Method of Data Extraction

- Evidence presented as public summary documents (PSDs), was used to investigate the relationship between submission components - such as the incremental cost-effectiveness ratio (ICER), financial impact, disease rarity - with the presented evidence base (randomised evidence vs RWE).
- An Excel database was created based on all major submissions to the PBAC between March 2020 and March 2025 where the PBAC decision relied upon a cost-utility analysis (CUAs, n=378).
- Randomised evidence was defined as evidence with a randomised component (i.e., RCTs) while RWE was defined as any non-randomised evidence (Makady et al., 2017).
- Data extraction/review of PSDs was split randomly amongst and conducted by 5 experienced Health Technology Assessment consultants.
  - Extracted data was reviewed by a second consultant (for double checking/conflict resolution and data cleaning).

Method of Data Analysis

- The data were analysed on Excel and R software to capture descriptive statistics (e.g., distribution of PBAC outcomes [recommended, deferred and rejected], proportion of submissions with life-threatening or rare diseases, proportion of submissions where PBAC accepted the treatment effect, etc.)
- Summary statistics were used to analyse the use of RWE in PBAC submissions and decision making over time and its impact on favourable PBAC recommendations.
- The probability of recommending a medicine for funding was estimated using multivariate probit regression models and analysed on STATA and R software.
- In the multivariate regression model the outcome variable (PBAC outcome) was coded as “Recommended” (1) or Rejected/Deferred (0).

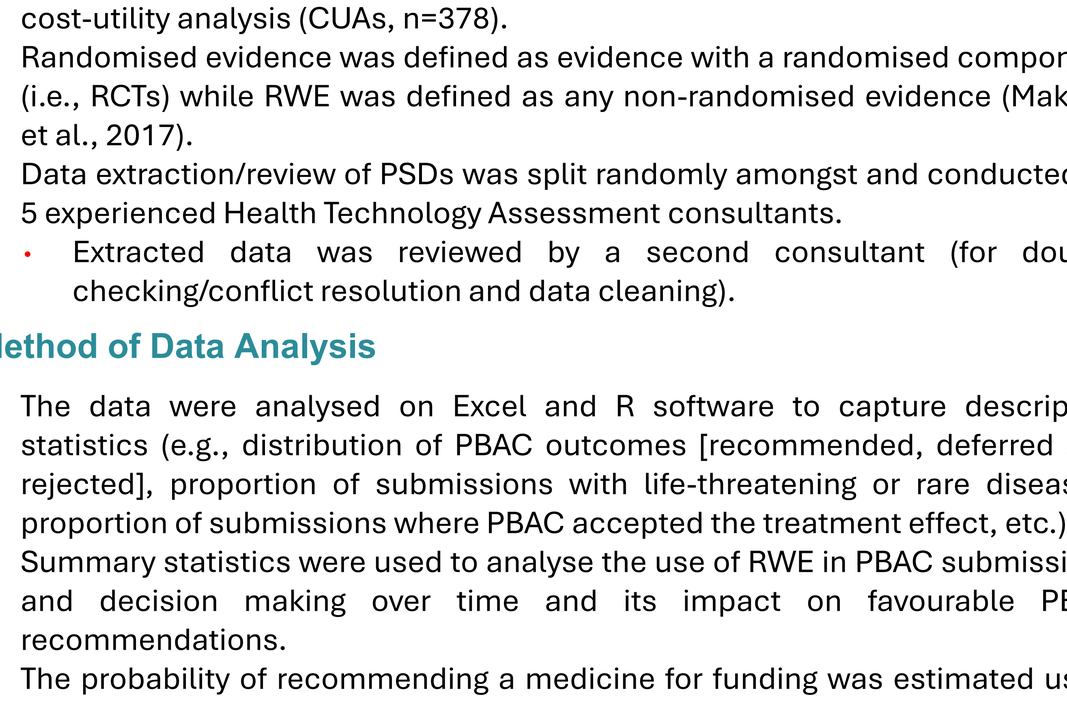

Figure 1: Proportion (%) of submissions with “significant” clinical evidence

Abbreviations: RWE = real world evidence. Notes: n = number of total submissions (treatment-indication pairs) in given year. “Significant” defined as the PBAC consider the size (point estimate) of the treatment effect to be clinically important and/or the measure of the size of the treatment effect is statistically significant at a 5% level.

RESULTS

16.4% of the CUAs from March 2020 to March 2025 used non-randomised evidence (62/378), with ~10 submissions per year. Of the submissions based on non-randomised evidence, the proportion considered by the PBAC to have a “significant” treatment effect appears to be increasing over time (from 29% in 2020 to 100% in 2025); **Figure 1**. Note: there is only one meeting worth of information for 2025 available at current, resembling a reduced sample size (n=42). The probability of recommendation is also higher for submissions with non-randomised evidence compared to randomised evidence, explained by RWE being more likely used for submissions with rare conditions (**Figure 2**). Despite this, regression results suggest that when holding all other variables constant at their mean, the probability of a recommendation increases by 5.42 percentage points for submissions with randomised evidence compared to non-randomised evidence (**Table 1**).

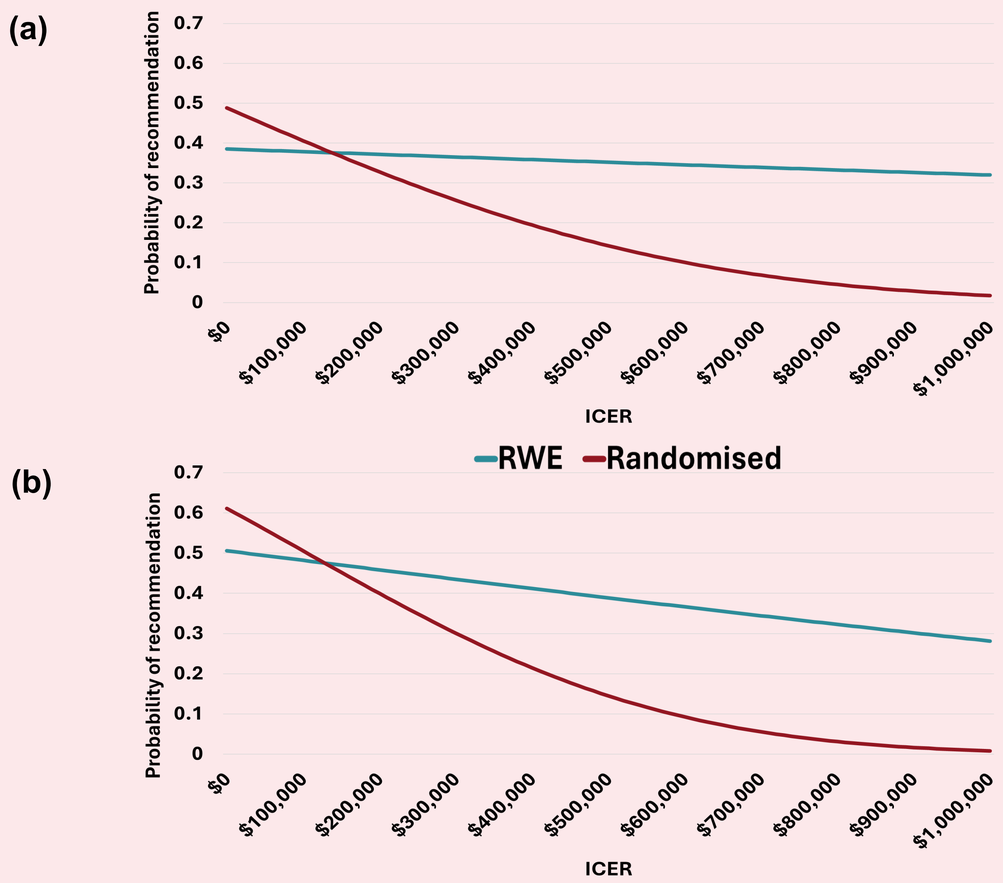

Figure 2: Predicted probability of recommendation given type of evidence in (a) all conditions and (b) rare conditions

Abbreviations: ICER = Incremental Cost-Effectiveness Ratio.

Table 1: Regression results

	Estimate	Std. Error	P-value	Change in % of recommendation
Intercept	-1.042	3.435	0.00242	-
Randomised evidence	1.421	2.400	0.55367	**5.42**

Abbreviations: Std. = standard.
Note: Variables with multicollinearity and which were non-significant (p>0.2) were removed from the regression.

CONCLUSION

While RWE use in PBAC decision making seems to be constant over the last five years, acceptance of RWE and its results in terms of treatment effect appears to be growing in certain circumstances, especially for rare conditions. By examining trends in the acceptance of non-randomised evidence over time, and comparing outcomes with RCT-based submissions, this research provides critical insights into the evolving role of RWE in Australian HTA processes. Further research is needed to better understand the contextual factors that influence how RWE is evaluated and weighted against traditional RCT data.

References

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Abbreviations: CUAs = cost-utility analyses, ICER = incremental cost-effectiveness ratio, n = number, PBAC = Pharmaceutical Benefits Advisory Committee, PBS = Pharmaceutical Benefits Scheme, PSD = Public Summary Document, RCT = randomized controlled trial, RWE = real-world evidence.