Using Real-World Evidence in Health Technology Assessment Submissions: A NICE Case Study

Davies H[1], Davis R[1], Mealing S[1], Barker E[1], Watts K[1], Eastwood I[1], Butler K[1]

[1] York Health Economics Consortium

INTRODUCTION

- Real world evidence (RWE), when correctly generated, reported and utilised, can improve our understanding of health and social care delivery, patient health and experiences, and the effects of interventions on patient outcomes.[1]
- As part of their 2021 to 2026 strategy, the National Institute for Health and Care Excellence (NICE) expressed their ambition to use RWE to resolve gaps in knowledge and drive forward access to innovations for patients.[1]
- However, it became apparent that there was a lack of consensus and transparency around using RWE in health technology assessment (HTA).
- To address such concerns, NICE developed an RWE framework in 2022.
- This tool clearly describes the best practices for planning, conducting and reporting on RWE studies.
- The objective of the framework is to improve the quality and transparency of evidence informing NICE guidance.[1]

Objectives:

- This study investigates what purpose RWE was used for within NICE technology appraisals (TAs) and seeks to understand how its use was viewed by the External Assessment Group (EAG) and NICE committee.

METHODS

- The NICE website was searched to source TAs published from April 2019 to May 2024 that mentioned at least one of ten identified RWE databases.
- For each TA identified via the search methods, four key pieces of information was extracted:
  1. The RWE database that had been used.
  2. Who the RWE was used by, i.e. manufacturer, EAG or NICE committee.
  3. For what purpose the RWE had been used.
  4. The opinion of the EAG and NICE committee on the use of the RWE.

RESULTS

- Thirteen TAs were identified that mentioned at least one of the ten RWE databases.
- The database mentioned most was The Clinical Practice Research Datalink (CPRD) which was used in 92% of the TAs extracted.

RWE database use

- In 62% of TAs, the RWE was solely used by the manufacturer. In 23% of TAs the RWE was used by both the manufacturer and either the EAG or NICE committee.
- The most common use of RWE was to inform or validate model parameters, with such use observed in all but one TA.
- For the remainder, RWE was used to justify structural modelling assumptions.

EAG/NICE use and opinion of RWE use

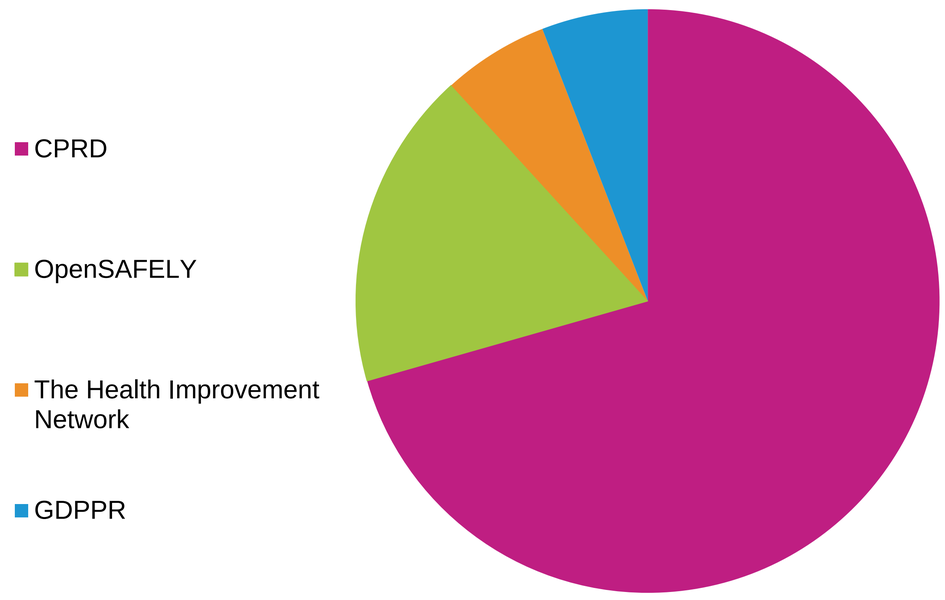
- In all TAs screened, the EAG and NICE committee did not criticise the use of RWE in general.
- Notably, in three cases the EAG leveraged RWE data to inform alternative base case parameter estimates.
- Moreover, in two cases the NICE committee recommended the manufacturer use RWE to validate a model assumption.
- Comments from the NICE committee focused on the relevance and applicability of the RWE to the decision problem.
- Such comments were issued in cases where RWE was ill-fittingly used to inform model parameters.

In 92% of TAs RWE was used to inform or validate model parameters.

In 23% of TAs, the EAG leveraged RWE data to inform alternative base case parameter estimates.

In 15% of TAs the NICE committee recommended the manufacturer use RWE to validate a modelling assumption.

Figure 1: RWE databases used in extracted TAs



EAG and NICE committee positively view the use of RWE in HTA

- Neither the EAG or NICE committee issued criticism for the use of RWE in general when employed in HTA submissions.
- In fact, both the EAG and NICE committee either employed RWE themselves or encouraged its use by the manufacturer to reduce uncertainty.

RWE must be relevant and applicable to the decision problem

- The value of RWE is realised when it is appropriately applied to a decision problem.
- Although RWE may provide robust evidence to inform model parameters via large sample sizes, it must be applicable to the population the parameter is set to represent.
- Otherwise, it's use can increase parameter uncertainty and negatively impact the robustness of model outcomes.

NICE principles to follow when generating or using RWE

- To use data of good provenance which is relevant and of sufficient quality to answer the research question.[1]
- To generate and report on RWE with transparency and integrity.[1]
- To use analytical methods which minimise the risk of bias and characterise uncertainty.[1]

CONCLUSIONS

- It is evident that decision makers respond positively to RWE when it has been correctly employed.
- For RWE to aid the decision-making process, data must be applicable and relevant to the population of interest.
- Correct use of RWE yields the potential to reduce parameter uncertainty.
- This study supports the use of RWE in HTA and demonstrates that a standard framework can ensure appropriate use of RWE.
- Future research could explore RWE use in other HTA agencies to strengthen the current study and broaden its applicability.

REFERENCES

1. National Institute for Health and Care Excellence. NICE real-world evidence framework. Corporate document [ECD9]. 2022.

CONTACT US

heather.r.davies@york.ac.uk

+44 1904 324084

York Health Economics Consortium

www.yhec.co.uk

Providing Consultancy & Research in Health Economics

INVESTORS IN PEOPLE® We invest in people Gold

Scan the QR code to visit the YHEC website

YHEC York Health Economics Consortium