# Assessing the value of real-world evidence in melanoma in health technology assessment appraisals

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## Background and Objectives

Health technology assessment (HTA) bodies are responsible for the evaluation and recommendation of new health technologies for adoption into healthcare systems and/or reassessment of existing technologies to improve patient care[1].

Relevant and available evidence from a range of sources is used for clinical and cost-effectiveness assessment of health technologies on targeted patient population treated in routine practice[2,3].

This analysis aimed to investigate the use of real-world data (RWD) in recent health technology assessment (HTA) appraisals for melanoma therapies in England, Canada, Australia and France.

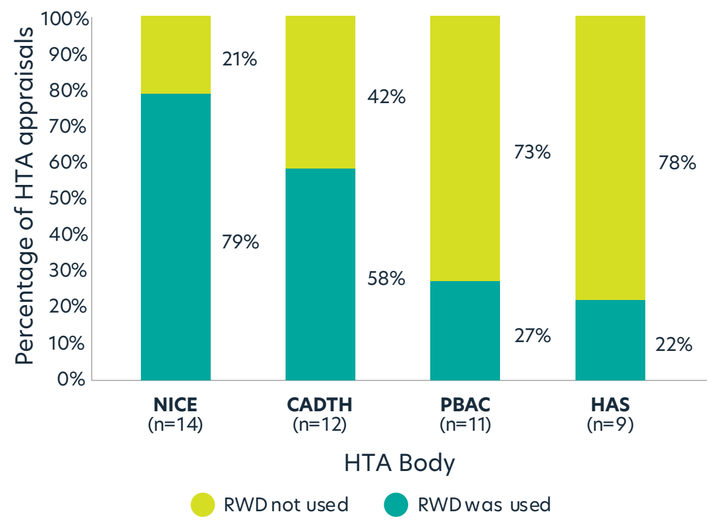
## Methods

All publicly available final HTA reports from the UK National Institute for Health and Care Excellence (NICE), the Canadian Agency for Drugs and Technologies in Health (CADTH) pan-Canadian oncology drug review (pCODR), the Australian Pharmaceutical Benefits Advisory Committee (PBAC), and the French National Authority for Health (HAS) between 1st January 2011 and 30th September 2019 for melanoma therapies were identified from which the type of RWD, context of which RWD was used, assessment commentary, and reimbursement outcomes were extracted.

## Results

Ten melanoma therapies (cobimetinib plus vemurafenib, dabrafenib, dabrafenib plus trametinib, encorafenib plus binimetinib, ipilimumab, nivolumab, nivolumab plus ipilimumab, pembrolizumab, talimogene laherparepvec, trametinib) were assessed in 46 final appraisal documents. RWD was used for clinical or cost-effectiveness assessment in 50% of final appraisals. RWD was for this purpose in all technology appraisals except talimogene laherparepvec and trametinib.

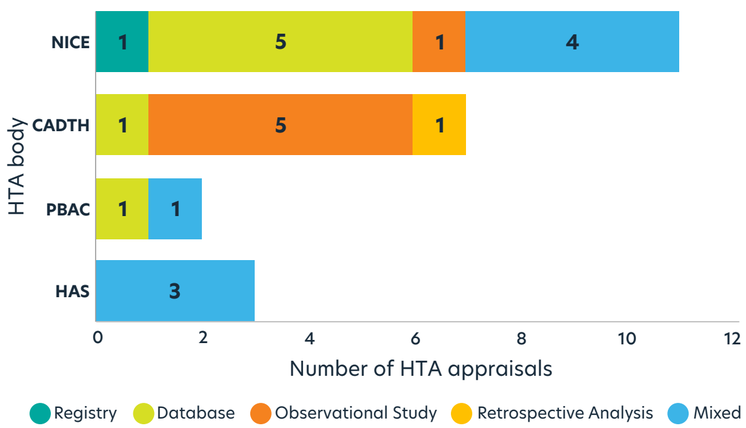
### Figure 1: Final submissions of HTA appraisals for melanoma which contained RWD




### Types of RWD that were used:

Electronic health record, registry, health survey, administrative data, non-randomized trial.

### Figure 2: Sources of RWD in HTA appraisals for melanoma




### Table 1: The source of RWD for parameters used in the 2016 HTA appraisals for cobimenib + vemurafenib for the treatment of melanoma by HTA body

| | Health Resource Use | Healthcare Cost | Survival | Utility Values |
|---|---|---|---|---|
| **NICE[4]** | Patient access scheme | Patient access scheme | × | Observational study |
| **CADTH[5]** | × | Observational study | × | Observational study |
| **PBAC[6]** | × | × | × | × |
| **HAS[7]** | × | × | Surveillance, epidemiology and end results register | Observational study |

## Discussion and Conclusions

RWD submitted spanned retrospective and prospective observational studies, as well as medical chart reviews. The focus of these were effectiveness, costs, quality of life, and safety.

RWD was considered where prospective randomized controlled trial data was not available at the time of decision making, or where long-term survival extrapolation needed external reference points, and to determine which treatments were being used in clinical practice[7-9].

There were cases where RWD was accepted and contributed to HTA decision making. In their assessment of ipilimumab, however, NICE specified that they did not consider that RWD studies provided additional relevant reliable data[9].

## References

A comprehensive list of the references is available upon request.

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