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Vignette-Based Economic Models in NICE Submissions: A Two-Year Review

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SUMMARY

OBJECTIVES

- Vignette studies can serve as an alternative to standardised methods for utility generation, such as EQ-5D, when other approaches are unsuitable or not available
- This study aimed to evaluate the prevalence and characteristics of NICE appraisals that employed vignette methodology

METHODS

- A targeted search was conducted of NICE STAs and HST evaluations from the previous two years
- Appraisals were included if data from a vignette study was incorporated within the final model used for decision-making
- An independent reviewer conducted data extraction on the included appraisals
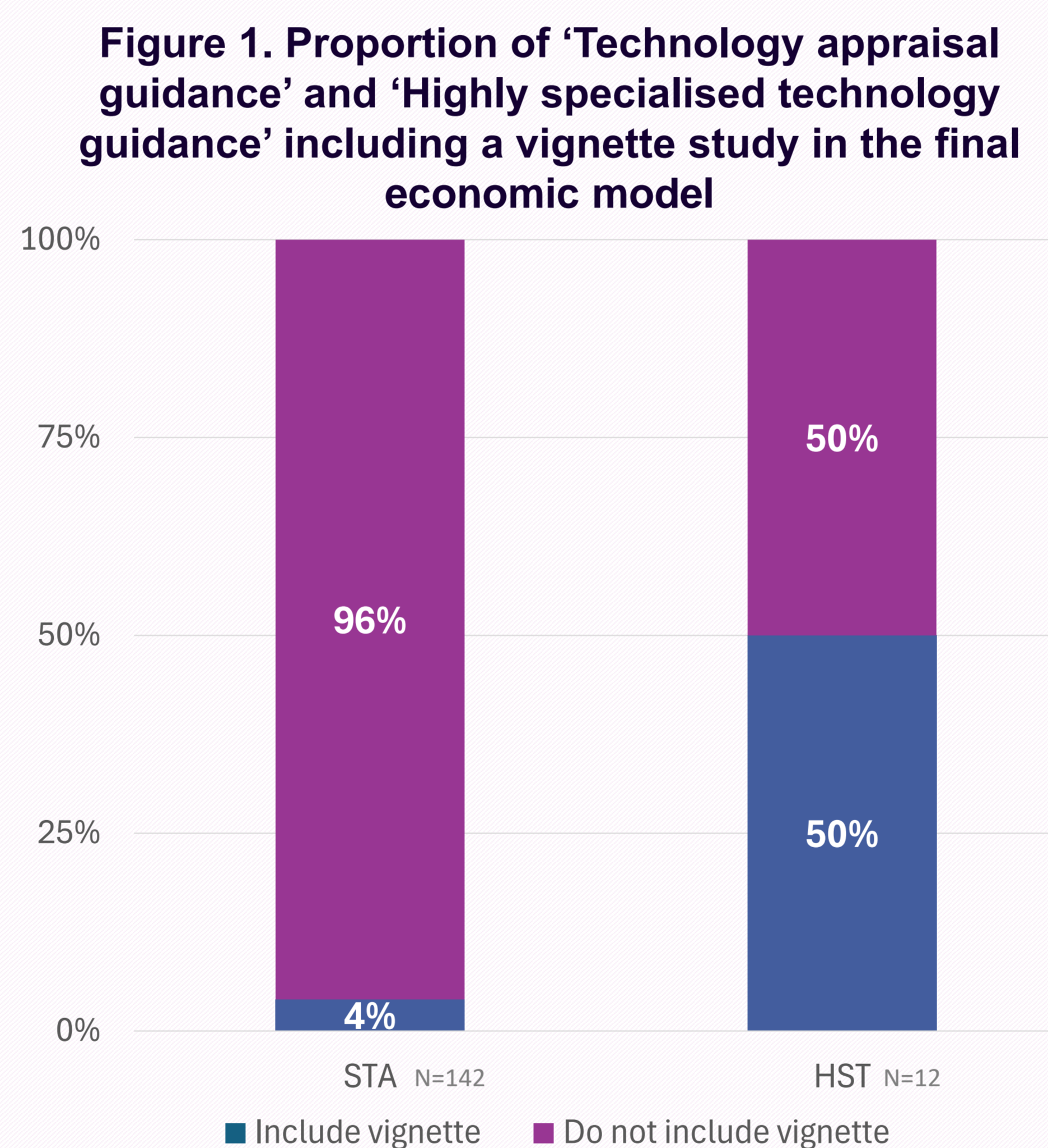
FINDINGS

- A total of 200 appraisals were assessed, of which 12 (6%) included utilities derived from a vignette study in their models
- 50% of all HSTs included a vignette study in their final economic model
- Metabolic diseases were the most frequently appraised disease area where utilities were derived through a vignette study

BACKGROUND & AIMS

- The National Institute for Health and Care Excellence (NICE) identifies EQ-5D as the preferred method for utility generation in its technology appraisal guide. However, if EQ-5D is unavailable or unsuitable, and no other standardised method is feasible, manufacturers must employ alternative methods to generate utilities.
- Vignette studies provide a valuable solution for manufacturers and patients by offering an accepted methodology for generating utilities in patient populations where standardised approaches are not feasible.
- Vignettes are concise, descriptive scenarios or case studies used to illustrate a patient's symptoms, medical condition, and history of disease or illness. They are particularly valuable for obtaining utility estimates, which are used to assess the effectiveness of the drug.
- This study aimed to evaluate the prevalence of NICE appraisals that incorporated vignette studies within their economic models and analyse the characteristics of these appraisals.

METHODS

- A targeted search of the NICE website was conducted on the 11th of February 2025.
- The search was refined using the filters 'Highly Specialised Technologies Guidance' and 'Technology Appraisal Guidance', and assessed dates between 1/01/2023 – 1/01/2025
- A single independent reviewer then excluded any appraisals that did not employ a vignette study to generate utilities within the final economic model used for decision-making.
- Following this, a single independent reviewer systematically extracted data from the committee papers to facilitate further analysis of the application of vignette studies in generating utilities.

RESULTS

- The results from the targeted search in NICE yielded a total of 200 appraisals.
- A total of 34 terminated appraisals were excluded from the analysis.
- Of the remaining 154 appraisals, 142 (92%) were categorised under 'Technology Appraisal Guidance' and 12 (8%) under 'Highly Specialised Technologies Guidance'.
- Of the non-terminated appraisals (n=154), 8% (n=12) utilised data from a vignette study in the final economic model. Of these, 6 (4%) were Single Technology Appraisals (STAs), and 6 were Highly Specialised Technologies (HSTs) evaluations (50% of all HSTs identified) (Figure 1).

Figure 1. Proportion of 'Technology appraisal guidance' and 'Highly specialised technology guidance' including a vignette study in the final economic model

- Of the appraisals utilising data from a vignette study in the economic model, 4/6 STAs and 6/6 HSTs received a positive recommendation for reimbursement.
- The findings indicated a mix of therapy areas in STAs, including oncology, neurology, and haematology, whereas most HSTs were in metabolic disorders.
- Among the 12 appraisals of interest, 9 had data from a preference-based generic HRQoL measure (e.g. EQ-5D) available, which is NICEs preferred source of utilities.
- The most common justification for utilising a vignette study rather than the EQ-5D was a lack of data in the full spectrum of the disease. This was often due to inability to collect HRQoL data in the most severe patients of the disease (Table 1).
- One appraisal used vignette studies to model caregiver disutility.

CONCLUSIONS

- Vignette studies play an important role in NICE submissions, providing utility estimates when standard HRQoL measures may not be sufficient. They help to capture patient (and caregiver) experience in cases when traditional methods, such as EQ-5D, struggle to reflect the disease burden.
- Our study demonstrated that manufacturers are often using vignette studies to support cost-effectiveness arguments in rare disease (HST) submissions.
- Their success suggests that vignette studies can be a valuable tool when patient populations are small, or when conventional measures do not fully capture the impact of a condition.
- The frequent use of vignette studies in rare diseases highlights the challenges of using generic preference-based measures in poorly understood or heterogeneous conditions. This underscores the need for alternative methods to quantify health outcomes and inform NICE decision-making.
- Further research should explore how NICE committees, external assessment groups (EAGs), and manufacturers engage with vignette-based evidence.
- The strategic use of vignette studies enhances cost-effectiveness cases and supports market access in areas with data limitations.

Table 1. Justifications for utilising a vignette study to generate utilities for an economic model

Justification type	Description	N° appraisals
Lack of data in the full spectrum of disease	Utility data for the most severe disease stages is often missing due to trial limitations or difficulties in collecting data from critically ill patients.	5
Lack of stratified data	Trial and SLR data often do not align with key health states, particularly for advanced disease stages, making them unsuitable for economic models.	4
EQ-5D content validity issues	EQ-5D does not capture all relevant aspects of HRQoL, such as social functioning, relationships, or financial burden, limiting its applicability in some conditions.	3
EQ-5D lacks sensitivity	EQ-5D may fail to detect meaningful differences in quality of life, with some patients reporting full health despite significant disease burden.	3
Unable to use EQ-5D due to	Standard HRQoL tools may not be suitable for certain populations, such as those with cognitive impairments or hereditary conditions affecting entire families.	3
Carer disutility modelling	Vignettes were used to estimate the impact on caregivers, capturing burden not reflected in patient-reported measures.	1

Note: Some appraisal have been referenced n several justifications

References

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