RWD24

Acceptance of Real-World Evidence in HTA in Japan: Is the Potential Being Realised?

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Objective

To understand the use and acceptance of real-world evidence (RWE) in health technology assessment (HTA) in Japan.

Background

- Consideration of RWE in HTA decision-making is increasingly being viewed as an effective way of accelerating patient access to innovative new treatments and driving healthcare improvements.[1]
- People of Asian ethnicities have long been underrepresented in clinical research,[2, 3] leading to a dearth of relevant data for use in local HTA. RWE is recognised as an important mechanism for closing this gap and demonstrating the effectiveness and safety of new therapeutic options in relevant populations.[3, 4]
- In Japan, the use of RWE in the regulatory assessment process has been promoted since 2014.[5] However, the use and acceptance of RWE in the HTA process is not well understood in Japan.

Methods

- Cost-effectiveness evaluation reports (in English or Japanese) for medicines, published by the Center for Outcomes Research and Economic Evaluation (C2H) between 2019 and 25th March 2025, were reviewed.[6]
- Each RWE use case by the manufacturer and/or Academic Technology Assessment Group (ATAG) was identified and categorised according to study design, evidence type (effectiveness and safety, economic modelling, utilities/quality of life [QoL], healthcare resource use [HCRU]). Extracted information included RWE source and ATAG acceptance and/or critique.

Results

- Out of 37 drug evaluation reports, 32 (86.5%) reported use of RWE. A total of 74 RWE use cases were identified, including 51 by manufacturers (**Figure 1**) and 23 by the ATAG. RWE was most often used to provide economic model inputs and HCRU evidence (**Figure 2**).
- Where RWE was presented to demonstrate effectiveness, issues with study design and methodology (e.g. selection bias) were raised by the ATAG. This use case was generally not accepted by the ATAG where data from clinical trials were also presented (**Figure 3**). In one case, where efficacy data were not available from an ongoing interventional trial, the ATAG accepted an effectiveness claim on the basis of observational studies, despite critique of the studies presented.
- RWE generated from overseas populations was reported in 9 cases (2 effectiveness and safety, 2 economic modelling, 5 utilities/QoL). ATAG expressed concern over the appropriateness of non-Japanese data in four cases, but ultimately accepted the RWE in two cases due to lack of alternative data. In total, use of overseas RWE was not accepted by the ATAG in 4/9 cases, with the key reason for non-acceptance being methodological concerns relating to data generation.
- The 23 RWE analyses conducted by the ATAG were predominantly database analyses to validate economic model inputs and HCRU estimates used by the manufacturer (**Figure 2**).

Conclusion

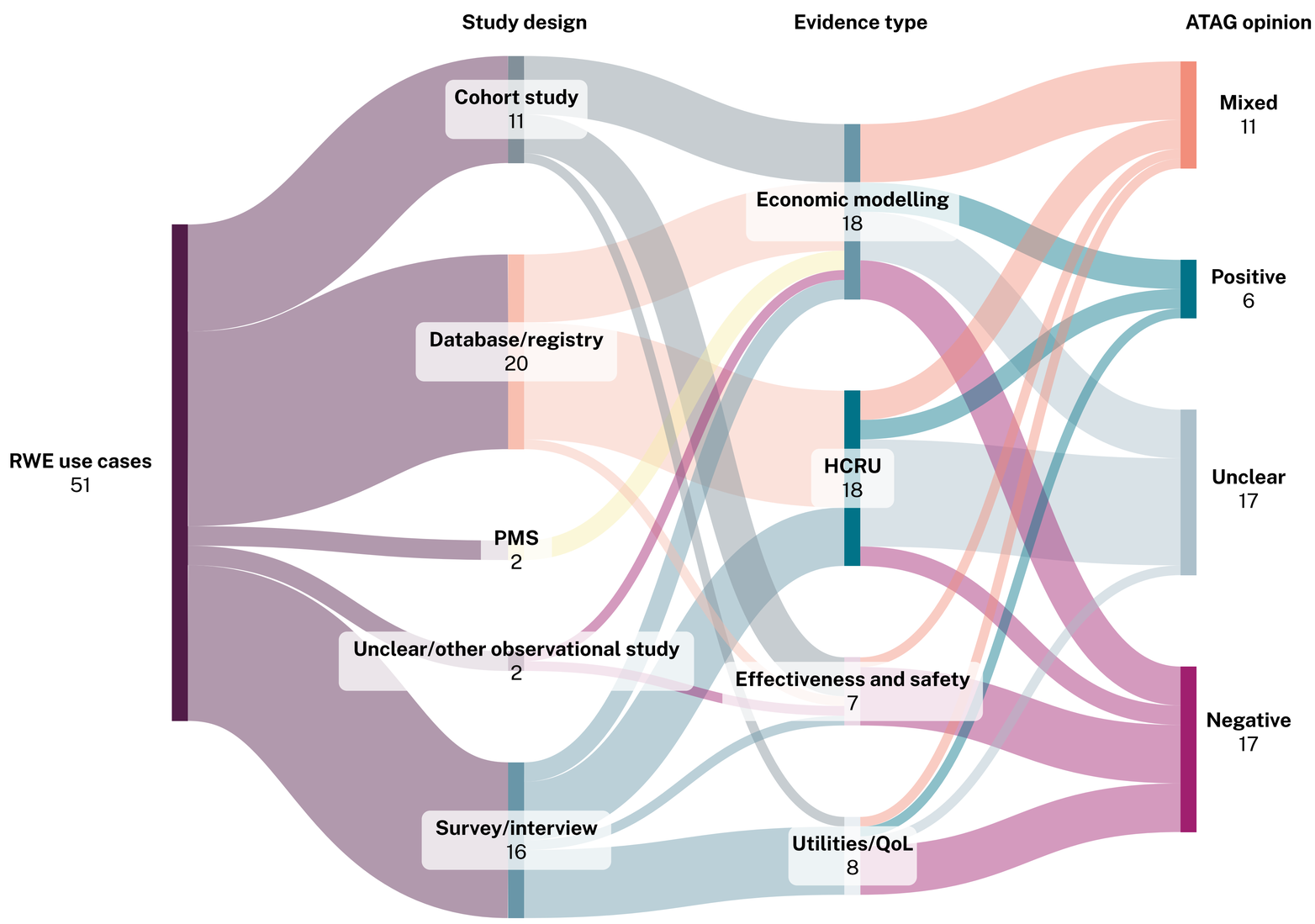
The majority of C2H assessments report the use of RWE; use of database analyses to inform economic modelling inputs was common. However, ATAG acceptance of RWE varied by use case.

Concerns with methodological rigour limited the acceptability of RWE, while RWE from overseas was critiqued on its applicability to the Japanese setting.

Rigour, transparency and consideration of context in RWE generation are paramount to its value and acceptance as supporting evidence in Japanese HTA.

FIGURE 1

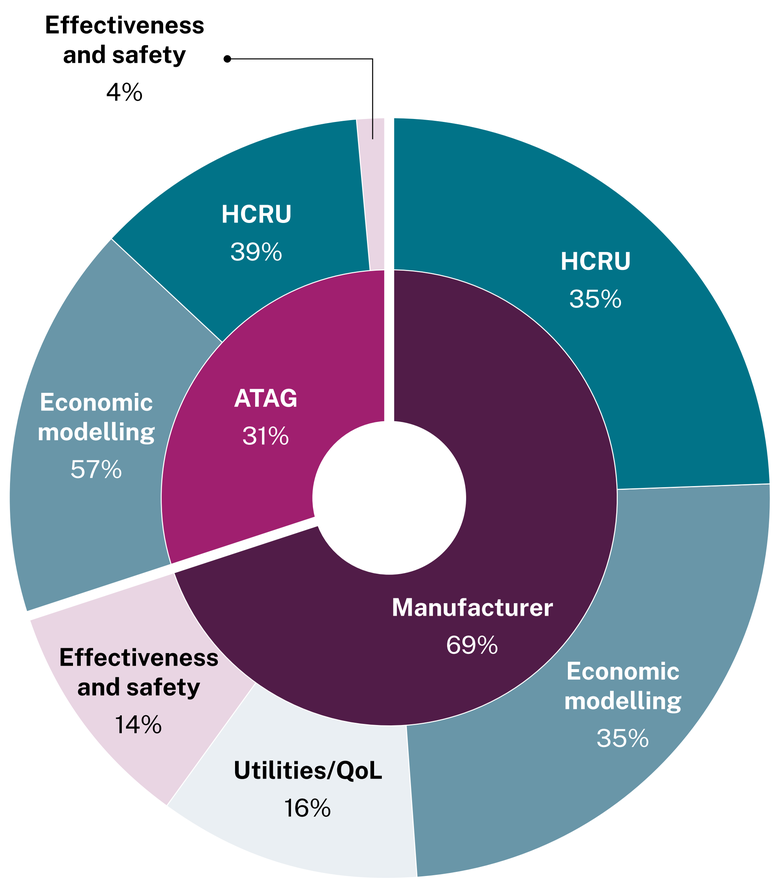
Use cases of RWE by the manufacturer



Categories of ATAG opinion: Positive, ATAG accepted the use of RWE for decision-making; Mixed, ATAG accepted some aspects of the use of RWE but critiqued others e.g. criticised study design but accepted evidence due to lack of alternatives; Negative; ATAG did not accept the use of RWE for decision-making; Unclear, ATAG acceptance/critique of manufacturer use of RWE not reported.

FIGURE 2

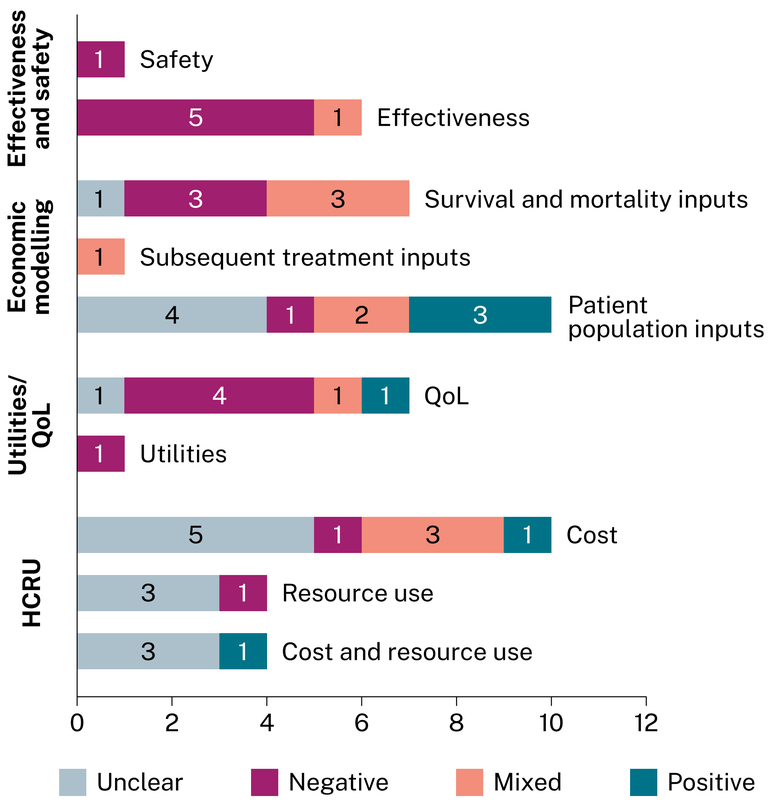
Use cases of RWE by the ATAG vs the manufacturer



For any use cases where it was unclear which stakeholder has used the RWE, it was assumed to have been the manufacturer.

FIGURE 3

ATAG acceptance of RWE use cases by the manufacturer in each area of the HTA appraisal



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Abbreviations: ATAG: Academic Technology Assessment Group; **C2H:** Center for Outcomes Research and Economic Evaluation; **HCRU:** healthcare resource use; **HTA:** health technology assessment; **PMS:** post-marketing surveillance; **QoL:** quality of life; **RWE:** real-world evidence.

References: [1]Cowling, T et al. J Med Econ. 2023;26(1):944–953; [2]Azzopardi, R et al. JACC Asia. 2023;3(5):724–735; [3]REALISE Working Group. Use of Real-World Data and Real-World Evidence to Support Drug Reimbursement Decision-Making in Asia. Available at https://hiper.nus.edu.sg/wp-content/uploads/2021/05/REALISE-Abridged-guidance-for-users-of-HTA_20201104-version-1.0-2.pdf (Last accessed 26 August 2025). 2021; [4]Lou, J et al. Int J Technol Assess Health Care. 2020;36(5):474–480; [5]Nishioka, K et al. Clin Parmacol Ther. 2022;111(1):35–43; [6]C2H. Core to Evidence-Based Health Policy. Available at https://c2h.niph.go.jp/en/ (Last accessed 27 August 2025). **Acknowledgements:** The authors thank Brenda Ow Yong, Costello Medical, for graphic design assistance.

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