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

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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]
- Several agencies have provided guidance on the use of RWE including the Food and Drugs Administration (FDA), who released a framework in August 2023.[3]

OBJECTIVE

- The aim of this study was to provide an updated assessment of the impact of NICE's framework on the use of RWE to inform the clinical effectiveness of interventions assessed in the technology appraisal (TA) programme, within the first 18-months of implementation.

METHODS

- The NICE website[4] was reviewed to identify TAs published pre-guidance (01.01.21 – 23.06.22) and post-guidance (24.06.22 – 01.01.24). 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, 271 TAs were identified. Of the 271 TAs, 60 were excluded (52 terminated, 8 updates) (**Figure 1**).
- Of the remaining 211, 103 (49%) were published pre-framework and 108 (51%) post-framework.
- Pre-framework, 28/103 TAs (27%) used RWE to inform clinical effectiveness versus 33/108 (31%) post-framework.
- Of the TAs that used RWE, 87% [53/61]) were recommended by NICE.

TAs with RWE by disease category

- Oncology TAs included RWE to inform clinical effectiveness more commonly than any other disease area both pre-framework (20/28 [71%]) and post-framework (18/33 [55%]) (**Figure 2**).
- The Cancer Drugs Fund (CDF) made up almost half (47% [18/38]) of RWE used in oncology TAs.
- The use of the CDF in oncology TAs substantially decreased pre-framework (60% [12/20]) to post-framework (22% [6/18]).

TAs with inclusion by real-world study

- Across all disease categories, types of real-world data used included the CDF dataset (pre-framework 43% [12/28] versus post-framework 18% [6/33]), retrospective studies (14% [4/28] versus 30% [10/33]), other registries (11% [3/28] versus 15% [5/33]) and other observational studies (32% [9/28] versus 36% [12/33]; e.g., non-interventional and prospective studies).
- Pre- to post-framework, the proportion of retrospective studies increased, while the proportion of CDF studies decreased (**Figure 3**).

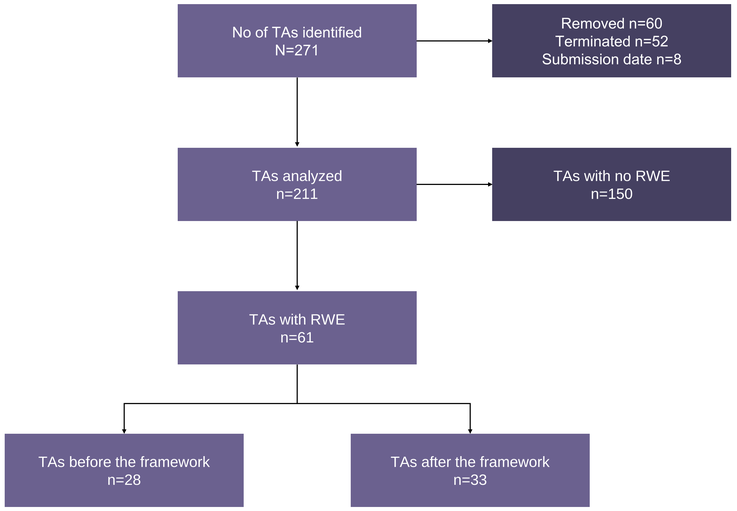
TAs with inclusion of RWE by evidence type

- The majority of TAs (64% [39/61]) used RWE as a main evidence source (*see graph for definition*), however the proportion decreased pre- to post-framework (pre-framework 71% [20/28] versus post-framework 58% [19/33] after) (**Figure 4**).

Reasons for inclusion of RWE in TAs

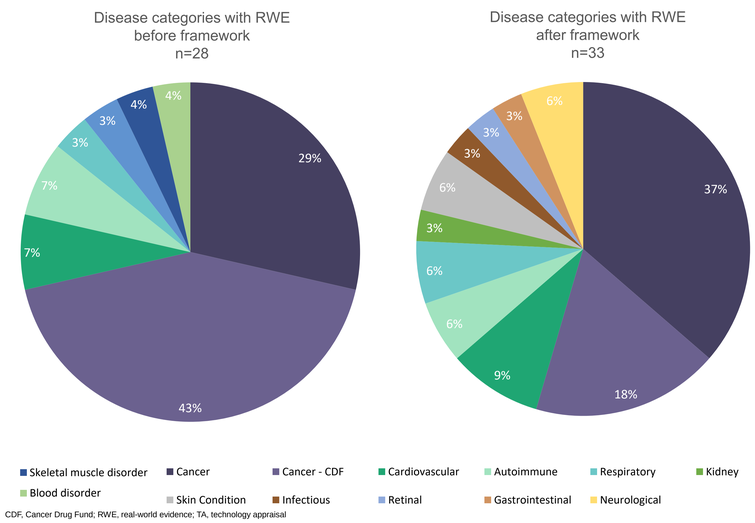
- The key reasons for the inclusion of RWE included the formation of indirect treatment comparisons (pre-framework 39% [12/31] versus post-framework 46% [16/35]) and to demonstrate generalizability of the evidence to National Health Service (NHS) clinical practice (29% [9/31] versus 31% [11/35]) (**Figure 5**).

Figure 1. Flow-diagram of TA review



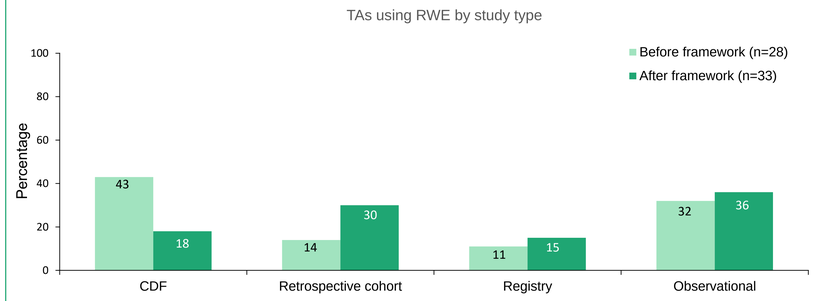
CDF, Cancer Drug Fund; RWE, real-world evidence; TA, technology appraisal

Figure 2. TAs with inclusion of RWE by TA disease category



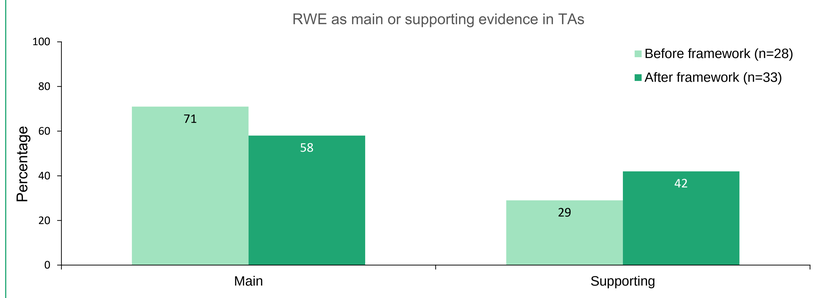
CDF, Cancer Drug Fund; RWE, real-world evidence; TA, technology appraisal

Figure 3. TAs with inclusion of RWE by real-world study type



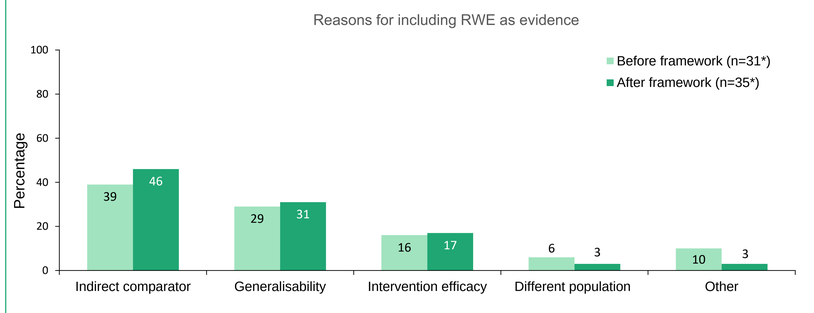
CDF, Cancer Drug Fund; RWE, real-world evidence; TA, technology appraisal

Figure 4. TAs with inclusion by RWE 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).
RWE, real-world evidence; TA, technology appraisal

Figure 5. Reasons for inclusion of RWE in TAs



*Some TAs used more than one source of RWE.
RWE, real-world evidence; TA, technology appraisal

DISCUSSION AND CONCLUSION

- Oncology TAs included RWE more than any other disease area. Challenges associated with conducting randomized controlled trials (RCTs) in rarer tumor types, regional discrepancies in the standard of care, and the availability of real-world data sources, including the CDF 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 may have been a shift in the types of real-world studies used. The use of the CDF's dataset appeared to decrease, while the use of RWE from other sources appeared to increase. 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 remained the most common use of RWE both pre- and post-framework (pre-framework 39% [12/31] versus post-framework 46% [16/35]). This likely reflects the use of ITCs to create comparisons in cases of single-arm trials and where trial comparators did not reflect the current NHS standard of care.
- In conclusion, while there appeared to be changes in the study types used, a longer timeframe may be needed to assess the true impact of the framework on the use of RWE.

References

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