From plan to action: assessing the impact of the NICE real-world evidence framework following its introduction in June 2022

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KEY LEARNINGS

NICE's Real-World Evidence Framework has increased RWE use in HTA submissions, positively influencing recommendations. However, more detail on how and where RWE was applied in NICE decision making would be helpful. Challenges in methods used and population representativeness highlight the need for further improvements to fully realise the framework's potential.

BACKGROUND

- Randomised controlled trials (RCTs) have long been considered the gold standard in clinical research. However, there is a growing recognition of the value of real-world evidence (RWE) in health technology assessments (HTAs), particularly in capturing the effectiveness of interventions in routine clinical practice.
- In response to this trend, the National Institute for Health and Care Excellence (NICE) introduced a Real-World Evidence Framework in June 2022. This framework aims to optimise the quality of RWE and address data uncertainties.
- Recent reviews of HTA submissions indicate a significant increase in the use of RWE, with 32% of submissions between 2018 and 2022 including RWE, particularly in oncology (68% of RWE submissions).[1] Post-framework (June 2022 to April 2024), RWE usage grew further, particularly for specific purposes like indirect treatment comparisons, where its application increased from 17% to 26%.[2]
- Despite these advancements, challenges remain. Issues such as population representativeness, statistical methods, and data maturity continue to pose obstacles to the effective use of RWE. Addressing these challenges will be crucial for the success of the NICE RWE Framework and the broader adoption of RWE in HTAs

OBJECTIVE

- Evaluate the impact of NICE's framework on RWE adoption in reimbursement decisions and identify key issues and opportunities associated with its implementation.

CONCLUSIONS

- The NICE RWE framework appears to demonstrate its potential to enhance reimbursement decisions and decrease uncertainty in the evidence base in both rare and non rare indications.
- RWE use in NICE submissions increased from 32% (2018-2022) to 64% (2022-2024), but only 56% of final guidance explicitly mentioned it in decision making.
- Despite ongoing challenges with data representativeness and maturity, 70% of the recommended medicines used RWE.
- Future improvements could focus on strengthening the RWE methodology and ensuring consistent integration of RWE discussions in the final guidance.

METHODS

A Targeted Review was conducted

- A review and synthesis of NICE HTA submissions from June 23, 2022, to April 25, 2024, was conducted. Extracted information included: disease area, RWE type, rationale for inclusion in the submission, primary objections from NICE, and final recommendation outcome.

RESULTS

Overview of Appraisals and RWE Use

- A total of 179 appraisals between 2022 and April 2024 (as per cut off date) were analysed, of which 38 were terminated.
- Approximately 64% (91/141) of submissions incorporated RWE during the NICE process. However, only 56% (51/91) explicitly mentioned RWE in the final NICE guidance.
- Forty-five percent (64/141) and 16% (23/141) of submissions related to oncology and autoimmune/inflammatory diseases respectively. (Fig. 2)
- Of these, 28% (39/141) and 10% (14/141) utilised RWE. (Fig. 2)
- Majority (56/91, 61.5%) of the appraisals that used RWE relied on multiple data sources
- RWE use was similar across most disease areas except blood disorders (75% rare vs. 100% non-rare), pulmonary diseases (100% rare vs. 0% non-rare), and nephrology (67% rare vs. 0% non-rare). (Fig. 4)

Impact of RWE on NICE Recommendations

- We observed a positive correlation between RWE inclusion and favourable NICE recommendations: 88% (124/141) of drugs were recommended, with 70% (83/124) utilising RWE. (Fig. 1) Additionally, five recommended drugs were part of the Cancer Drugs Fund scheme.

Reasons for Inclusion and NICE Critiques

- Main reasons for RWE inclusion from the company were generalisability to the UK population (34%, 31/91), reassurance of trial outcomes in routine practice (30%, 27/91) and to support extrapolated outcomes in economic modelling (23%, 21/91). (Fig. 3A)
- Primary objections from NICE included issues with population representativeness (20%, 18/91), concerns about statistical methods (15%, 14/91) and uncertainty due to data immaturity (12%, 11/91). (Fig. 3B)

Figure 1. Progression of drug appraisals and RWE utilisation

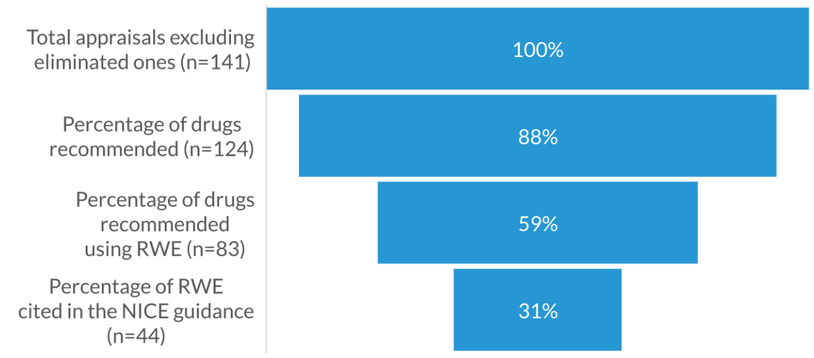


Figure 2. Percentage of appraisals and RWE usage by disease area

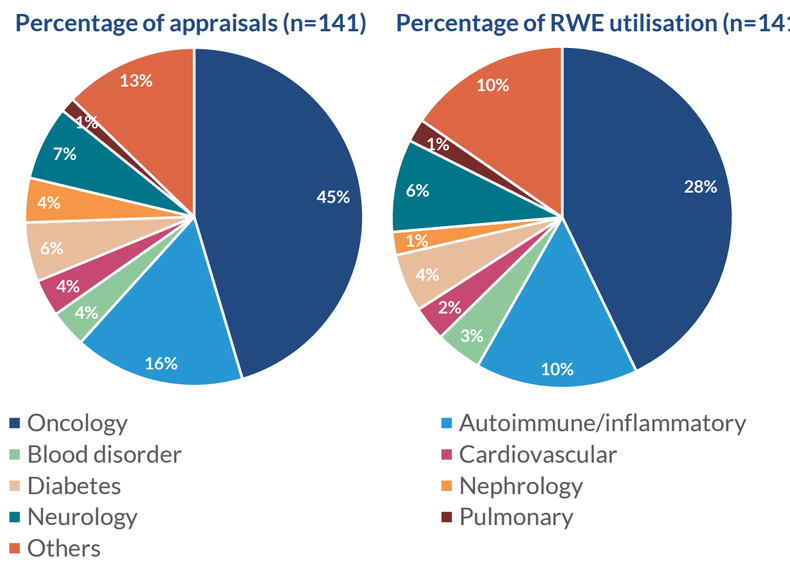
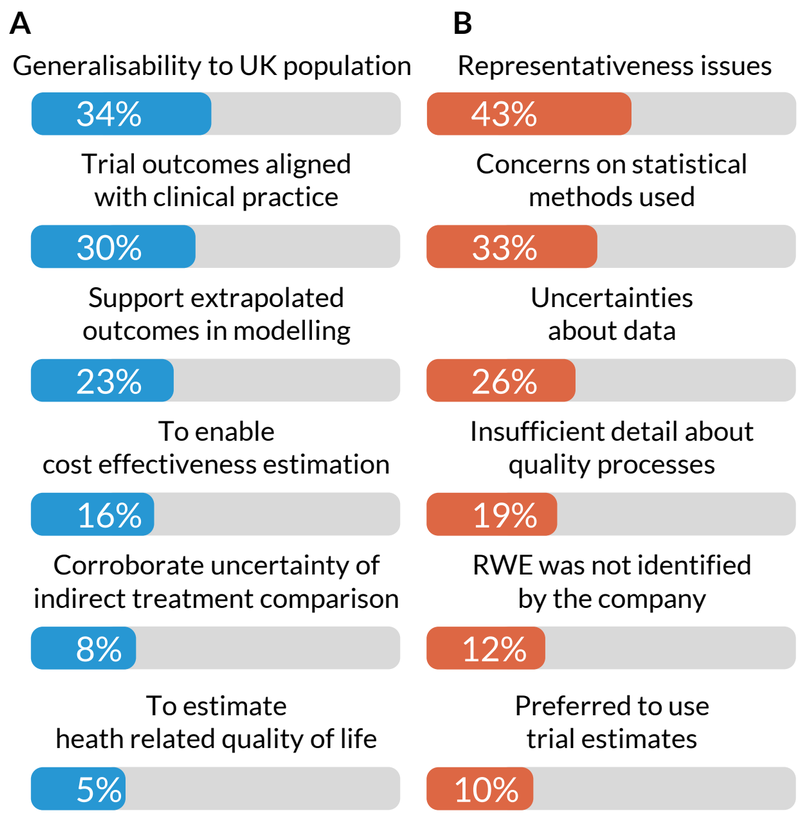
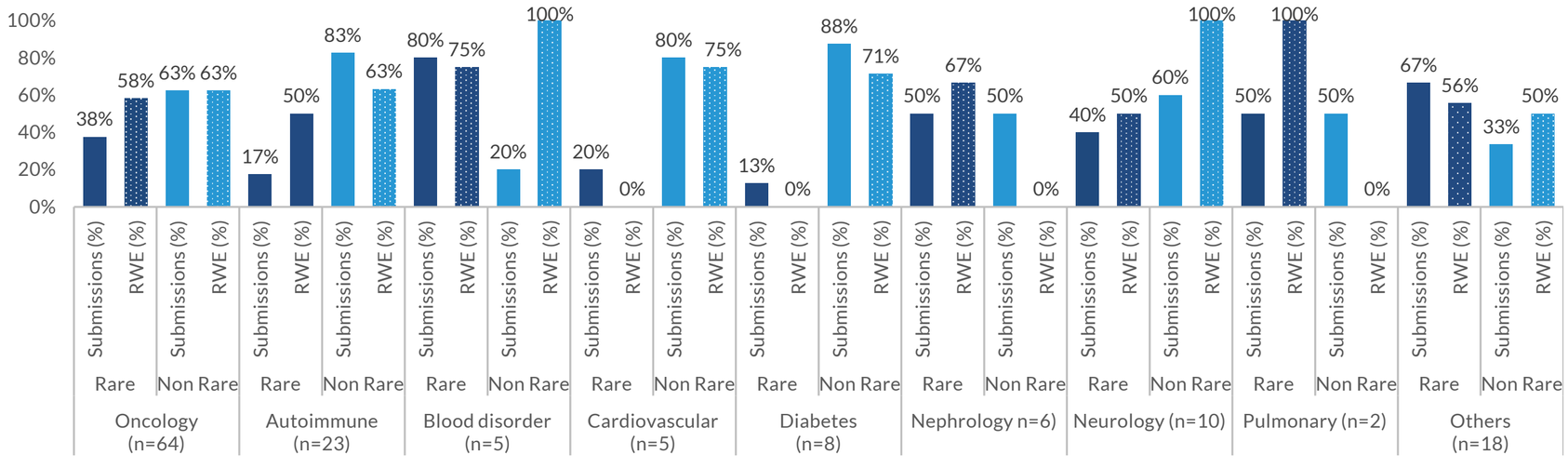


Figure 3. Reasons for RWE inclusion from company perspective (A) and critiques from NICE (B)



Footnote: Panel A illustrates the most common reasons for including real-world evidence (RWE) from the company perspective. This list is not exhaustive, as additional reasons such as dose estimation or demonstration (5%), comparative effectiveness evidence (5%), addressing unmet medical need (4%), rates of complications (1%), and baseline event rates (1%) were also reported. Percentages are calculated based on 91 appraisals where RWE was used. Panel B reflects the percentage distribution of critiques raised by NICE, based on 42 appraisals that provided specific comments.

Figure 4. RWE use by disease area in submissions: rare vs. non-rare conditions



Footnote: Dark blue bars represent rare disease studies, and light blue bars represent non-rare disease studies. The solid bars indicate the percentage of total studies in each category (rare or non-rare), while the dotted bars represent the percentage of those studies (within the solid bars) that use RWE. Each disease area shows the total number of studies in parentheses.

Abbreviations RCT: Randomised Controlled Trials; RWE: Real-World Evidence; HTA: Health Technology Assessment; NICE: National Institute for Health and Care Excellence.

References
1. Liu H. Value Health 2022;25
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