HTA124

Real world data to inform health technology assessments in England: a review of submissions to the National Institute for Health and Care Excellence since 2018

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INTRODUCTION

- The National Institute for Health and Care Excellence (NICE) in England develops recommendations for new and existing medicines and treatments based on evidence submitted across various categories including the Health Technology Appraisal (HTA) programme.
- Randomised controlled trials (RCTs) have historically been considered the 'gold-standard' evidence source for NICE HTA appraisals.
- However, real world data (RWD) generated from non-interventional studies are increasingly being used alongside evidence from RCTs to inform funding and licensing decision-making for new technologies.[1]
- Prior to the publication of the NICE Real World Evidence Framework in June 2022,[2] there was no standardised guidance available to support the planning, implementation and reporting of real-world studies for HTA submissions in England.

OBJECTIVES

- This review aimed to describe the utilisation of RWD in NICE HTA submissions in England since 2018.

METHODS

- A structured review of all NICE HTA submissions and committee appraisals between 01 January 2018 and 17 March 2022 (data cut-off date) was conducted in May 2022.
- Relevant information, including a description of study characteristics (i.e. therapeutic area, study design and data source), committee comments relating to RWD and recommendations made, was extracted and recorded in an electronic database.

RESULTS

- A total of 219 HTA submissions were appraised by NICE between 2018 and 2022 (as of data cut-off date) (Table 1).
- Of these, 32% (69/219) included RWD derived from an observational/ non-interventional study.

Table 1. Overview of HTA appraisals published since 2018

Year	Total HTA submissions initiated	Total submissions appraised by NICE committee	Total submissions that included RWD
2018/19	68	61	18
2019/20	54	44	16
2020/21	72	59	10
2021/22	68	55	25
TOTAL	262	219	69

Disease/therapeutic areas

- The most common disease/therapeutic area for submissions including RWD was oncology (68% [47/69]; Table 2).
- Of the appraisals containing RWD, 7% (5/69) were for a rare disease.

Table 2. Overview of disease/therapeutic areas of submissions including RWD

Disease/ therapeutic area	n	% (n=69)
Oncology	47	68%
Blood disorder	6	9%
Central nervous system/neurology	5	7%
Autoimmune/inflammatory disease	4	6%
Diabetes	1	1%
Nephrology	1	1%
Respiratory	1	1%
Cardiovascular	1	1%
Other	3	4%

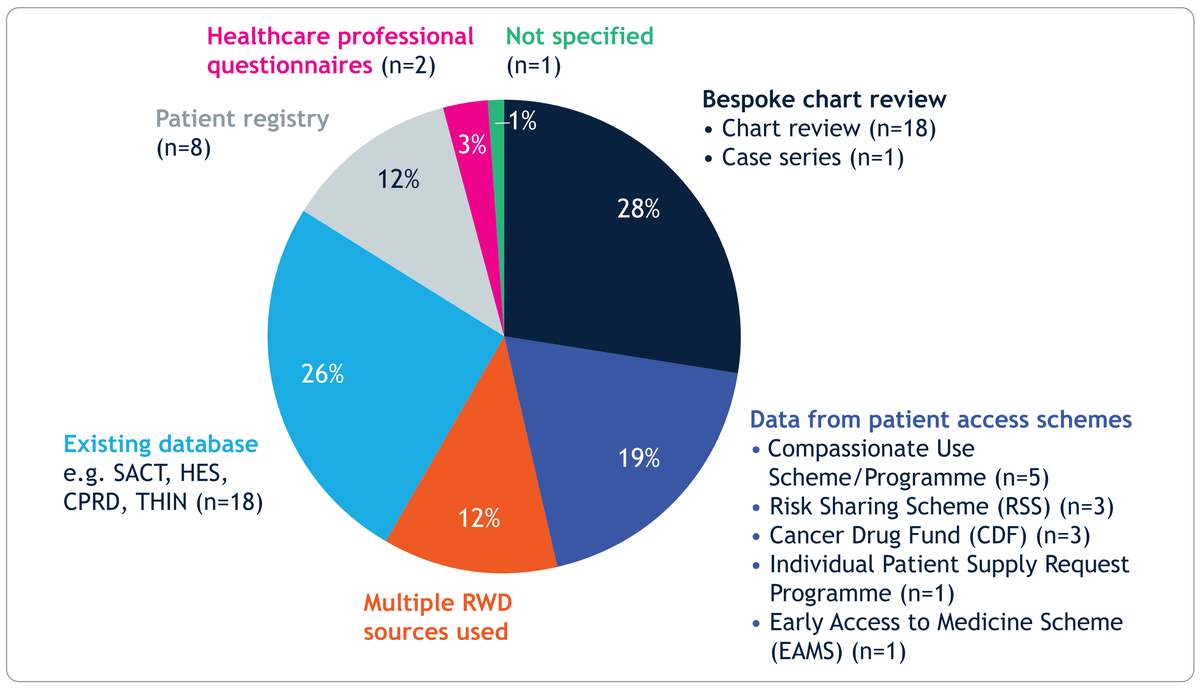
Study design

- Of the studies including RWD, 46% (32/69) were retrospective studies, 36% (25/69) were prospective studies and 12% (8/69) incorporated a mixed design, while 6% (4/69) had unknown study design.

Data sources

- Sources used to generate RWD included bespoke chart reviews (28% [19/69]), existing databases (including Hospital Episode Statistics [HES], Clinical Practice Research Datalink [CPRD], Systemic Anti-Cancer Therapy Dataset [SACT], the Health Improvement Network [THIN] (26% [18/69]), patient access schemes (19% [13/69]) and registries/other (16% [11/69])), while 12% (8/69) of submissions included RWD from multiple sources (Figure 1).

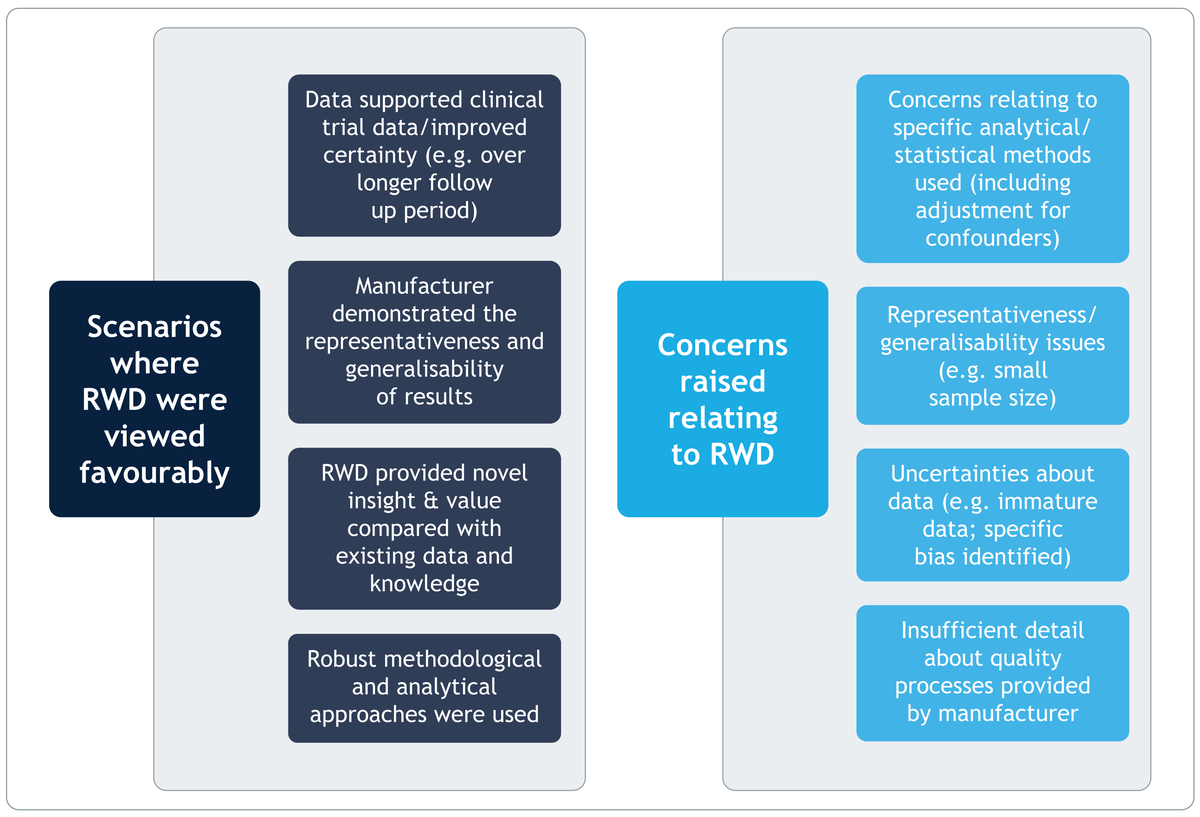
Figure 1. Data sources used to generate RWD



Committee appraisals

- The specific comments and degree of rigour applied when appraising RWD varied between submissions, with the committee specifically appraising the quality and robustness of RWD in 48% (33/69) of RWD submissions. Key considerations included the rationale provided, the generalisability & representativeness of results, the analytical methodology used (e.g. confounder adjustment), and wider study design including sample size and follow-up duration (Figure 2).

Figure 2. Overview of committee comments in relation to RWD



- Scientific rationale justifying the inclusion of RWD was provided by the manufacturer in the majority of submissions (84% [58/69]).
- The NICE committee specifically recommended that further RWD were collected for 13% (n=29) of the total 219 submissions in order to address identified uncertainties and data gaps (19 of 29 had not yet submitted any RWD).

CONCLUSIONS

- These findings highlight the increasing importance of RWD for technology appraisals, with approximately one-third of submissions comprising RWD since 2018.
- This represents a considerable increase compared with our previous review of HTA submissions in 2015-16 whereby 10% of NICE HTA submissions contained RWD.[1]
- These findings also support the requirement for standardised guidance, such as the recently published NICE Real World Evidence Framework,[2] to inform best practices for the collection and reporting of RWD for HTA submissions.

REFERENCES

1. Glen, F. & Pulfer, A. (2017). The use of real world data for NICE decision-making: A review of submissions to three treatment effectiveness evaluation programmes in 2015 and 2016. *Value in Health*, 20(9), A693.
2. National Institute for Health and Care Excellence (2022). NICE real-world evidence framework. Available at: www.nice.org.uk/corporate/ecd9/chapter/overview (last accessed October 2022).

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DISCLOSURES

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