The Increasing Use of Real-World Data in Health Technology Appraisals of Oncology Therapies: A Systematic Review

EPH82

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Introduction

- The therapeutic appraisal (TA) process is used by the National Institute for Health and Care Excellence (NICE) to recommend new and existing treatments within the UK National Health Service (NHS)1.
- TAs predominantly reference evidence for randomised control trials.
- In 2022 NICE published its Real-World Evidence (RWE) Framework2, providing best practices around developing RWE to support regulatory decisions.
- The value of RWE is highlighted in the field of oncology, where ethical and/or practical considerations can prohibit the execution of randomised-control studies.

Methods

How the systematic literature review was undertaken:

- A systematic literature review was undertaken into all active Oncology TAs between financial year 2000/01 – 2022/23. TAs were extracted on 23rd August 2023.
- Dual review was undertaken based on a set of questions and a standardised report form was completed for the review of each TA.
- The review was undertaken independently by each reviewer and any disagreements were resolved through discussion and joint review.
- A third reviewer confirmed any complex statistical methods.
- Case studies of TAs with common concerns were created to highlight specific concerns on the use of RWD by the committee or evidence advisory group.

Questions addressed by the systematic literature review:

- How many oncology TAs used RWD in their submission?
- Which specific RWD datasets are used in oncology TAs?
- What methods are used for RWD in oncology TAs?
- How complex are the methods used in oncology TAs?
- What are the key concerns of the committee in oncology TAs?

Results – Use of RWD

- Of 501 Oncology TAs, 279 were included in the study (Figure 1).
- Of these, **135 (48.4%) used some form of RWD**.

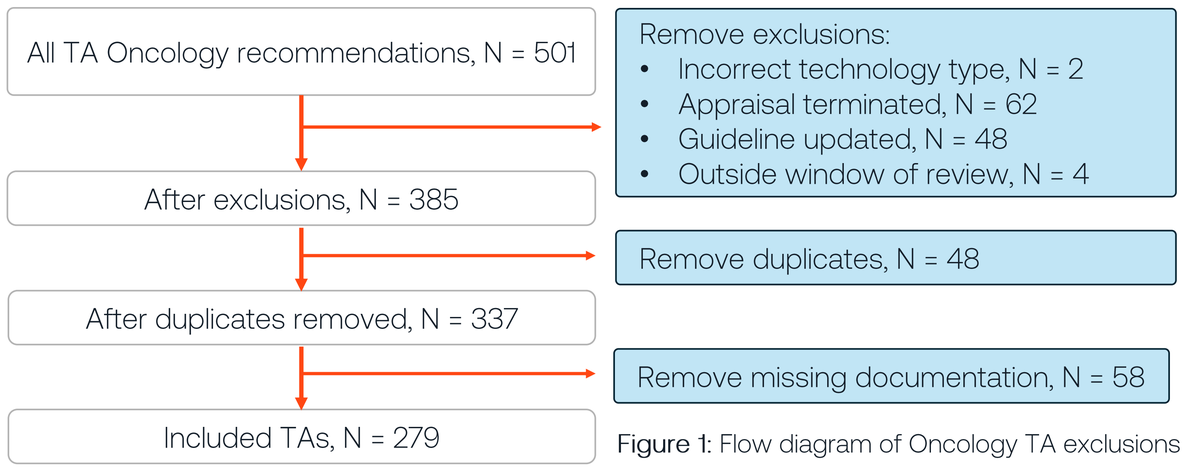
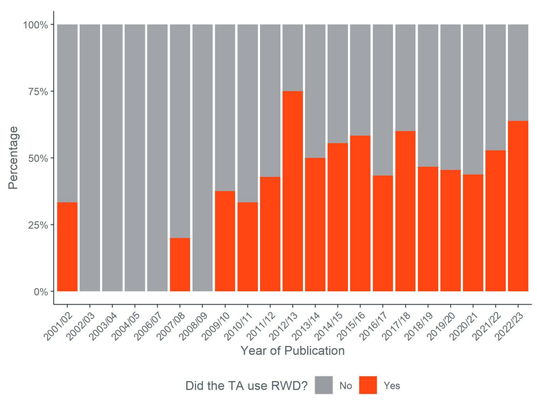


Figure 1: Flow diagram of Oncology TA exclusions

- 84 (62.2%) TAs with RWD were *"Recommended"* and 34 (25.2%) "*Optimised*".
- The number of TAs using RWD has increased over time (Figure 2).
- One study used RWD as early as 2001/02 and consistent use of RWD did not begin until 2009/2010..



- The number of studies using RWD increased from 3 (37.5%) in 2009/10 to 23 (63.9%) in 2022/23

Conclusions

- RWD is becoming increasingly used in the submission of Oncology TAs in the UK.
- There has been a recent increased use of RWD and improved methods.
- However, common issues remain such as missing variables and lack of generalisability limiting the effectiveness of RWD in TA submissions.
- These concerns and increased use has highlighted the importance and need for detailed UK patient level datasets to support future TA submissions.

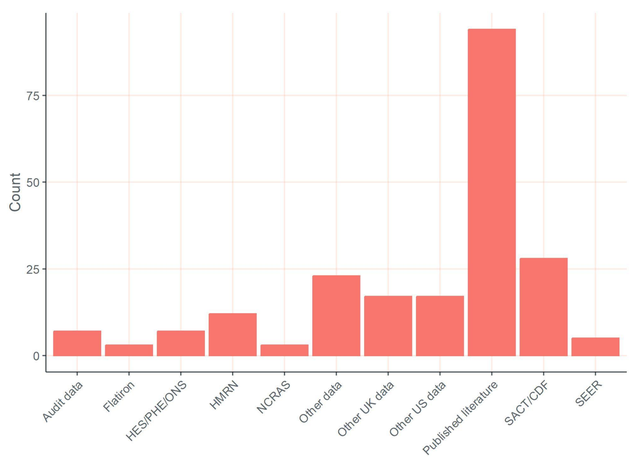
Acknowledgments and References

1. http://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance
2. https://www.nice.org.uk/corporate/ecd9/chapter/overview

Objectives

- To summarise the use of real-world data (RWD) in oncology TAs between 2000/01 and 2022/23
- To describe the types of datasets and types of methods used in oncology TAs over time
- To describe key concerns of the committee in oncology TAs both generally and with regard to the RWD.

Results – Sources & Methods



- Data sources predominantly originated from the UK, US and published literature (Figure 3).
- Published literature generally contained utility values and survival curves.
- Published literature was used 94 times.
- Multiple RWD sources were used in 47 (34.8%) TAs with 1 TA using 8 different data sources.

- Methods used have changed over time with utility values and survival analysis historically the most used.
- Since 2015 and 2018 respectively, matching adjusted indirect comparator (MAIC) and external control arm (ECA) analyses have been used in TA submissions (Figure 4).
- In 2022/23 MAICs were used in 5 (13.9%) TAs whilst ECAs were used in 2 TAs (5.6%) having increased in use over time.

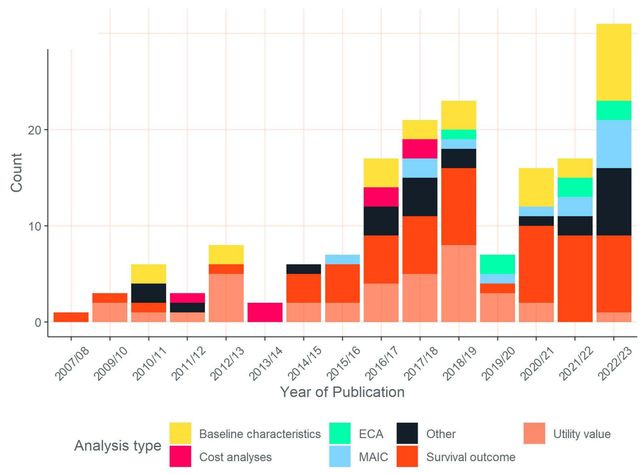


Figure 4: Analyses used in Oncology TAs stratified by financial year of publication.

Figure 2: The distribution of Oncology TAs using RWD stratified by financial year of publication. Note: No Oncology TAs were included in financial year 2005/06

Case Study 1

TA653 - *Osimertinib for treating EGFR T790M mutation positive advanced non-small-cell lung cancer*

Key details:

- Decision: Approved
- Cost per QALY: <£30,000
- RWD used: Systemic Anti-Cancer Therapies (SACT) dataset
- Statistical method used: Baseline characteristics, Survival outcome

Key Concerns:

- Areas of uncertainty, particularly difference in median overall survival, potential reasons include:
  1. comorbidities are unavailable in SACT
  2. misalignment and missingness in the performance status
  3. key data items such as frequency of cerebral metastases were not available in SACT data

Case Study 1

TA692 Pembrolizumab for treating locally advanced or metastatic urothelial carcinoma after platinum-containing chemotherapy

Key details:

- Decision: Not recommended
- Cost per QALY: <£50,000
- RWD intended for use: SACT Note data not used in committee review
- Statistical method used: Baseline characteristics, Survival outcome

Key Concerns:

- Lack of comparison between
- pembrolizumab and best supportive care
- Inappropriate statistical modelling
- Lack of generalisability to the UK population

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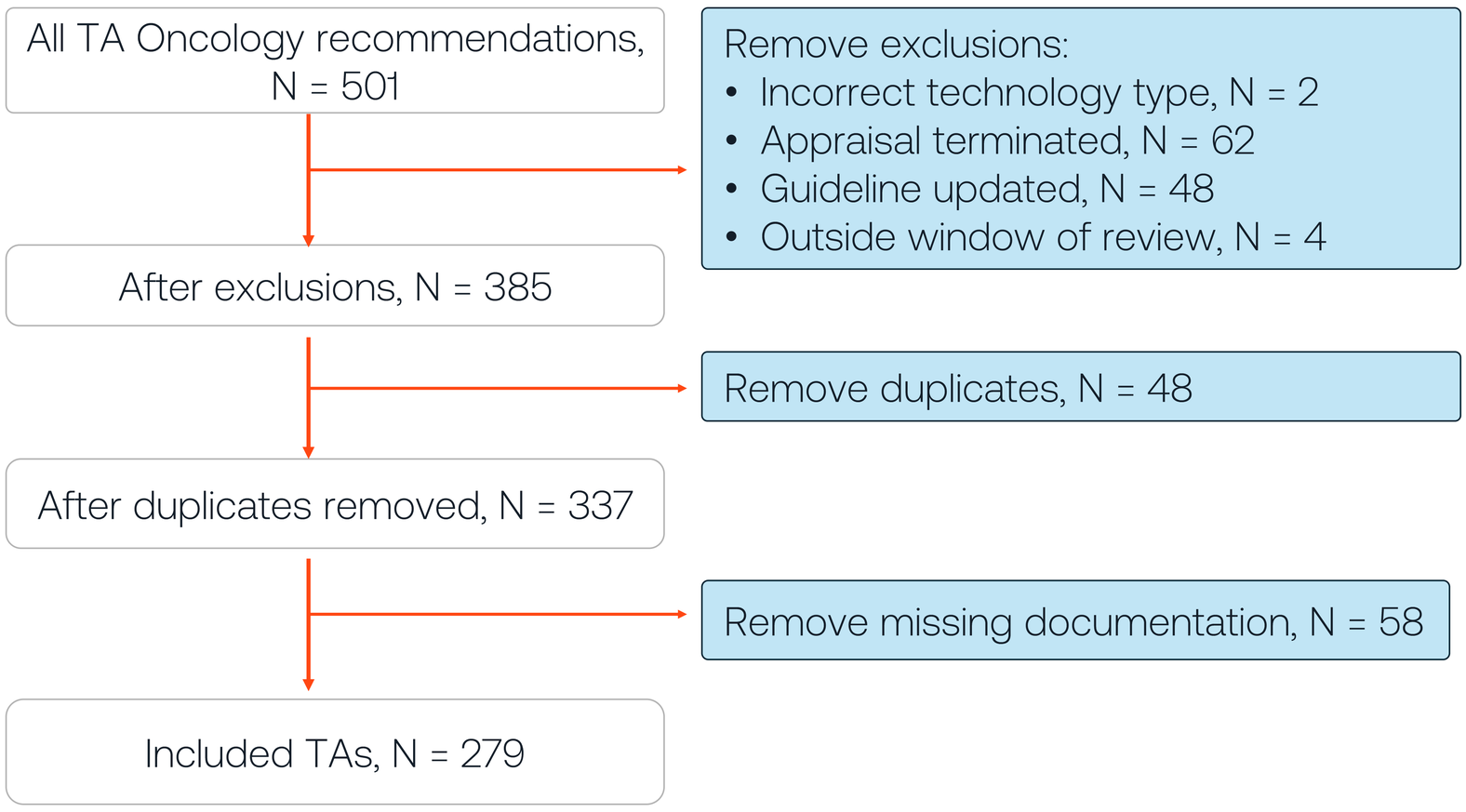

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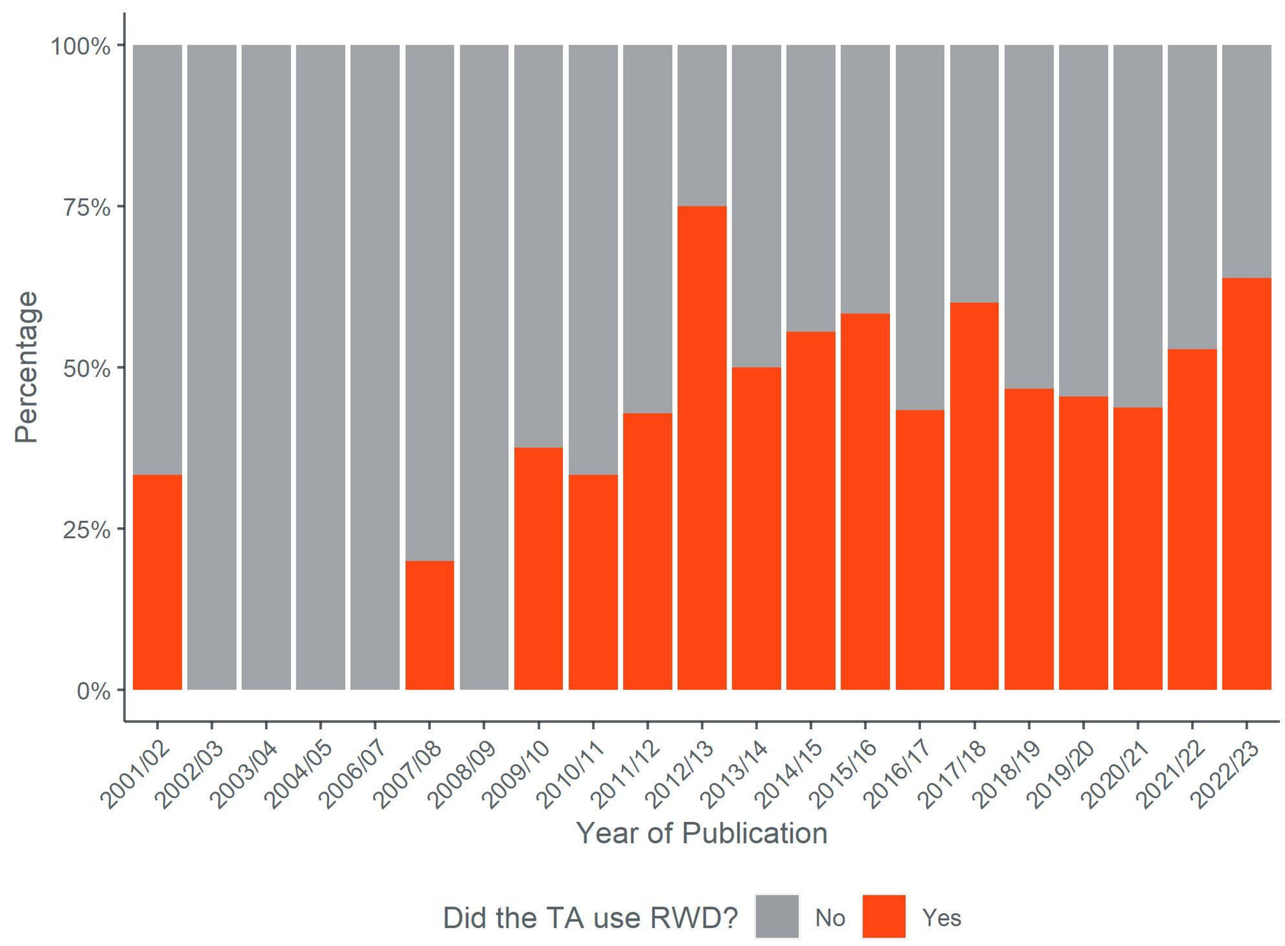

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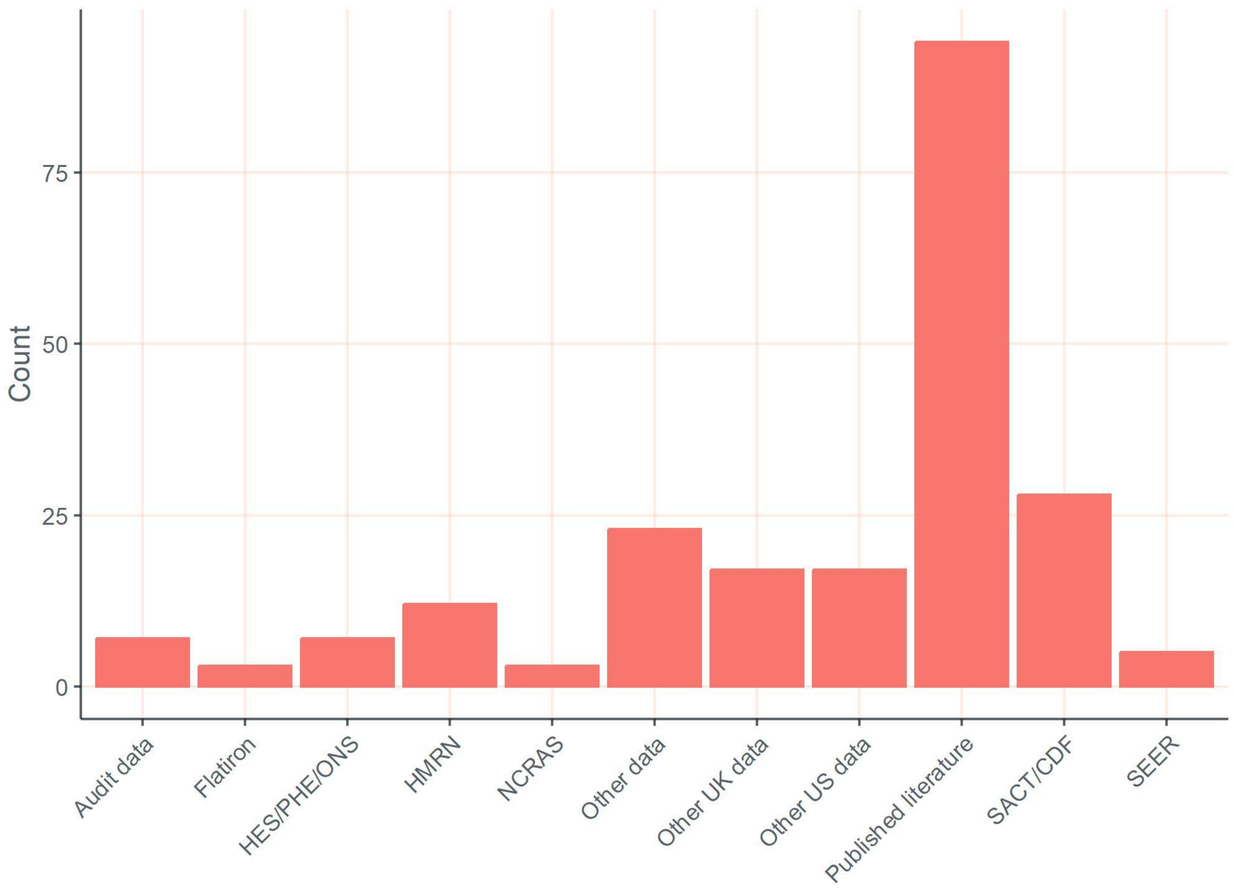

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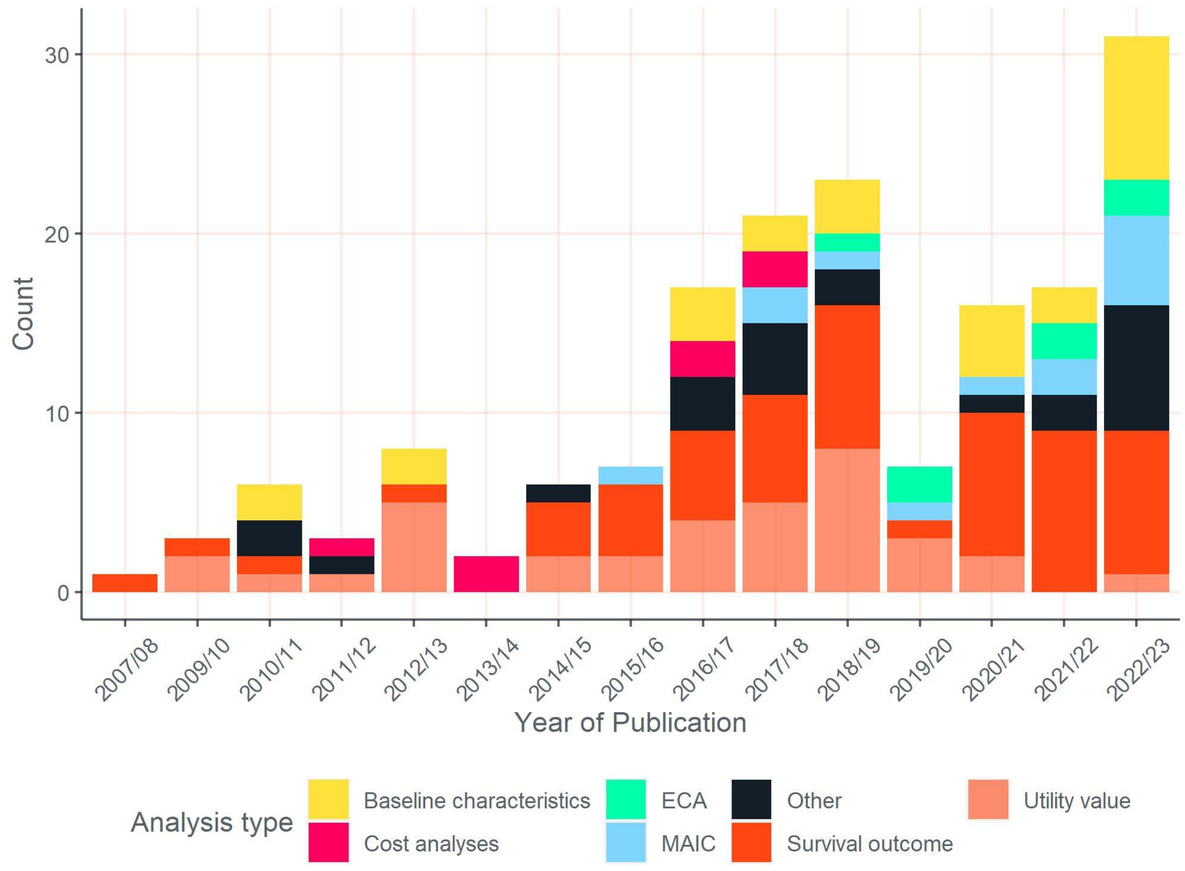

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