RWD155

Using Real-world-evidence To Inform Pathway Evaluations at NICE: Benefits and Challenges

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Background

The National Institute for Health and Care Excellence (NICE) is piloting a new approach to health technology evaluations as part of its Proportional Approach to Technology Appraisals programme. The pilot aims to develop a core economic model across a disease pathway that can be reused for future evaluations in the same disease area and evolve over time, saving resource and improving consistency of inputs for decision making. Development of these models offers an opportunity for exploring uses of real-world evidence (RWE) through supporting new model structures and population of key model inputs. This study considers the benefits and challenges of using RWE in the development of an economic model in renal cell carcinoma (RCC) and also considers how the pathway approach may fit into existing guidance for using RWE worldwide.

What we did and why

Structured interviews with some key stakeholders in the development of RWE use-cases in the RCC pathway pilot project.

A targeted review of opportunities and challenges identified in published HTA agency guidance documents on methodology and policy for use of RWE.

This was done to understand how the uses of RWE in pathways evaluations would fit into existing frameworks and policies.

RCC pilot explained[1]

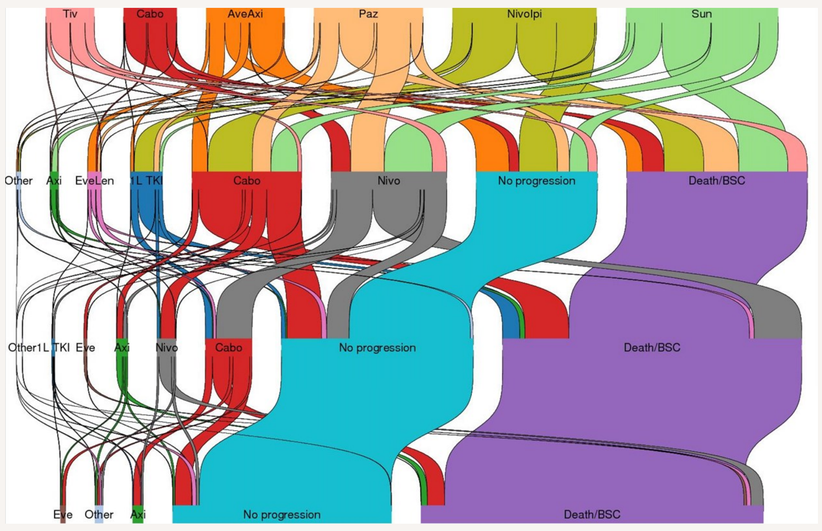
Remit and structure

- People with advanced or metastatic renal cell carcinoma –all treatment sequences informed by real world clinical practice
- Relative treatment effects network meta-analyses from clinical trials on key outcomes

Real-world evidence use-cases

- Establishing baseline risk (survival)
- Validating model outcomes
- Characterising real-world treatment sequences (see Figure 1)
- Time on treatment, discontinuation and relative dose intensity
- Baseline characteristics

Figure 1: Sankey diagram showing transitions between lines of therapy for RCC in a real-world dataset[2]



Benefits

- **Novel modelling structures:** Real-world treatment decisions in clinical practice use sequences of treatments. This is explicitly reflected in the pathway model, avoiding assumptions about subsequent treatments
- **Relevancy and robustness:** Real-world clinical practice generally has higher risk than in clinical trials, applying relative treatment effects to the 'backbone' of real-world risk is most relevant for decision making.
- **Generalisability:** Using evidence from a subset of the UK general population limits concerns about generalisability and reliance on clinical expert input for key model parameters.

Challenges

- **Changing treatment landscape:** The treatments available are constantly changing (including adjuvant therapy in RCC) which means there will always be a delay to accurately showing current clinical practice
- **Quality and risk of bias:** Not all registries will have all the most relevant data points needed for decision-making and data quality will always be an issue in RWE generation
- **Access and confidentiality:** In the UK, access to cancer data is challenging due to data governance and concerns about confidentiality, as well as how the data will be used in decision making
- **Expertise:** There is a need for increased capacity and expertise for processing, analysing and understanding uses of RWE to realise its full potential

What we learned

A total of 6 HTA agencies from 5 countries published guidance or framework on the use of RWE in HTA - these policy statements or guidance have slightly different objectives, but they all embrace the benefits of RWE in facilitating a comprehensive assessment by increasing generalisability, complimenting clinical trial data and filling evidence gaps.

Pathway evaluations are a shift from NICE's current approach to technology appraisals. They offer a unique opportunity to restructure the HTA process to take advantage of new methodological advances and accessibility of RWE. The first pilot of this approach extensively used high quality RWE to populate the model which enhanced the robustness of the appraisal and increased confidence in decision making.

All the use cases in this pilot demonstrated that RWE can be used as the foundation of economic modelling, whilst maintaining clinical trial evidence for establishing relative treatment efficacy.

However, the challenges associated with the use of RWE go beyond the quality of the data. A systematic and collaborative approach is required ranging from improved access to secured data environments, access to routinely collected data, linkage between databases, capacity and expertise in understanding and assessing the data, and recognition of the value RWE can create.

> "RWE formed the backbone of the renal cell carcinoma model. Without it, we would not have the data required to populate the model and would have needed a different model structure that did not achieve the aims of modelling a whole pathway"
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> **NICE Pathway Model Stakeholder**

References

1. Lee et al. Treatments for renal cell carcinoma [ID6186]: A Single Technology Appraisal. Peninsula Technology Assessment Group (PenTAG), 2023
2. UK Renal Oncology Collaborative (2022) John McGrane, Ricky Frazer, Ama Challapalli, Amit Bahl

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