HTA guidance updates and usage of Real-World Evidence (RWE) in reimbursement decisions – A 2023 perspective

HTA196

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Background

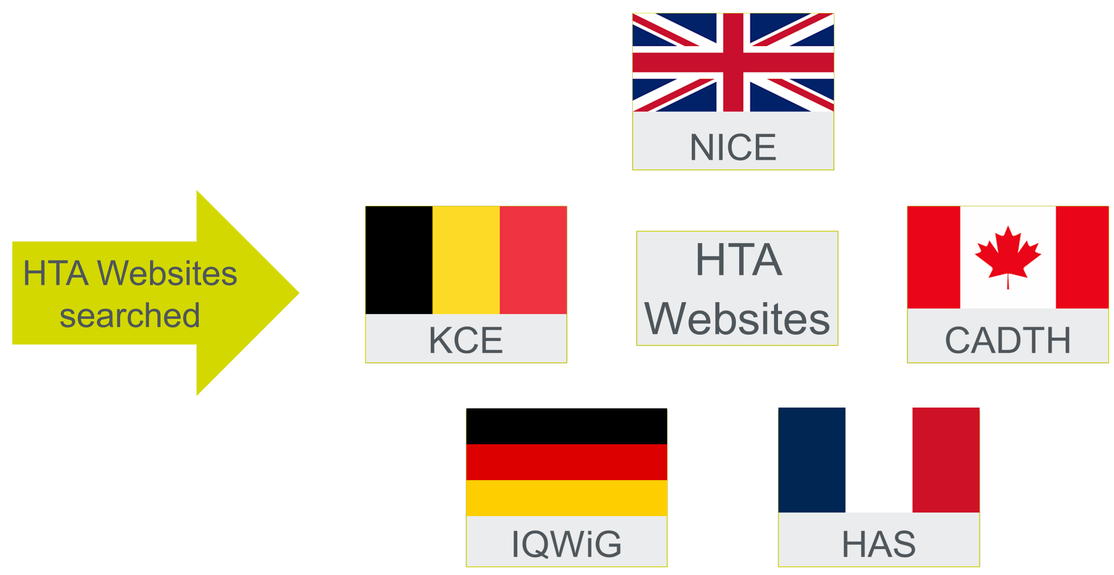
RWE is gaining momentum where obtaining evidence from randomized controlled trials is challenging or is sparse. Typically, in HTAs, RWE is used to support RCT findings to increase precision and enhance the decision-making process for HTA bodies and payers. Thus, these bodies are increasingly accepting RWE in decision making. The objective of this research was to identify HTA guidance updates on usage of RWE and analyzing trends in reimbursement decisions involving RWE in 2023.

Methods

Hand searching of the below websites was undertaken to retrieve HTA submissions and latest guidance on the use of RWE in HTA decision making during 2023 (Figure 1). Additionally grey literature was searched to retrieve the relevant evidence.

- UK-NICE: National Institute for. Health and Care Excellence
- Canada-CADTH: Canadian Agency for Drugs and Technologies in Health
- France-HAS: Haute Autorité de Santé
- Germany-IQWiG: Institute for Quality and Efficiency in Health Care
- Belgian-KCE: The Belgian Health Care Knowledge Centre

Figure 1: Key websites searched



Results

- The search identified a guidance document from CADTH and HAS position paper.
- CADTH provided guidance covered reporting of RWE (12 sections covering research question & study design; setting & context; data specifications, access, cleaning method linkages; data sources, data dictionary and variables; participants; exposure definitions & comparators; outcomes; bias confounding and effect modifiers or sub-group effects; statistical methods; study findings; interpretation and generalizability; and limitations) and recommendations on submission preparation to both regulatory and HTA agencies (Figure 2).
- CADTH launched this guidance for faster patient access to newer technologies while resolving issues of uncertainty.
- HAS paper suggested use of RWE as an external control arm in absence of RCTs and conducting pragmatic trials (trials within a cohort, registry-based RCT, contactless trial or direct-to-patient trial).
- RWE usage in HTA submissions showed upward with 12/38 NICE and 19/33 CADTH submissions referring to RWE while appraising technologies. Most of these (9/12, NICE submissions; 18/19 CADTH submissions) received positive outcome from agencies (Table 1).

Figure 2: Summary of CADTH guidance for reporting of RWE

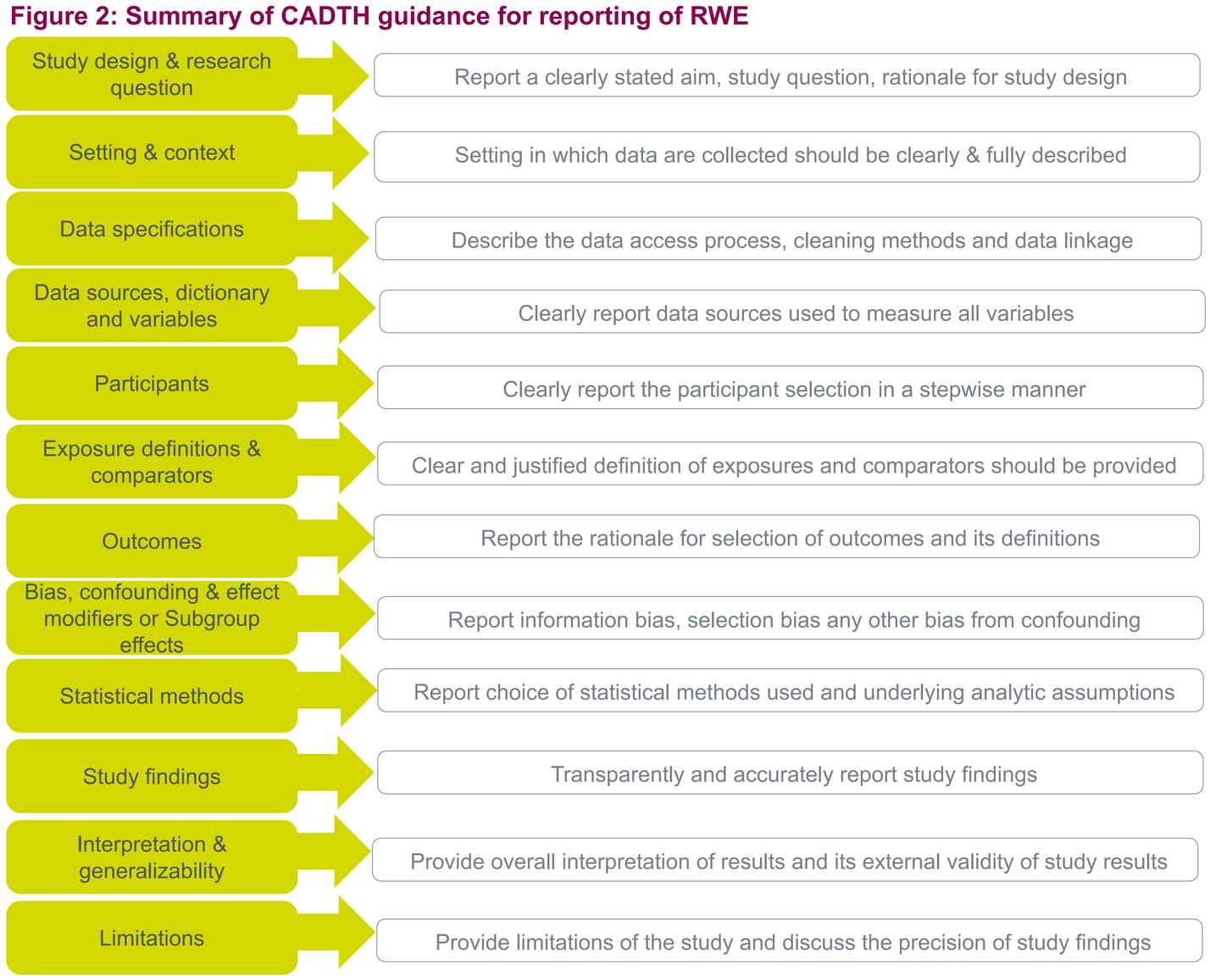


Table 1: RWE usage in HTA submissions across all therapeutic areas (Jan 2023-June 2023)

HTA websites	Submissions	Oncology	Blood and immune	Others	All disease areas
NICE	Total number of records	13	10	15	38
	Number of records with RWE	3	4	5	12
	Positive recommendations for RWE	3	2	4	9
CADTH	Total number of records	8	10	15	33
	Number or records with RWE	8	7	3	19
	Positive recommendations for RWE	8	7	2	18

Others: Metabolic, Digestive, Kidney, Neurological, Respiratory, Musculoskeletal, Neurodevelopmental conditions

Conclusions

RWE usage in HTA submissions is increasing with availability of recently published guidance to generating best-practices for planning, conducting, and reporting of RWE. Early engagement and further collaboration between HTA agencies and industry is warranted.

REFERENCES

[1] https://www.cadth.ca/sites/default/files/RWE/MG0020/MG0020-RWE-Guidance-Report-Secured.pdf
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[3] https://www.cadth.ca/
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[6] https://www.inahta.org/members/kce/