Comparison of the use of real-world evidence for clinical effectiveness in HTA pre- and post- introduction of the NICE framework

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BACKGROUND

- Data insufficiency has led to payers becoming more receptive of real-world evidence (RWE) to inform clinical effectiveness in reimbursement decision-making.[1]
- On the 23rd of June 2022, the National Institute for Health and Care Excellence (NICE) introduced a framework to improve the quality of RWE used to inform guidance and to identify where RWE can reduce uncertainties.[2]

OBJECTIVE

- The aim of this study was to assess the framework's impact on the use of RWE to inform the clinical effectiveness of interventions assessed in the technology appraisal (TA) programme, within the first year of implementation.

METHODS

- The NICE website[3] was reviewed to identify TAs published pre-guidance (23.06.21 – 23.06.22) and post-guidance (24.06.22 – 24.06.23). TAs were removed if they had been terminated or if they were treatment guideline updates from TAs published more than 5 years ago. For each TA that included RWE in the clinical effectiveness section, the following were recorded: NICE recommendation, disease area, study type, location, the contribution to the clinical evidence, and the reason for inclusion.

RESULTS

- In total, 201 TAs were identified. Of the 201 TAs, 47 were excluded (42 terminated, 5 updates) **Figure 1**.
- Of the remaining 154, 72 (47%) were published pre-framework and 82 (53%) post-framework.
- Pre-framework, 20/72 TAs (28%) used RWE to inform clinical effectiveness versus 24/82 (29%) post-framework.
- 86% [38/44]) of HTAs that used RWE were recommended by NICE.

HTAs with RWE by disease category

- Oncology HTAs included RWE more commonly than any other disease area both pre- (15/20 [75%]) and post-framework (14/24 [58%]) (**Figure 2**).
- Pre-framework, the majority of oncology HTAs used the CDF (60% [9/15]), post-framework the proportion of oncology HTAs using the CDF substantially decreased (21% [3/14]).

HTAs with inclusion by real-world study

- Types of real-world data used to inform clinical effectiveness included the CDF dataset ([12/44] 27%), retrospective studies ([10/44] 23%), other registries ([5/44] 11%) and other observational studies ([17/44] 39% e.g., non-interventional studies and prospective studies). The real-world study types changed post-framework (**Figure 3**). While the proportion of observational studies increased (6/20 [30%] versus 11/24 [46%]), the proportion of Cancer Drugs Fund (CDF) studies decreased (9/20 [45%] versus 3/24 [13%]).

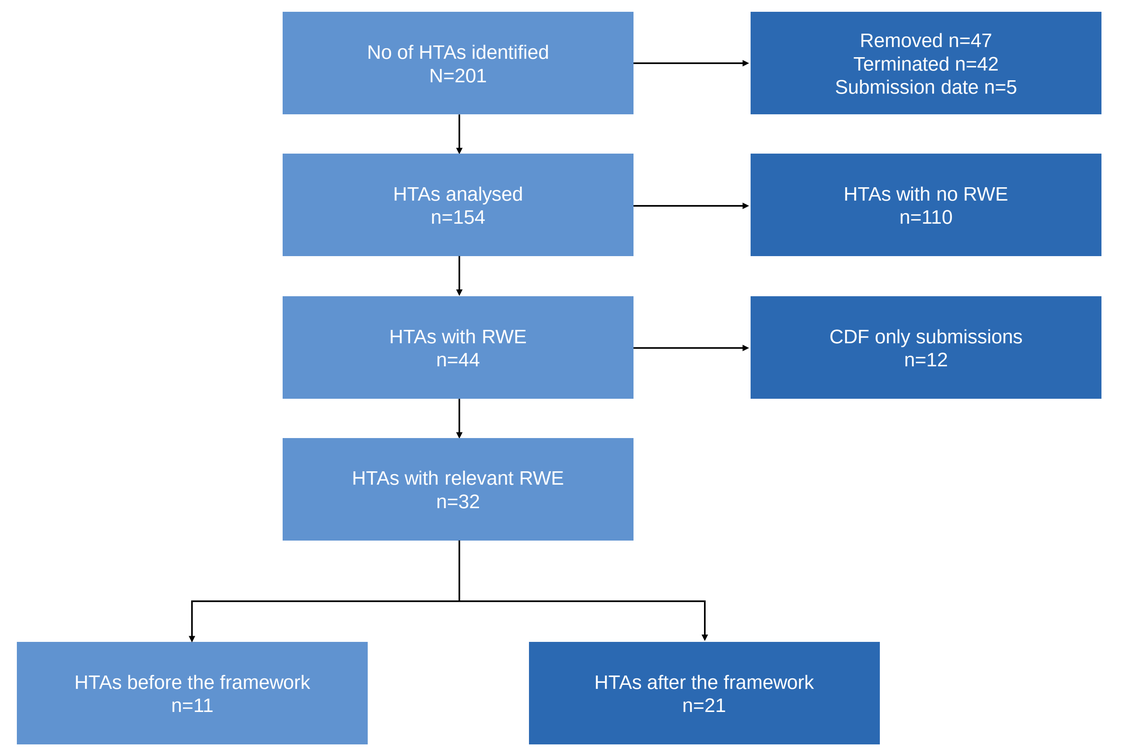
HTAs with inclusion of RWE by evidence type

- The use of RWE as a main or supporting evidence remained similar pre- and post-framework (**Figure 4**).

Reasons for inclusion of RWE in HTAs

- The main reasons for the inclusion of RWE included the formation of indirect treatment comparisons (20/46 studies [43%]) and to demonstrate generalisability of the evidence to National Health Service (NHS) clinical practice (15/46 [33%]). The use of RWE to form indirect treatment comparisons increased post-framework (8/20 [40%] to 12/24 [50%]) (**Figure 5**).

Figure 1. Flow-diagram of HTA review



CDF, Cancer Drug Fund; HTA, health technology assessment; RWE, real-world evidence

Figure 2. HTAs with inclusion of RWE by HTA disease category

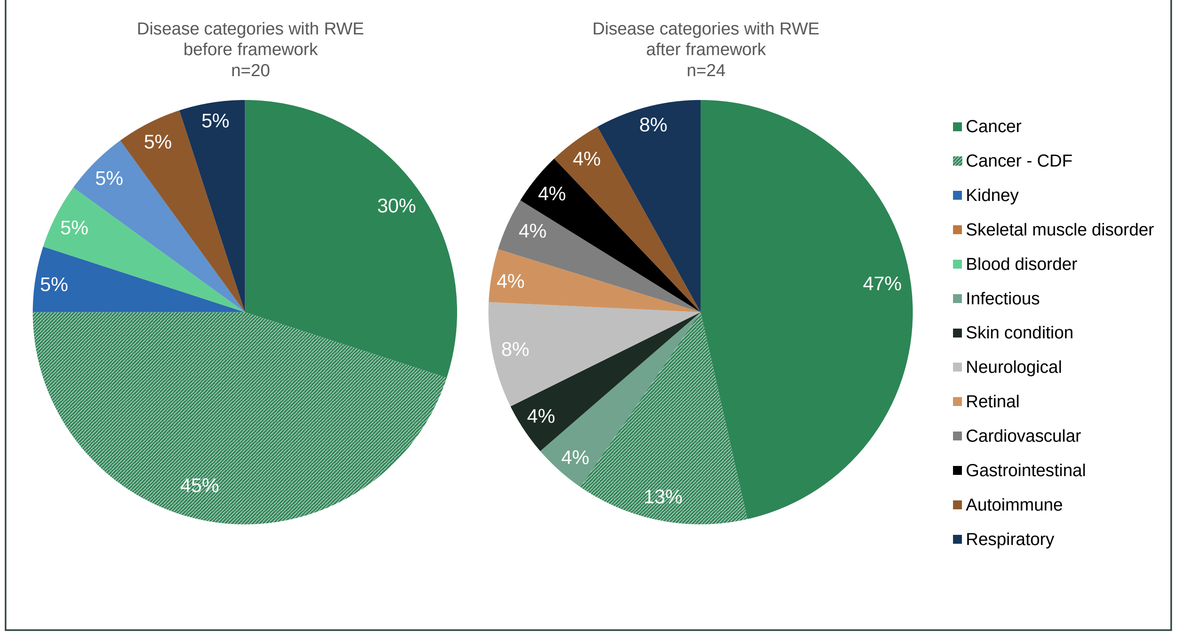
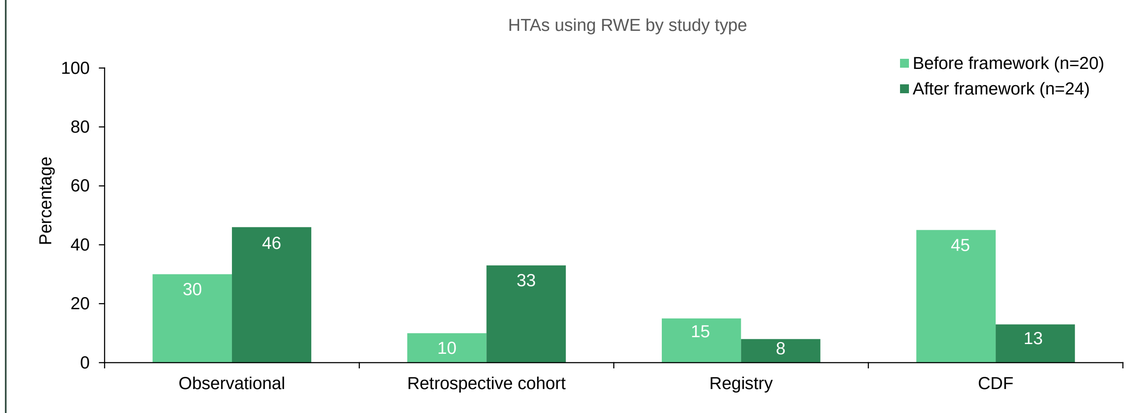
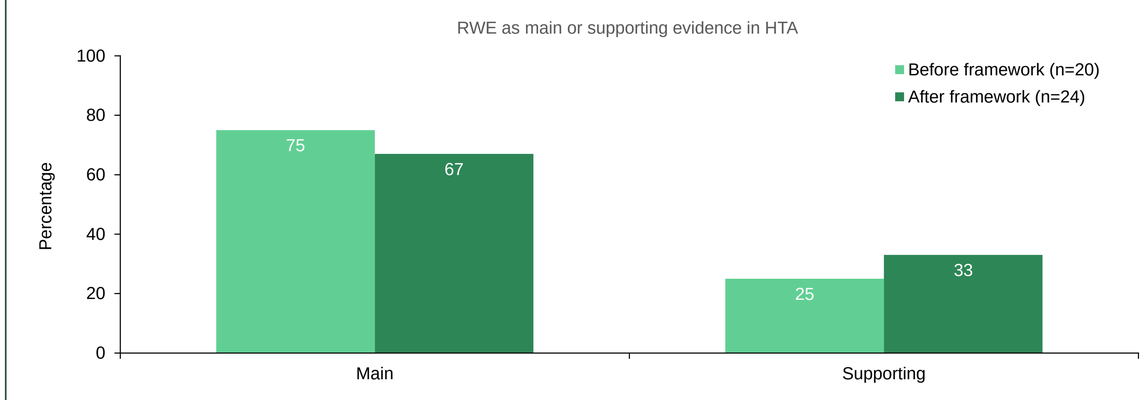


Figure 3. HTAs with inclusion by real-world study



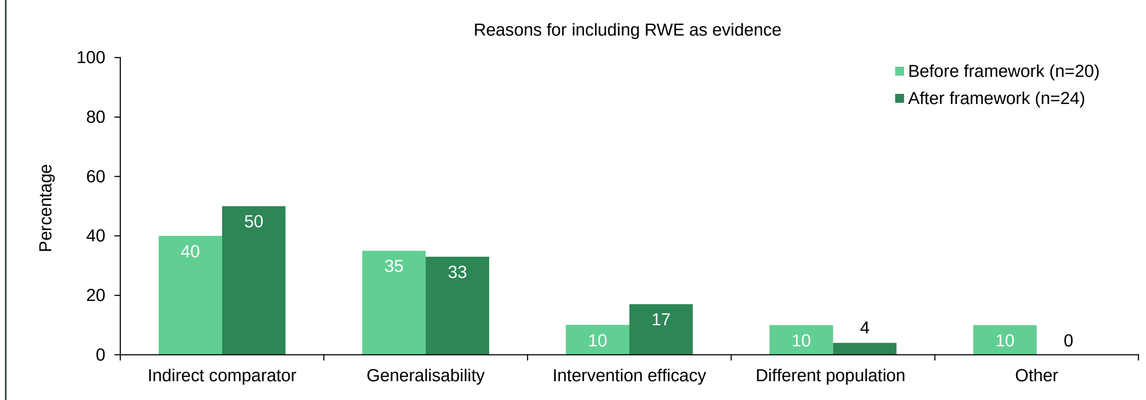
CDF, Cancer Drug Fund; HTA, health technology assessment; RWE, real-world evidence

Figure 4. HTAs with inclusion of RWE by evidence type



*RWE was defined as main evidence when it was mentioned in the final NICE guidance as a key evidence source for decision-making and supporting evidence when it was either mentioned elsewhere in the NICE guidance or only included in the committee paper(s).
HTA, health technology assessment; RWE, real-world evidence.

Figure 5. Reasons for inclusion of RWE in HTAs



HTA, health technology assessment; RWE, real-world evidence.

DISCUSSION AND CONCLUSION

- Oncology HTAs included RWE more than any other disease area. Challenges associated with conducting randomised controlled trials (RCTs) in rarer tumour types, regional discrepancies in the standard of care, and the availability of real-world data sources, including the CDF's Systemic Anti-Cancer Therapy (SACT) dataset, may have driven this.
- Post-framework, the proportion of TAs using RWE to support clinical effectiveness remained unchanged. However, there was a shift in the types of real-world studies used. The use of the CDF's SACT dataset decreased, while the use of RWE from other sources increased. The CDF is reserved for promising oncology drugs associated with too much uncertainty for routine commissioning. Non-CDF real-world data may have helped reduce some of this uncertainty.
- The use of RWE to form indirect treatment comparisons increased post-framework (17% versus 26%), likely reflecting an increase in the assessment of single-arm trials and trials where comparators do not reflect the NHS standard of care.
- In conclusion, while there were trends within the oncology and non-oncology disease areas, a longer timeframe may be needed to assess the true impact of the framework on RWE usage.

References

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