RWD85

A systematic literature review on the use of real world evidence in NICE technology appraisals indicated for multiple myeloma

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Introduction

Real world evidence (RWE) is increasingly used to inform regulatory and reimbursement decision making. There were approximately 6,000 new cases of multiple myeloma (MM) per year in the UK between 2016 and 2018 [1]. In the last 5 years alone, the National Institute for Health and Care Excellence (NICE) in England has appraised 21 medicines to treat MM [2].

Given the relatively large number of health technology appraisals (TAs) in MM that have been submitted by manufacturers, it is important to understand how RWE was historically used so that learnings can be applied to future TAs.

Objectives

The objective of this study was to systematically investigate how RWE has been used by manufacturers in NICE TAs of MM medicines and to describe the impact of RWE on reimbursement decisions made by NICE.

Methods

A systematic literature review (SLR) was undertaken whereby RWE was defined as evidence generated from real world data (RWD) relating to patient health or experience or care delivery collected outside of highly controlled trials [3]. All active TAs published from the earliest date until 31st May 2023, as reported in the list of TAs on the NICE website, were considered [2]. The SLR was registered on the international prospective register of systematic reviews (PROSPERO), CRD42023420032.

Searches were undertaken of the NICE TA database published on the NICE website [2]. All identified NICE TAs underwent a two-stage screening to assess eligibility for full review. One reviewer completed both stages of screening independently while a second reviewer independently screened 20% of TAs in each stage.

TAs of pharmaceutical technologies indicated for MM were eligible for full review if there was ≥1 document from the TA Evidence stage that was accessible for screening and review and if RWE study results were reported. RWE studies that formed part of a SLR were excluded as only primary research studies were considered. Terminated appraisals due to non-submission from the manufacturer, appraisals that were updated since original publication and multiple technology appraisals (MTAs) were also excluded.

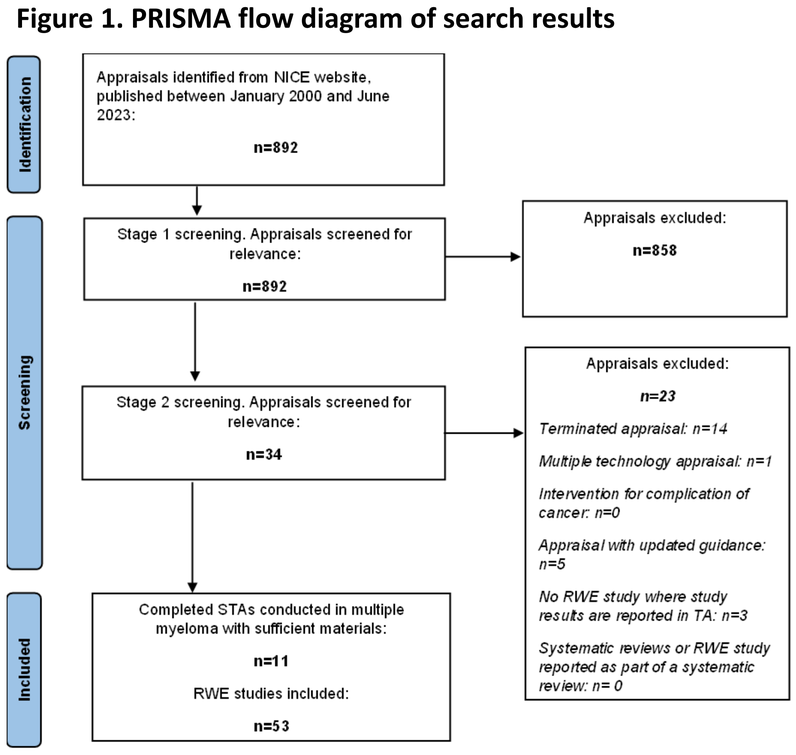
Clinical outcomes (treatment effects), feedback provided to manufacturers on the included RWE studies and impact of these studies on NICE's recommendation for the medicine were assessed.

Results

Between January 2000 and May 2023, NICE published 892 TAs. Of these, 11 TAs published between January 2016 and February 2023 met all eligibility criteria for data extraction (Figure 1). Among the eligible TAs, 53 RWE studies were referenced.

The median number of RWE studies per TA was 5 studies (interquartile range 4-6 studies).

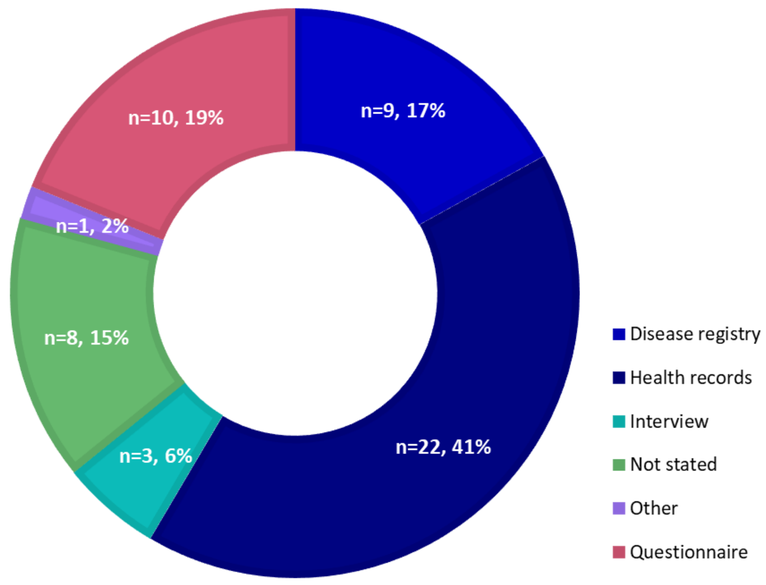
Figure 1. PRISMA flow diagram of search results



Results continued

Approximately half of the RWE studies were undertaken in a single country (n=28, 53%) and most studies included data collected from the UK (n=40, 75%). Figure 2 shows that the most frequently used data source was health records (n=22, 41%), whereas data sources were not reported for a minority of studies (n=8, 15%). Where study design was reported (n=41, 77%), almost all were retrospective studies (n=38, 93%).

Figure 2. Data sources of included RWE studies

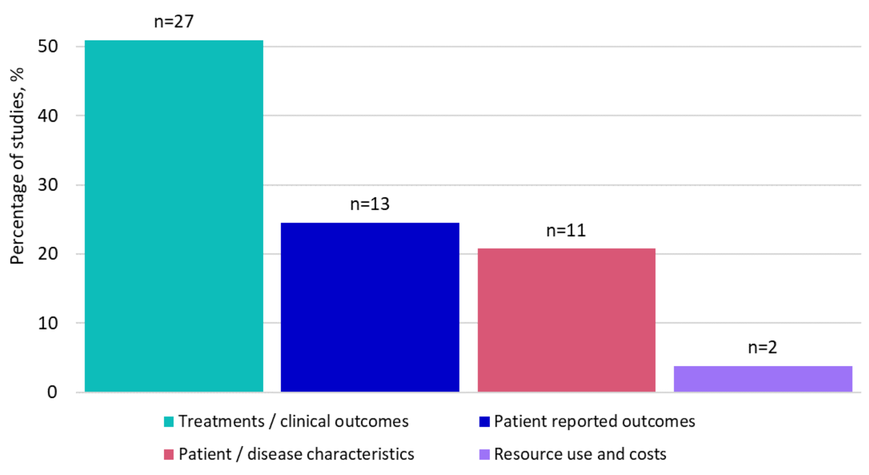


RWE studies were mostly undertaken to generate evidence on treatment characteristics and clinical outcomes (n=27, 51%), patient reported outcomes (n=13, 25%) and patient and disease characteristics (n=11, 21%), Figure 3.

Overall survival and progression-free survival were the most frequently reported clinical outcomes. A range of treatment duration measures were reported including time on treatment, time to progression and treatment-free interval.

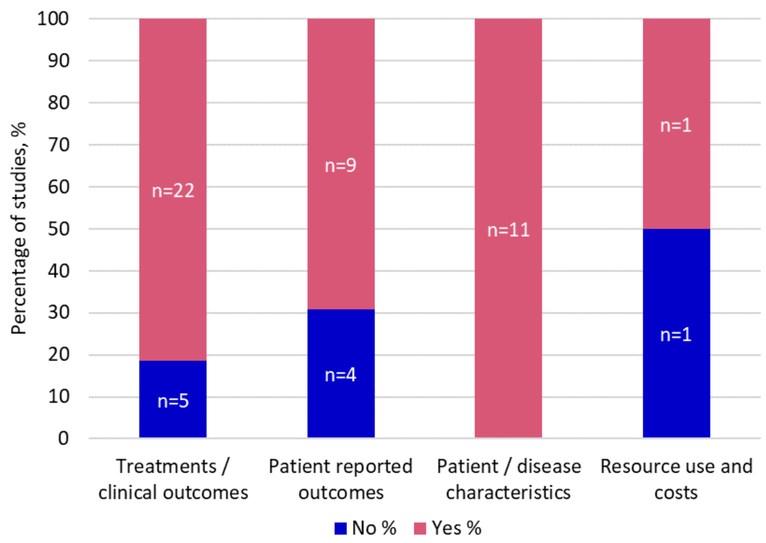
Approximately half of studies that were referred to in the 11 TAs were published either as journal articles (n=20, 38%) or conference abstracts, posters or presentations (n=7, 13%).

Figure 3. Evidence generated by RWE studies



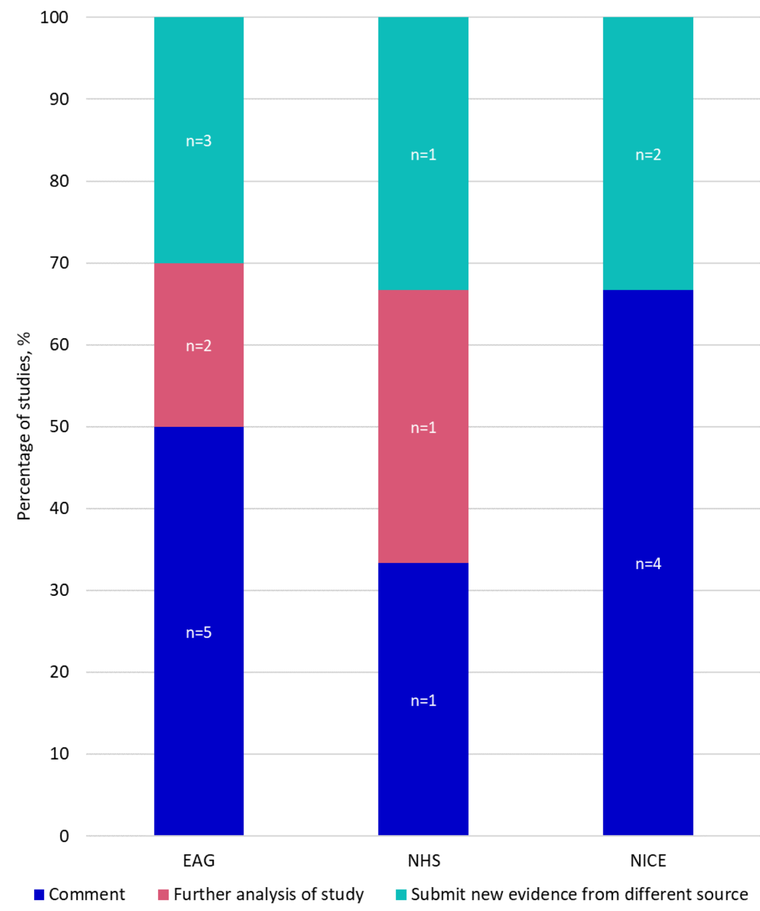
Feedback was not documented for approximately a fifth of RWE studies (n=10, 19%). Where feedback was documented (n=43), 63% of studies received positive comments (n=27). Over half of the feedback was provided by the External Assessment Group (EAG) (n=26, 58%). Figure 4 shows that feedback was given on all studies reporting patient and/or disease characteristics but only on some studies that generated other types of evidence.

Figure 4. Feedback received by manufacturers by evidence type



Manufacturers did not always respond to feedback received. Where responses were documented, these ranged from comments without action, further analysis of the RWE study and submission of additional evidence from other data sources (Figure 5).

Figure 5. Manufacturer responses to feedback on RWE studies



All of the 11 TAs included in the SLR received positive recommendations from NICE, either as Recommended for use in the Cancer Drugs Fund (n=1, 9%), Optimised (n=7, 64%) or Recommended (n=3, 27%).

There was no identifiable trend in terms of type of NICE recommendation by number of RWE studies, type of evidence or feedback received by manufacturers.

Conclusion

This SLR screened all NICE TAs that were published up until May 2023. Out of the TAs of MM medicines that were screened, only approximately a third were eligible for data extraction.

Among all the reviewed TAs, multiple RWE studies on different types of evidence were incorporated into most submissions. Where feedback was given, many studies were well received but not all studies received feedback.

Given that studies were included as part of evidence packages and due to the general and brief nature of documented feedback, it was not possible to determine the direct impact of individual studies on NICE recommendations.

Future research should consider accessing additional, publicly available data sources to investigate the direct and indirect impact of RWE on NICE recommendations.

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Disclosures

J Dempsey was an employee of Pfizer during the study. C Duffield and C Tsang are employees of Pfizer and may hold stock or stock options. This study was funded by Pfizer Ltd.

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