HTA13

SURVEY DATA IN VALUE DEMONSTRATION: ACCEPTABILITY WITHIN HEALTH TECHNOLOGY ASSESSMENTS IN THE UK

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BACKGROUND

Acceptability of survey data

Surveys are recognised by the National Institute for Health and Care Excellence (NICE) as a suitable data source for real-world evidence (RWE)[1] but it is unclear how NICE views unstandardised surveys compared with validated assessment tools such as the EQ5D[2], SF-36[3], AQLQ[4] and DAS28[5]

- ► In some situations, particularly in rare diseases, existing data gaps in health technology appraisals (HTA) require the use of data generated from unstandardised surveys
- ► Unstandardised surveys (henceforth called "surveys") are data collection instruments that lack standardised formats, validation and replicability, but offer more flexibility in data collection
- ► Our study assesses the influence of survey data in NICE HTAs to answer two main questions:

1. How does the incorporation of survey data in rare disease HTAs contribute to the outcome of the assessment process?
2. What types of comments made by the evidence review group (ERG) provide useful insights on the acceptability of survey data?

METHODS

Identifying NICE single technology appraisals (STA) containing survey data

- ► NICE STAs for rare diseases submitted in 2019–2023 were identified using the terms 'survey', 'questionnaire' and 'interview' to capture STAs that provided survey data as supporting evidence
- ► STAs incorporating validated assessment tools only were excluded

Grouping of survey characteristics

STAs that included survey data were grouped based on survey outcomes and survey characteristics

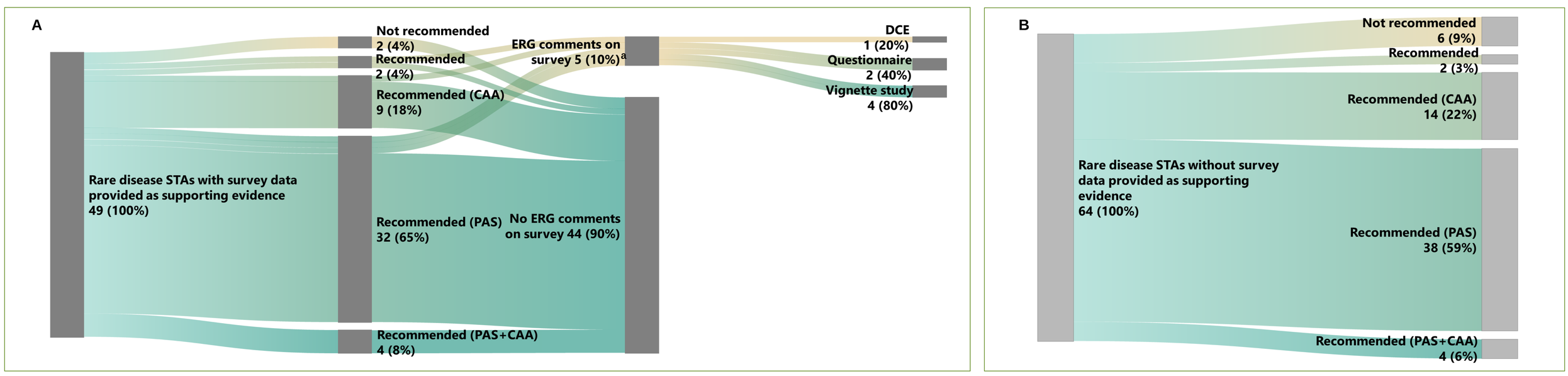
Thematic analysis of ERG comments

Survey-specific ERG comments reported in the earliest available public committee meeting slides were reviewed

- ► Where comments were found, the earliest available committee papers were reviewed for additional comments on the survey. Comments were extracted and categorised according to key survey themes to allow for analysis

RESULTS

Figure 1. Characteristics of rare disease STAs (2019–2023) A) with survey data provided as supporting evidence B) without survey data provided as supporting evidence



Abbreviations: CAA, commercial access agreement; DCE, discrete choice experiment; ERG, evidence review group; HTA, health technology assessment; PAS, patient access scheme; STA, single technology appraisal
Notes: [a] One submission included more than one type of survey data

Overview of survey data in rare disease STAs (Figure 1.)

- ► Of 113 rare disease STAs submitted in 2019–2023, 49 provided survey data as supporting evidence
  - The identified surveys comprised of questionnaires, interviews, discrete choice experiments (DCEs) and vignette studies. Multiple types were often submitted in the same STA
- ► A slightly higher proportion of STAs that included survey data received positive recommendations (96%) compared with those that did not (91%)

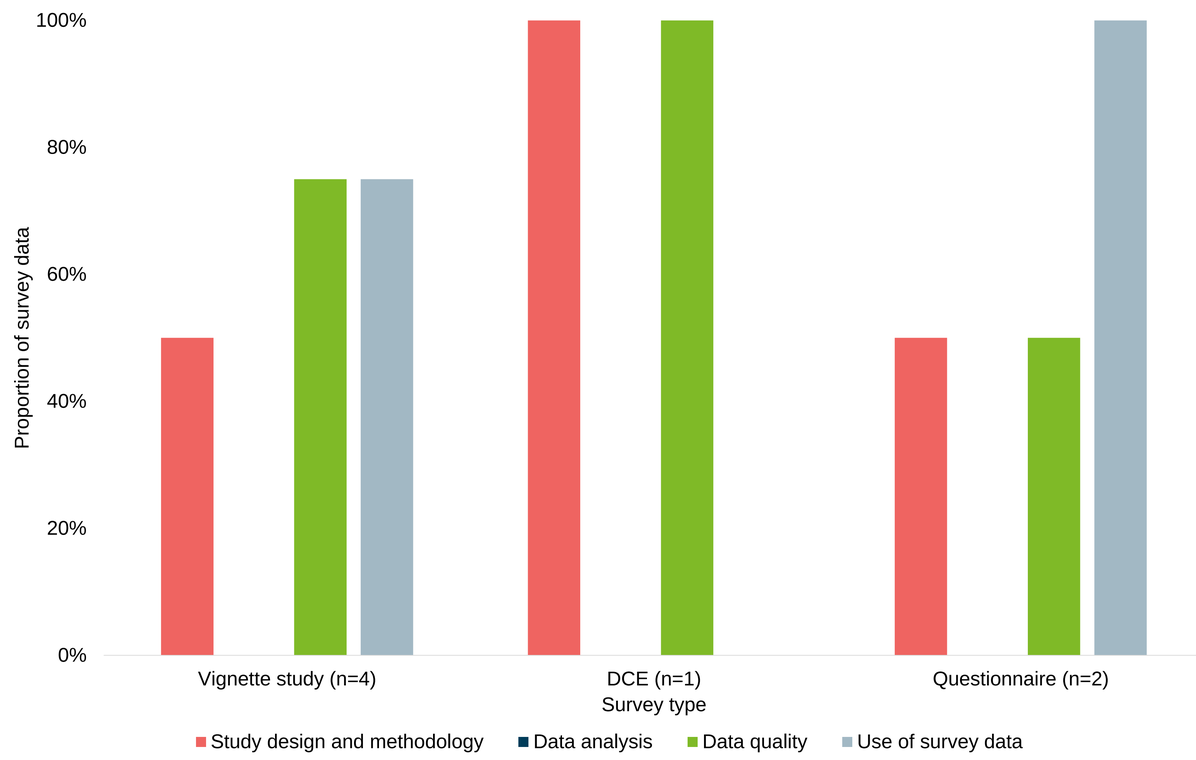
Surveys critiqued by the ERG (Figure 1.)

- ► The ERG only commented on survey data in 10% of submissions (n=4 vignette studies[6,7,9,10], n=2 questionnaires[7,8] and n=1 DCE[9])
  - All comments related to some aspect of the survey (such as design and methodology) as opposed to the choice of a unstandardised survey instead of a validated assessment tool
  - All STAs with critiqued surveys received positive recommendations by NICE
  - Submissions that received negative recommendations did not have any comments from the ERG on the surveys provided as supporting evidence
- ► Some survey types were more likely to receive comments from the ERG than others
  - A third of vignette studies (4/12, 33%) and a fifth of DCEs (1/5, 20%) received comments
  - However, only 2 of the 34 questionnaires (5%) received comments

ERG comments (Figure 2.)

- ► Comments related to study design and methodology, data analysis, data quality and use of survey data
  - Of these, study design, methodology and the quality of the data were a concern across all survey types
  - The DCE, 3 of the 4 vignette studies and 1 of the 2 questionnaires received comments on their data quality

Figure 2. Types of comments provided by the ERG[6–10]



Abbreviations: DCE, discrete choice experiment; ERG, evidence review group

CONCLUSIONS

- ► Following the introduction of the NICE RWE framework, about half of rare disease STAs have incorporated at least one survey as supporting evidence
- ► While the absence of survey data did not seem to impact the likelihood of a technology's recommendation, a high proportion of positive STA recommendations included survey data
- ► Less common survey types such as DCEs and vignette studies were subject to increased ERG scrutiny due to scepticism regarding their methods and data quality, indicating a possible increased complexity compared with questionnaires
- ► Questionnaires received little commentary from the ERG, suggesting a high level of understanding by submitters of what is acceptable, possibly due to sufficient guidance provided by NICE
- ► A limitation of this study is that we used comments made by the ERG as a measure of NICE acceptance. A more detailed analysis is necessary to explore the reasons behind ERG comments and whether they stem from specific limitations of the survey

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