HTA29

The Use of Real-World Evidence in NICE Health Technology Appraisals: A Quantitative Review of Submissions to NICE Between 2022 to 2024

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BACKGROUND

Real world evidence (RWE) consists of data that is typically not collected in randomised clinical trials (RCTs)[1]. This could be from disease registries, wearables and electronic health records[1]. Such data can be collected through prospective and retrospective studies or through post-marketing surveillance of therapies in routine healthcare delivery[2]. RWE can be collected at varying stages of the drug development lifecycle and has been used to inform or optimise the design of future RCTs[2]. In health technology appraisals (HTAs), RWE has typically been utilised as supplementary data to address evidence gaps and help inform the safety and efficacy of a new therapy. In recent years, the use of RWE in technology appraisals has steadily increased. This is evidenced by agencies such as NICE developing frameworks to support the generation of real-world data that is fit for purpose by manufacturers[2]. With this growing interest in and acceptance of RWE by NICE, it is important to understand the contemporary use of and landscape of RWE. Through this quantitative review, we describe the use of RWE in NICE submissions between 2022 to 2024.

METHODS

A review of technology appraisals (TAs); single TA (STA) and highly specialised technologies (HST) published by NICE between January 2022 to April 2024 was conducted. NICE technology appraisals assess the clinical and cost-effectiveness of a new medicines (technology). Manufacturer submissions were collated after filtering for STA and HST submissions on the NICE website. These candidates were subsequently screened and submissions that incorporated primary research based on RWE included (inclusion criterion).

From the resulting filtered list, the following data were captured: the section of the appraisal RWE data was used; the reason for use; therapeutic area; country of origin of the originating data; source; indication, technology and outcome.

RESULTS

In total, 180 submissions were identified, 58 (25%) met the inclusion criteria, 46/58 (79%) STAs and 12/58 (21%) HSTs which is captured in Figure 1. These covered 56 unique or unique combinations of technologies. 75 unique RWE projects informed the 58 appraisals, an average of 1.3 RWE projects per submission.

Figure 1: Results of the search for NICE TAs

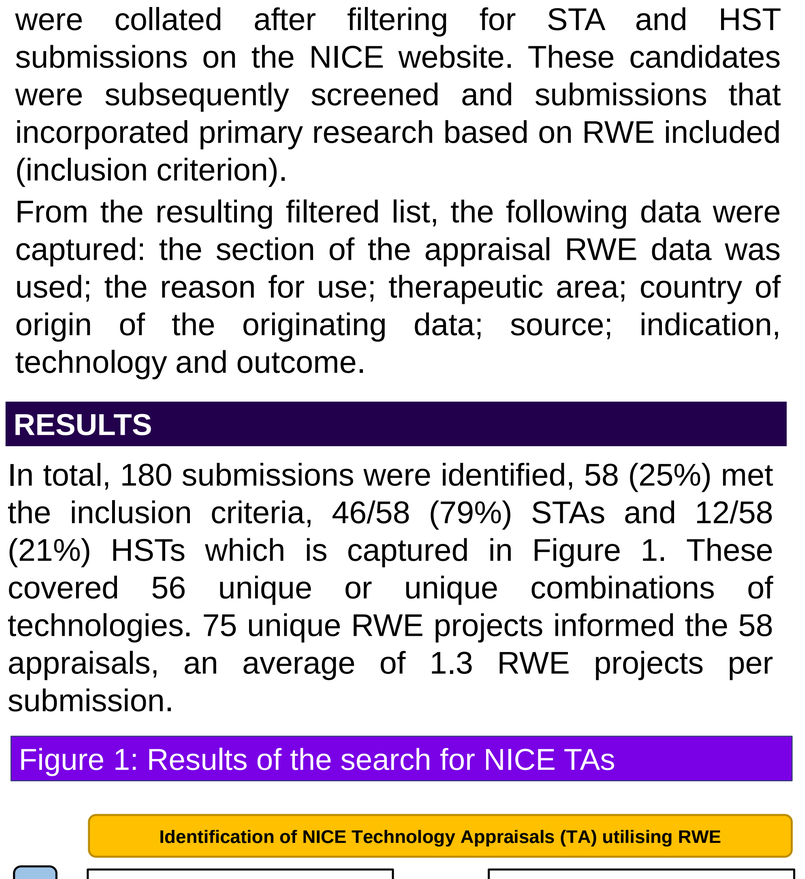

Figure 1 – A PRISMA flow diagram outlining the systematic review to obtain the NICE TAs for review[3].

Figure 2: Reasons for use in technology appraisals

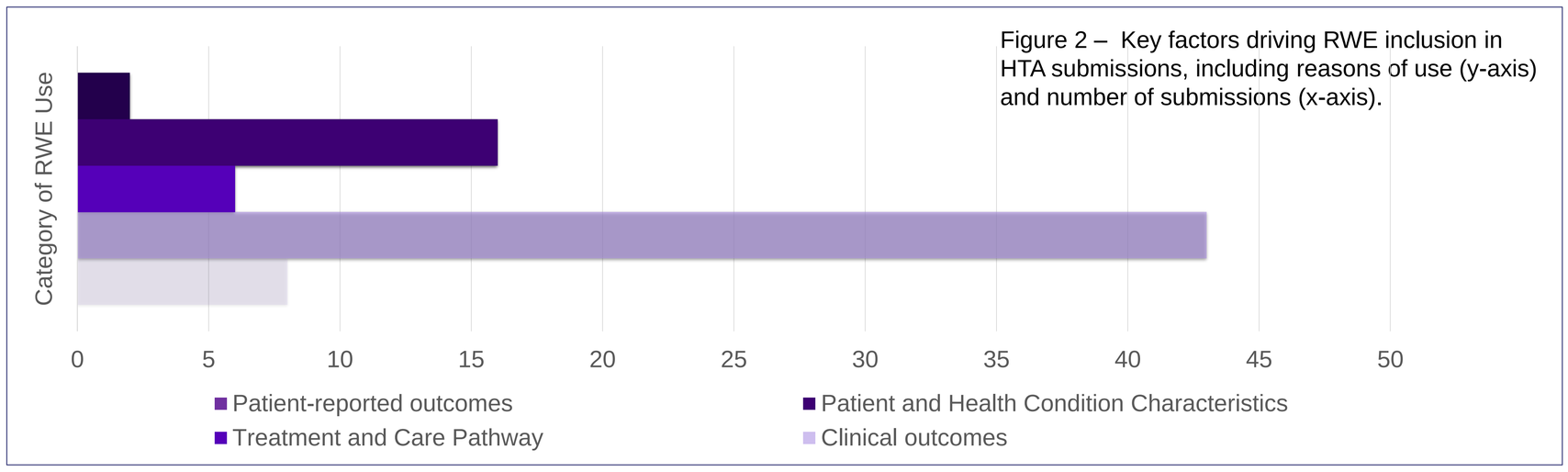

Figure 2 – Key factors driving RWE inclusion in HTA submissions, including reasons of use (y-axis) and number of submissions (x-axis).

Figure 3: Geographical distribution of real-world data provided in TAs

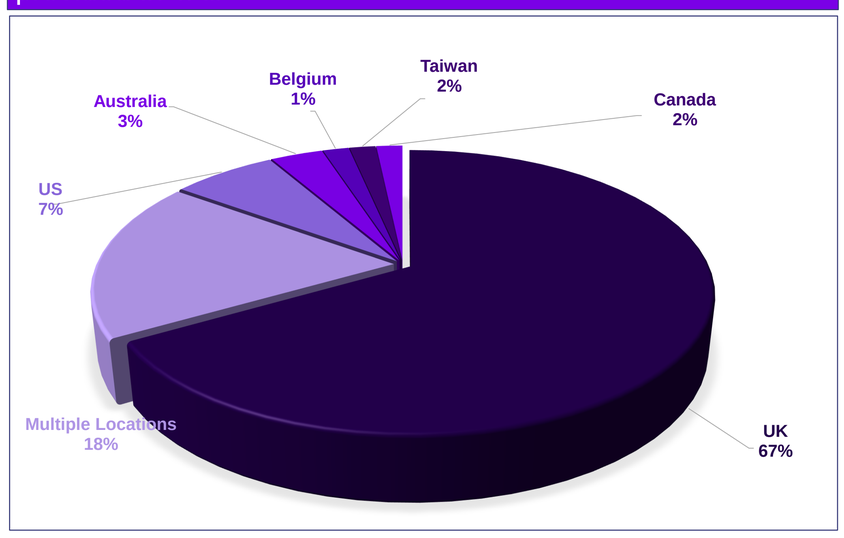

Figure 3 – Chart showing the location of RWE data. The percentage indicates the proportion.

Figure 4: Section of TA RWE used to support

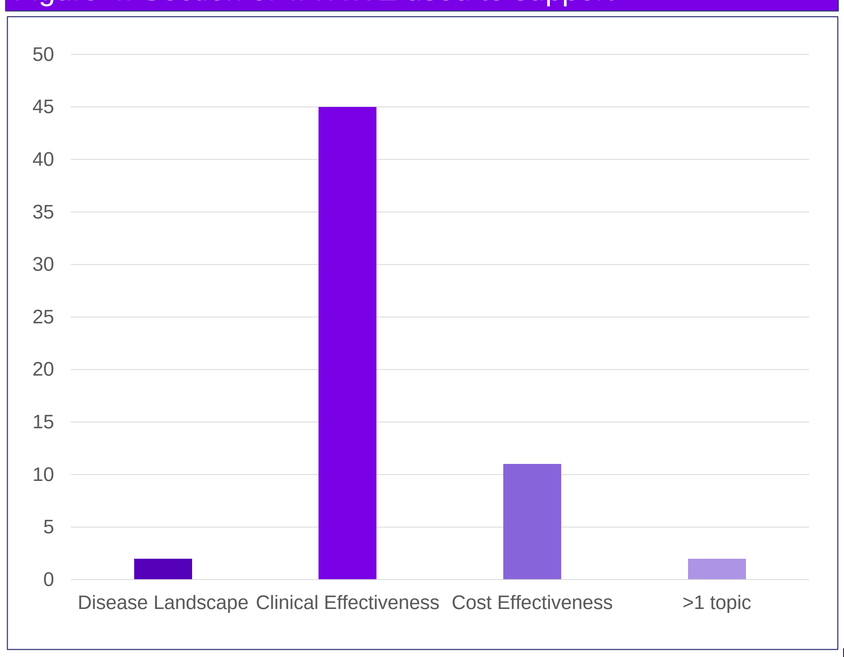

Figure 4 – Illustrates the frequency of RWE application across various sections of HTA reports. The y axis represents the number of times RWE was used in that section. The x axis represents the section of the NICE appraisal.

Figure 5: Disease areas use RWE by a company.

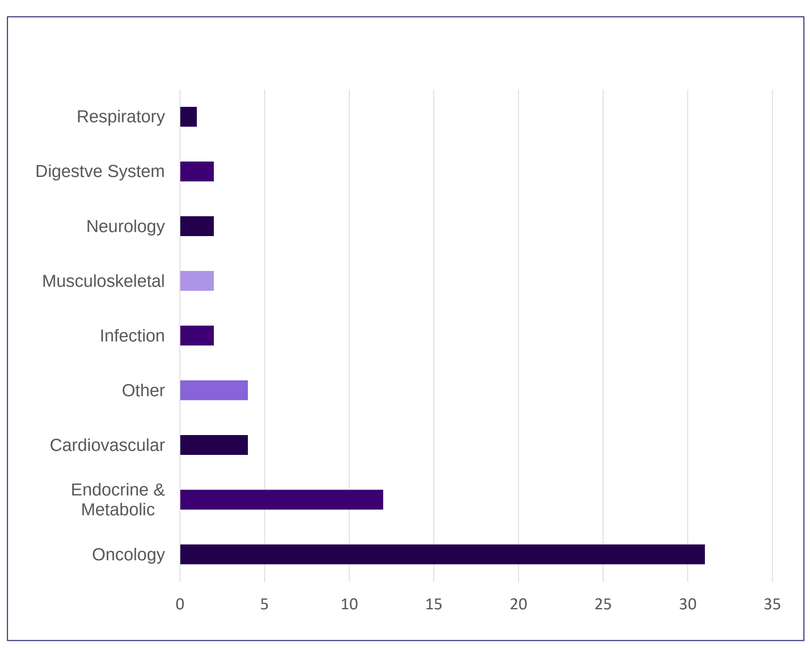

Figure 5 – The number of therapy areas that the TA using RWE were involved in. The x axis represents the number of TAs, and the y axis represents the therapy area.

DISCUSSION

- Of of the 58 TAs that leveraged primary RWE research, RWD were mainly used to inform the clinical effectiveness (78%), cost effectiveness (12%) and disease landscape (5%) sections of the submissions.
- Of the submissions that included RWE, 45% were recommended, 40% were optimised, 13% were not recommended and 5% went through the Cancer Drug Fund as the result.
- It was not surprising that oncology was the most represented therapeutic area, 31/58 (53.4%), Figure 3. The proportion of oncology submissions that leveraged primary RWE research was broadly consistent with that of oncology studies overall.
- Uses of RWE projects primarily included description of; clinical outcomes (n=43), patient and health condition characterisation (n=16), resource utilisation and disease burden (n=8), treatment and care pathway (n=6) and patient-reported outcomes (n=2) as shown in Figure 4.
- The underlying data source for the 75 projects geographically originated from: UK-only (46/75[61.3%]), multiple locations (15/75[20%]), USA-only (7/75[9.3%]), Australia-only (2/75[2.7%]) and 1/75(1.3%) for each of Belgium, Canda, France, Israel, and Taiwan as shown in Figure 5.
- It is important to note that this study specifically included only instances where the developer included, in their submission, data from an RWE project that the developer initiated themselves, and not where they may have leveraged a previously published RWE data. As a result, it is expected that the proportion of projects using RWE reported here may be less other published data that have sought to address similar topic.

CONCLUSIONS

Given the remit of NICE, it is not surprising the majority of RWE was to support evidence on outcomes. Although the introduction of RWE Framework may not have led to a substantial quantitative change in TAs supported by RWE, it is likely its short-term impact will be qualitative, thereby improving its likelihood to impact decision making. With almost 40% of the projects using real-world data from outside the UK, there is an obvious need for frameworks to support consistent approaches to data transportability.

REFERENCES

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DISCLOSURES

OD, HT are employees of Sanofi and may hold stock options in Sanofi.
AA and RA were employed by Sanofi at the time the work was carried out.