The use of real-world evidence in health technology assessment and the application of the NICE RWE framework: A review of NICE technology appraisals

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Introduction

Real-world evidence (RWE), derived from real-world data (RWD), such as electronic health records, claims data, patient registries, and observational studies, is increasingly used to support health technology assessments (HTAs) and inform healthcare decision-making. RWE provides valuable insights that can complement and strengthen traditional HTA methodologies (1,2). Previous reports have suggested that RWE may increase the likelihood of a technology receiving a positive recommendation from the National Institute for Health and Care Excellence (NICE) (3). However, its wider use and integration remain limited, which, we hypothesise, could be due to inadequate reporting, insufficient assessment of data quality, and the use of inappropriate analytical approaches.

The NICE RWE framework was published in June 2022 to provide guidance on when and how to incorporate RWD into technology appraisals (TAs), as well as best practices for generating and reporting RWE (4). Yet, the extent to which the framework has been applied in recent appraisals remains unclear. Examining whether and how the NICE RWE framework is being used, and exploring current patterns of RWE use in HTA, can together inform recommendations for more effective use of RWE.

Objectives

The objectives of the review were to:

1. **Assess trends in the use of RWE and its impact on recommendations**
2. **Examine NICE RWE framework adoption**
3. **Characterise the types, sources, and relevance of RWE**

Methodology

The NICE website was screened to identify TAs completed since the publication of the NICE RWE framework (June 2022 to May 2025). Appraisal documents were reviewed to determine whether RWE was used or referenced, and whether the NICE RWE framework or associated quality assessment tools were used or referred to (4).

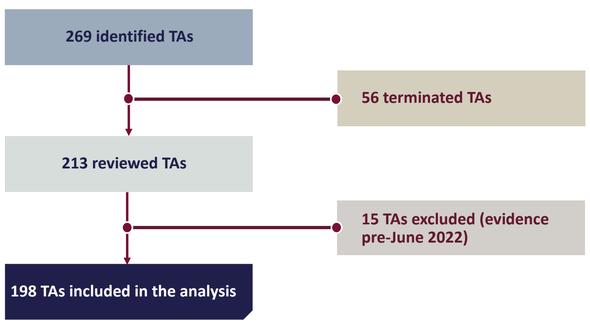
Information was then extracted on the technology, therapeutic area, RWE categories, data sources, and the NICE recommendation. All information was tabulated into an Excel® spreadsheet and analysed using a narrative approach.

RWE use was categorised into pre-defined groups to facilitate comparison across studies. Where two or more categories (e.g. types or sources of RWE) were reported within the same study, these were classified as “multiple” categories or sources in the analysis.

Results

A total of 269 appraisals were identified, of which 213 were reviewed following exclusion of terminated TAs. A further 15 were excluded because the evidence review and evaluation occurred before June 2022 (prior to when the NICE RWE framework was published) (Figure 1).

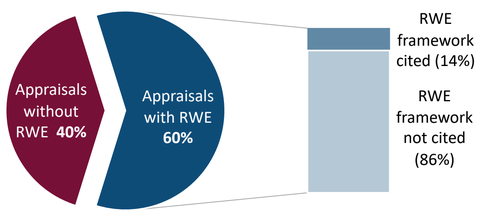
Figure 1: Flowchart of TA selection



RWE use and NICE RWE framework citation

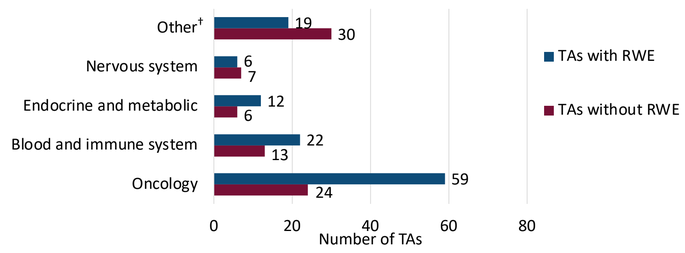
RWE was used in 60% of the reviewed appraisals, with the NICE RWE framework cited in 14% (Figure 2). Best practice tools for assessing data suitability, quality, applicability, and risk of bias were identified in only 4 appraisals (3 used the Data Suitability Assessment Tool [DataSAT] and 1 assessed risk of bias), while none reported using NICE’s preferred approaches for planning, conducting, and reporting RWE (data not shown). This indicates limited integration of such tools/guidance within HTA submissions using RWE.

Figure 2: Appraisals using RWE and NICE RWE framework citation



As shown in Figure 3, RWE was most frequently applied within oncology, blood and immune, endocrine and metabolic, and nervous system diseases.

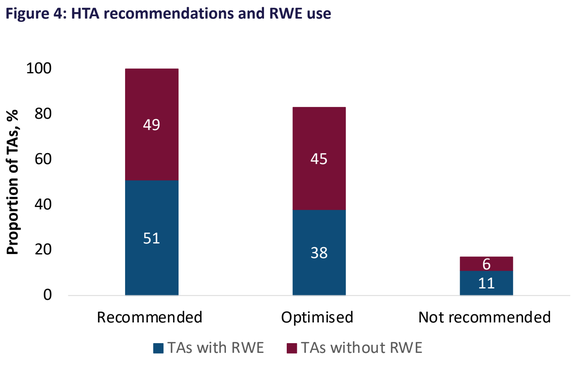
Figure 3: RWE use by therapeutic area



† Includes digestive system, circulatory system, infectious diseases, musculoskeletal system and connective tissue, genitourinary system, skin and subcutaneous tissue, eye, respiratory system, mental and behavioural, and ear and mastoid process diseases.

Figure 4 shows that appraisal outcomes were broadly similar regardless of RWE use (51% recommended with RWE versus 49% without), though optimised outcomes were more frequent without RWE, likely due to other factors and variation in appraisal contexts.

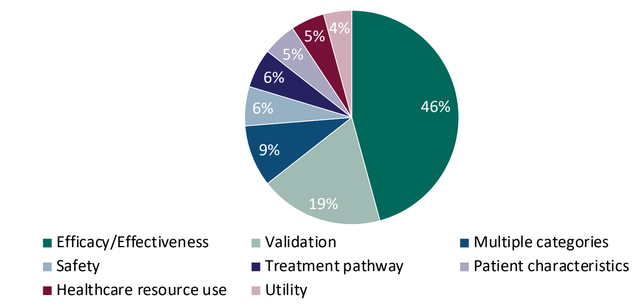
Figure 4: HTA recommendations and RWE use



Type of RWE use

In the reviewed appraisals, RWE was frequently employed to inform estimates of efficacy/effectiveness (46%), safety (6%), treatment pathway (6%), patient characteristics (5%), healthcare resource use (5%), and utility values (4%). In 19%, RWE was used to validate submission data by assessing its consistency with real-world practice, while 9% of appraisals used RWE to inform multiple categories (Figure 5).

Figure 5: TAs by type of RWE use



RWD source

A variety of sources were used to generate RWD in the reviewed appraisals. The most common source was published prospective or retrospective studies, some of which were based on databases such as national registries or hospital records. These were followed by data from the National Cancer Registration and Analysis Service (NCRAS), Flatiron data, and national or multi-national disease registries, which offer broader population-level insights. The evidence originated from national, international, or mixed sources, with most international data coming from the US and Europe. International data were typically used when national data were unavailable. Table 1 presents additional details on the source categories and their relative contributions.

Table 1: Technology appraisals by RWE source

Type of source	Number of TAs	Percentage of total TAs with RWE
Other studies†	56	47
NCRAS‡	20	17
Multiple sources	10	8
Flatiron data	6	5
Other sources§	6	5
National disease registry¶	4	3
Multi-national disease registry††	4	3
SEER	4	3
CPRD	3	3
Early access/patient support schemes‡‡	2	2
Regional disease registry (HMRN)	2	2
US Oncology Network	1	1

† Includes retrospective/prospective studies; Adelphi; Pilot studies.
‡ Includes SACT; NHS Digital; HES; CDF SACT data; NHS England CDF.
§ Includes market access data; RWE service provider or company data/survey; Hospital reports;
Other claims data; UK mRCC.
¶ Includes ITP Registry; BADBIR; Pompe registry; GEMFIN; RaDaR; Prostate Cancer Registry; SSNAP.
†† Includes ECHO-EU; SHaRe.
‡‡ Includes patient support programmes; Early access schemes.

Conclusion

RWE is often used in NICE TAs to inform efficacy/effectiveness, safety, treatment pathways, patient characteristics, healthcare resource use, and utility values. It is typically applied either as primary evidence, e.g. external controls in indirect comparisons, or to support data validation from multiple sources. Despite RWE’s use in HTA, the NICE RWE framework and best practice tools were rarely cited, highlighting the need for greater methodological rigour and adherence to reporting standards to ensure transparency and reliability. Systematic use of frameworks and standards could strengthen the credibility of evidence and support appraisal decisions.

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Abbreviations

BADBIR, British Association of Dermatologists Biologic and Immunomodulators Register

CDF, Cancer Drugs Fund

CPRD, Clinical Practice Research Datalink

DataSAT, Data Suitability Assessment Tool

ECHO-EU, Endometrial Cancer Health Outcomes – Europe

GEMFIN, Grupo Español de Enfermedades Mieloproliferativas Crónicas Filadelfia Negativas

HES, Hospital Episode Statistics

HMRN, Haematological Malignancy Research Network

HTA, health technology assessment

ITP, immune thrombocytopenia

mRCC, metastatic renal cell carcinoma

NCRAS, National Cancer Registration and Analysis Service

NHS, National Health Service

NICE, National Institute for Health and Care Excellence

RaDaR, Registry of Rare Kidney Diseases

RWD, real-world data

RWE, real-world evidence

SACT, systemic anti-cancer therapy

SEER, Surveillance, Epidemiology, and End Results

SHaRe, Sarcomeric Human Cardiomyopathy Registry

SSNAP, Sentinel Stroke National Audit Programme

TA, technology appraisal