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Are Best Practice Methods for Expert Elicitation Being Applied in NICE Highly Specialised Technology (HST) Submission? - a Review and Evaluation

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Elicitation of expert opinion plays an increasingly important role in bridging the evidence gap in health-care decision-making, including Health Technology Assessment. Sufficient understanding and transparency in the process and methods used in elicitation can contribute to the generation of high-quality evidence.

Background

- Expert elicitation is commonly employed where there is a paucity of evidence for health care decision making (HCDM), especially for Highly Specialised Technologies (HST) for rare diseases.
- This method, however, can introduce bias and heuristics (i.e., cognitive shortcuts used by individuals when facing complex judgments).
- There are also limited guidelines about the appropriate structure of an elicitation process, the application of which could potentially reduce bias or at least quantify the level of uncertainty.
- Bojke et al. (2021)[1] established a set of 9 principles to develop reference case methods for expert elicitation to inform HCDM in Health Technology Assessment (HTA), which are well aligned with the guidelines from INFARMED (2019).[2]

Principles

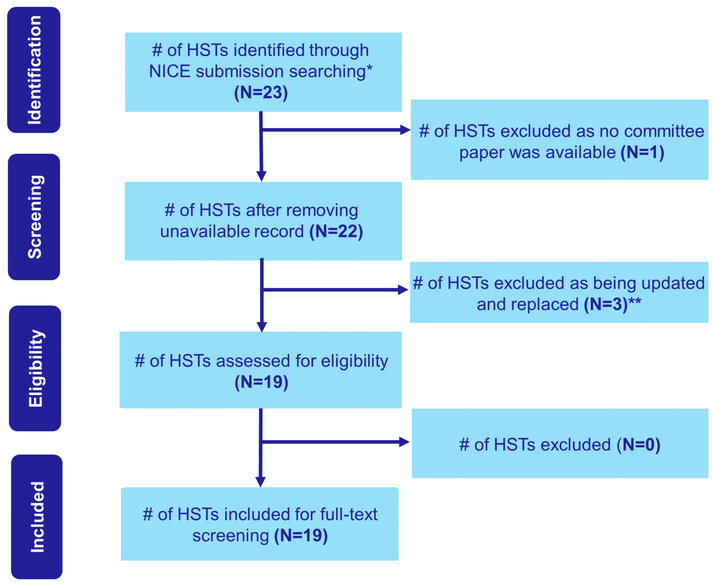
- **Principle 1:** Ensure transparency in the process and reporting of elicitation for validation of the results and high-quality outcomes
- **Principle 2:** Provide useful information for the decision problem
- **Principle 3:** Aim for consistency but respect the constraints of the decision-making context. The quantities that are elicited should be consistent with the model parameters and model structure.
- **Principle 4:** Reflect uncertainty at the individual expert level by acknowledging imperfect knowledge that may exist in experts' judgments and exploring the uncertainty of the outcomes.
- **Principle 5:** Recognize and act on biases. The techniques used in designing and eliciting should aim to mitigate against biases and heuristics, while suitable training should be given to experts.
- **Principle 6:** Be suitable for experts who possess substantive skills and who are less likely be trained in probability and statistics. Suitable methods for HCDM should fit the purposes and experts' capability unless additional training is provided.
- **Principle 7**: Recognize where adaptive skills are required (i.e. adapting knowledge of the related quantity), especially when experts are not familiar with the quantities.
- **Principle 8:** Recognize, and act on, between-expert variation. It is essential to understand the reasons behind heterogeneity and to reflect it in the pooled distribution (e.g., through group consensus or mathematical aggregation methods).
- **Principle 9:** Promote high performance. The elicitation exercise, where possible, should identify, discuss, and account for different levels of normative expertise to encourage equal performance among experts.

Objective

- The current study critically examined how structured expert elicitation has been reported in HST submissions to the National Institute of Health and Care Excellence (NICE) compared to the Bojke et al. and INFARMED recommendations. [1,2]

Methods

Figure 1. PRISMA flow diagram

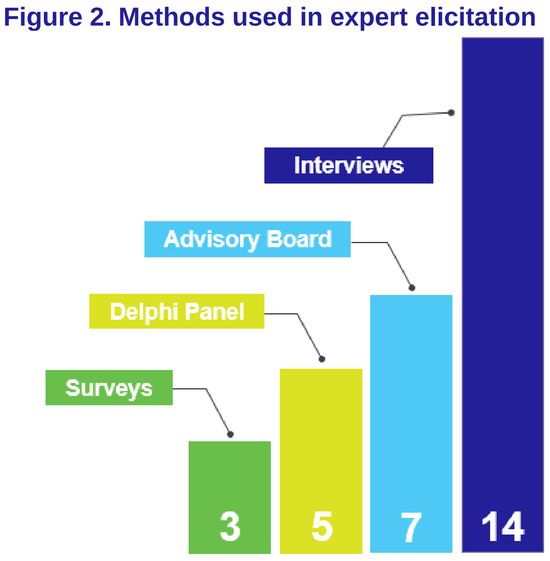


- A targeted review was conducted to identify NICE HST submissions that involved expert elicitation, between January 2015 (first published HST) and March 2023. Submissions without fully documented committee papers or being replaced by resubmissions were excluded from the current review.
- The HSTs were critically appraised against the criteria to determine whether they met each principle in full (>80%), partially, or not.

Results

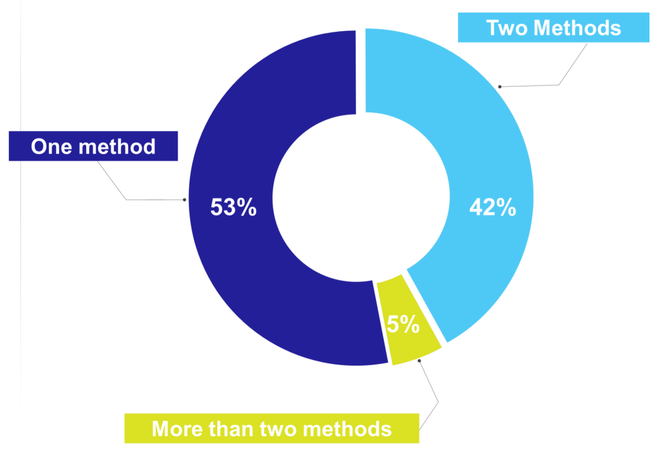
- Out of 23 identified HSTs, 19 were included for review and 4 were excluded (Figure 1). All included HSTs (n=19) included expert elicitation. Interview was the most common technique (14 HSTs), followed by advisory board (7), Delphi panel (5) and survey (3) (Figure 2). The majority of HSTs (95%) used one (53%) or two methods (42%) for elicitation, while only 5% of the submissions had more than two elicitation exercises (Figure 3). The most frequently combined methods were advisory board and interviews (5 HSTs). Most HSTs (79%) involved fewer than 10 experts.

Figure 2. Methods used in expert elicitation



Note: The sum of the total number is larger than 19 since some HSTs used multiple methods.

Figure 3. Number of methods used per submission



- Health-related quality of life (64% of HSTs) and healthcare costs (58%) were the most frequent topics where expert opinion was sought. Eight HSTs (42%) aimed to inform and/or validate economic model assumptions, structures, and data sources.
- Only one submission met most principles (6 of 9). Most submissions were transparent (summarised the process [95%], reported results [79%]; Principle 1) and provided information that addressed the decision problem (89%; Principle 2), with consistency among the process, context and capacity of the decision-making entities (84%; Principle 3). The majority (95%) used method specifically for HCDM, otherwise, the necessary background was provided (Principle 6). Experts were familiar with the target quantity or had relevant experience (Principle 7), as stated in 84% of submissions.
- Only 11% of the submissions reported between expert variation (Principle 8). Little to no information was provided regarding methods to explore uncertainty (Principle 6), to recognise and act on biases (Principle 5) and to account for differing levels of normative expertise (Principle 9).
- Since the publication of INFARMED (2019) and Bojke et al. (2021), as shown by the light blue and dark blue line in Figure 4, there has been clear improvement in transparency (Principle 1), being informative (Principle 2), consistency (Principle 3) and suitability for expert's skills and capability (Principle 6). However, little evidence has been found regarding other principles.

Figure 4. Results matrix detailing individual HST agreement with each principle

Not met | Partially met | Fully met* | INFRAMED guidelines (2019)[2] published | Bojke et al. (2021)[1] published

Included HST submissions	Principle 1	Principle 2	Principle 3	Principle 4	Principle 5	Principle 6	Principle 7	Principle 8	Principle 9
HST4[3]	Not met	Not met	Not met	Not met	Not met	Not met	Not met	Not met	Not met
HST5[4]	Partially met	Not met	Partially met	Not met	Not met	Not met	Partially met	Not met	Not met
HST7[5]	Partially met	Fully met*	Partially met	Not met	Not met	Not met	Partially met	Not met	Not met
HST8[6]	Partially met	Fully met*	Fully met*	Fully met*	Not met	Partially met	Partially met	Fully met*	Not met
HST9[7]	Partially met	Fully met*	Partially met	Not met	Not met	Partially met	Partially met	Partially met	Not met
HST10[8]	Partially met	Fully met*	Fully met*	Not met	Not met	Partially met	Not met	Not met	Not met
HST11[9]	Partially met	Fully met*	Fully met*	Not met	Not met	Partially met	Partially met	Not met	Not met
HST12[10]	Partially met	Fully met*	Partially met	Not met	Not met	Partially met	Partially met	Partially met	Not met
HST13[11]	Partially met	Fully met*	Partially met	Not met	Not met	Partially met	Partially met	Partially met	Not met
HST14[12]	Partially met	Fully met*	Partially met	Not met	Not met	Partially met	Partially met	Not met	Not met
HST15[13]	Partially met	Fully met*	Partially met	Not met	Not met	Partially met	Partially met	Partially met	Not met
HST16[14]	Partially met	Fully met*	Partially met	Not met	Not met	Partially met	Partially met	Not met	Not met
HST17[15]	Partially met	Fully met*	Partially met	Not met	Not met	Partially met	Partially met	Not met	Not met
HST18[16]	Partially met	Fully met*	Partially met	Not met	Not met	Partially met	Partially met	Fully met*	Not met
HST19[17]	Partially met	Fully met*	Partially met	Not met	Not met	Partially met	Partially met	Partially met	Not met
HST20[18]	Fully met*	Fully met*	Partially met	Not met	Not met	Partially met	Not met	Not met	Not met
HST21[19]	Partially met	Fully met*	Partially met	Not met	Not met	Partially met	Partially met	Not met	Not met
HST22[20]	Partially met	Fully met*	Partially met	Not met	Not met	Partially met	Partially met	Not met	Not met
HST23[21]	Partially met	Fully met*	Fully met*	Fully met*	Not met	Partially met	Partially met	Not met	Not met

Abbreviations: HST, Highly Specialised Technology

*Principles were judged to be fully met if the study reported on ≥80% of the principle criteria based on pre-developed data extraction sheets (available upon request).

Conclusions

Despite the limited availability of best practice guidelines for the structure of the elicitation processes, recommendations regarding the transparency and applicability of the information to the decision problem were mostly applied across the NICE HST submissions. On the other hand, little was stated on how to handle uncertainty and biases and ensure high performance.

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Disclosures

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References

1. Bojke L, Soares M, Claxton K, Colson A, Fox A, Jackson C, Jankovic D, Morton A, Sharples L, Taylor A. Developing a reference protocol for structured expert elicitation in health-care decision-making: a mixed-methods study. Health Technol Assess. 2021 Jun;25(37):1-124. doi: 10.3310/hta25370 2. Perelman J, Soares M, Mateus C, Duarte A, Faria R, Ferreira L, Saramago P, Veiga P, Furtado C, Caldeira S, Teixeira MC, Sculpher M (2019): Orientações Metodológicas para Estudos de Avaliação Económica. INFARMED - Autoridade Nacional do Medicamento e Produtos de Saúde, I.P., Lisboa. 3. HST4 - Migalastat for treating Fabry Disease (2017). 4. HST5 – Eliglustat for treating type 1 Gaucher disease (2017). 5. HST7 – Strimvelis for treating adenosine deaminase deficiency-severe combined immunodeficiency (2018). 6. HST8 – Burosumab for treating X-linked hypophosphataemia in children and young people (2018). 7. Inotersen for treating hereditary transthyretin amyloidosis (2019). 8. HST 10 - Patisiran for treating hereditary transthyretin amyloidosis (2019). 9. HST11 - Voretigene neparvovec for treating inherited retinal dystrophies caused by RPE65 gene mutations (2019). 10. HST12 - Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2 (2019). 11. HST13 - Volanesorsen for treating familial chylomicronaemia syndrome (2020). 12. HST14 - Metreleptin for treating lipodystrophy (2021). 13. HST15 - Onasemnogene abeparvovec for treating spinal muscular atrophy (2021). 14. HST16 - Givosiran for treating acute hepatic porphyria (2021). 15. HST17 - Odevixibat for treating progressive familial intrahepatic cholestasis (2022). 16. HST18 - Atidarsagene autotemcel for treating metachromatic leukodystrophy (2022). 17. HST19 - Elosulfase alfa for treating mucopolysaccharidosis type 4A (2022). 18. HST20 - Selumetinib for treating symptomatic and inoperable plexiform neurofibromas associated with type 1 neurofibromatosis in children aged 3 and over (2022). 19. HST21 - Setmelanotide for treating obesity caused by LEPR or POMC deficiency (2022). 20. HST22 - Ataluren for treating Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene (2023). 21. HST23 - Asfotase alfa for treating paediatric-onset hypophosphatasia (2023).