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# Emerging practice taskforce "Expert elicitation methods for health care decision making"

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ISPOR Report

## Recommendations on the Use of Structured Expert Elicitation Protocols for Healthcare Decision Making: A Good Practices Report of an ISPOR Task Force

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ABSTRACT

Healthcare decision making, including regulatory and reimbursement decisions, is based on uncertain assessments of clinical and economic value. This arises from the evidence supporting those assessments being uncertain, incomplete, or even absent. Qualitative, structured expert elicitation (SEE) is a valuable tool for extracting expert knowledge about an uncertain quantity and formulating that knowledge as a probability distribution. This creates a useful input to decision modeling and support, particularly in areas with limited evidence, such as advanced therapy products, precision medicine, rare diagnoses, and other areas with high uncertainty.
Structured SEE protocols are used to improve the transparency, accuracy, and consistency of quantitative judgments from experts, limiting the effect of heuristics and biases. This task force report introduces 5 commonly used protocols for SEE (Sheffield elicitation framework; modified Delphi method; Cooke's classical method; investigate, discuss, estimate, aggregate protocol; and the Medical Research Council reference protocol). It describes the common elements of SEE, discusses how these protocols differ in their implementation of those elements and illustrates the use of the protocols.
The report then reviews the relevant constraints on implementing SEE within the context of healthcare decision making and considers the strengths and weaknesses of these protocols in light of those considerations. Because this is an introductory report on an emerging topic, specific recommendations on practice are not made. However, there are broad recommendations based on the suitability of the different protocols in various decision contexts. The report concludes with recommendations for further research to better guide future practice.

*Keywords:* Bayesian estimation, IDEA protocol, modified Delphi, SHELF, structured expert elicitation, uncertainty.

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Highlights

- Because no prescriptive guidance for the use of SEE is available, this task force report has identified 5 different protocols (Sheffield elicitation framework; Cooke's classical method; investigate, discuss, estimate, aggregate; modified Delphi and the MRC reference protocol) and compares them on the level of elicitation, individuals or groups, and the mode, with or without interaction of experts and the level of aggregation.
- To prevent behavioral and cognitive biases, healthcare decision making using SEE should carefully consider the specificities of the setting they work in before selecting a particular SEE protocol.
- The task force concludes that many studies using SEE have not documented well the SEE approach used. In particular, if no particular protocol was chosen, the task force urges decision makers to be transparent in the reporting of the methods and results.

### Introduction

Healthcare decision making (HCDM), including regulatory and reimbursement decisions, is typically grounded in uncertain assessments of clinical and economic value (See Appendix 1 in Supplemental Materials found at https://doi.org/10.1016/j.jval.2024.07.027 for a full list of acronyms). Uncertainty, in part, arises from the evidence supporting those assessments being itself uncertain, incomplete, or even absent. For example, evidence regarding the parameters used in health economic models, such as transition probabilities, is often uncertain. Therefore, resulting cost-effectiveness and budget impact for a new technology are also uncertain.

The uncertainty surrounding decision making is particularly challenging in emerging medical fields with only limited evidence, such as advanced therapy products,[1] in the field of precision medicine where evidence usually is difficult to obtain through clinical trials[2] or where regulators must extrapolate evidence obtained in larger populations to a smaller (genomically defined) subgroup or to populations that fall outside the clinical trial inclusion criteria.[3]

Uncertainty can also be the result of a small number of patients (rare diagnoses), limited duration of follow-up, or trial design (use of surrogate outcomes or lack of an active comparator).

Judgments from individuals who have expertise on relevant subject matter can help better understand uncertainty to support value assessments and facilitate decision making. However, it is important to distinguish quantitative "elicitation" from qualitative inputs from experts, for the purpose of "validation," where the focus is expert consultation or expert discussion in a deliberative process. In contrast, structured expert elicitation (SEE) is the process of extracting expert knowledge about some uncertain quantity or quantities and formulating that information into probability distributions.[4]



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## Aims of the report

- Provide emerging good practice guidance in formal (structured) elicitation methods to support a range of HCDM processes, across a range of settings.
- Harmonize methodology across HCDM settings, where appropriate, and ultimately, improve decision making.

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## SEE Task Force Members

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**ISPOR support**: Elizabeth Molsen

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## Rationale for task force

- Many decision makers are using EE as part of their processes.
  - Structure processes (SEE) can support robust evaluation/decision making
  - Methodology used for SEE in HCDM is heterogeneous
  - Need to consider what guidance is most appropriate in this context.
- The priority for global guidance on SEE in HCDM is more apparent given recent policy changes.
  - Processes to provide promising medicinal products to patients earlier in the R&D pathway (adaptive pathways).
  - Focus on other technologies, including diagnostics, medical devices, and complex interventions (public health or social care).
    - Evidence base for these interventions and programs is often less developed.
    - Less rigorous regulatory requirements.

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## Why an ISPOR TF?

- The use of 'real world data' is increasingly promoted - ISPOR's RWE Initiative.
  - Expected insufficiencies in the evidence base.
- Aligned with ISPOR's mission is to promote health economics and outcomes research excellence to improve decision making for health globally.
  - Expert elicitation is a valuable tool to support decision making.
  - The TF will develop emerging good practices on an important process for decision makers from a global perspective.
  - Will consider alternative guidance and methods when applying SEE
- Recommendations will be of broad interest to a number of ISPOR stakeholders now and in the future.
  - Timely, currently methods updates by NICE, UK, ZIN and CADTH

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## Contents of the report (and what is not covered)

Describing SEE methods to inform evidential uncertainties to support HCDM:

- Outline the rationale for SEE and its use in HCDM
- Outline the available protocols/guidance for expert elicitation and methods within these:
  - Sheffield method (SHELF)
  - Delphi method
  - Cooke's classical method
  - IDEA protocol
  - MRC protocol
- Consider the applicability of these protocols/guidance in different contexts/settings

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## Professional expert opinions widely used

- Background information on the disease area, current clinical practice, and how this might alter through use of a new technology, service, or intervention;
- Interpreting evidence and its generalisability to current health services;
- Defining the decision problem, e.g. identifying relevant comparators and the patient;
- Population or subgroups most likely to benefit from the new technology;
- Sense-checking data used in reviews and economic modelling (including the plausibility of model structures, inputs, and results);
- Formal consultation on draft guidance.

Peel *et al* Appl Health Econ Health Policy, 2018

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## Expert elicitation: elicitation of quantities

- Expert opinion / Expert Knowledge Elicitation (EKE) – (Peel *et al*, 2018)
  - Qualitative information about treatment pathways, patients most likely to benefit, interpretation of evidence, review of draft guidance
- Weight elicitation and decision support (MCDA) – (Marsh, et al 2018)
  - Eliciting weights for criteria to be considered for decision support
- Preference elicitation (DCE) - (Johnson et al, 2013)
  - Quantifying the relative preference of (several) alternatives
- Structured Expert Elicitation (SEE) – (Bojke et al, 2021)
  - Eliciting quantitative information from experts about unknown quantities, for instance about number of patients to benefit from treatment

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## Why we need guidance for SEE?

- Improve quality of SSE studies and facilitate peer-review of studies using SEE
- Present and describe *differences* and *how* they are used
  - SHELF, DELPHI, Cooke, IDEA, MRC
- Methods differ how quantities are elicited
  - Individual vs. group, with or without feeback, fixed vs. variable interval method (FIM/VIM), aggregation (consensus or mathematical), and fitting distributions
- Multiple sources of biases (anchoring, availability, representativeness)
  - Guidance in design and analysis of structured elicition may avoid bias
- Guidance on when SEE is recommended given resources needed
  - Informal vs. formal elicitation protocols
- Intention is to be *method agnostic*

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## SEE for diagnostic performance FDG-PET (cancer)

Purpose is evaluate use of FGD-PET in a generic economic model (GEM):
- across multiple tumors
- multiple stages (diagnosis, staging, surveillance and re-staging)

SHELF method used
- Quantify diagnostic work-up for pancreatic cancer
- Elicitation of additional diagnostic procedures (imaging, blood, biopsies) following initial CT


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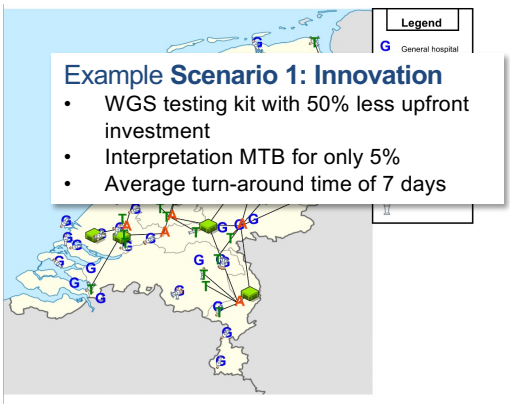
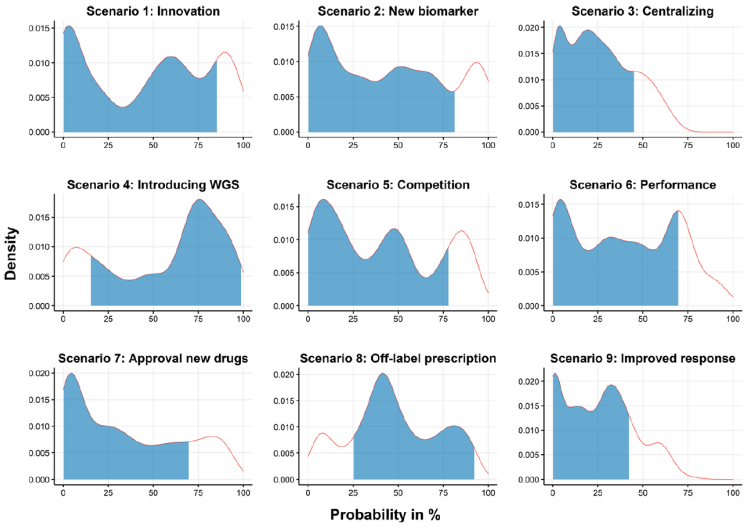
# Scenario analysis: probability of WGS being used




Example **Scenario 1: Innovation**

- WGS testing kit with 50% less upfront investment
- Interpretation MTB for only 5%
- Average turn-around time of 7 days




Van de Ven, Simons et al,
BMC Cancer (2021)

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# Constraints

- Consider the benefits of formal elicitation vs. fast estimates from experts,
- Appreciate strategies to mitigate risk of bias, including willingness of experts to provide reliable inputs,
- Consider the resource cost (number of quantities, time, money, etc) required to execute a formal elicitation,
- Identifying and accessing (either face to face, online, groups or individuals) experts appropriate to provide assessments,
- Prepare experts (evidence dossier) for the quantities to be elicited,
- Return of results to experts and testing of face-validity

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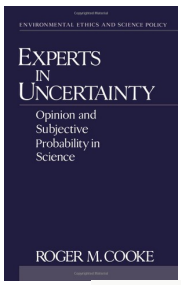
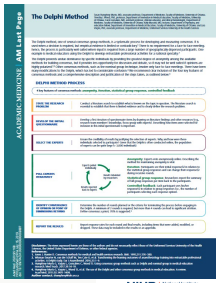
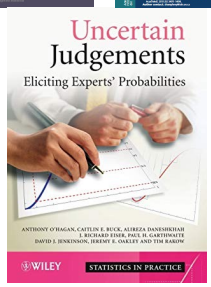
## We reviewed 5 elicitation protocols

1. Cooke
2. Delphi
3. IDEA
4. Sheffield
5. MRC









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## Key difference: Expert interaction

No interaction

*Cooke*

Anonymous interaction

*Delphi*

Group discussion

*Sheffield*

*IDEA*

*MRC (option)*

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## Key difference: Aggregation

| Behavioural (consensus) | Mathematical (opinion pool) | Mathematical with performance-weights |
|---|---|---|
|  |  |  |
| *Sheffield*<br>*Delphi* | *IDEA*<br>*MRC* | *Cooke* |

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Cooke

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Sheffield

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