Designing Fit-For-Purpose Patient Preference Studies - A COA Developer Perspective

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Background

> The importance of capturing and meaningfully incorporating patients' experiences, perspectives, needs, and priorities (i.e., the patient voice) into drug development and evaluation is well-established.[1,2]

> Patient Preference studies are increasingly recognized as a valuable means of generating patient insights to inform decisions across the medical product life-cycle (MPLC), including: product design and development; clinical trial design and operationalization; benefit-risk assessment for regulatory approval and reimbursement; and clinical practice.

> With experiential understanding of the disease and treatment, patients can offer a unique perspective that differs from that of healthcare professionals or competent authorities.

Aims

> Existing methodological recommendations and available regulatory guidance emphasize the importance of patient-centric preference study design.[3,4] However, to date, there is limited practical guidance regarding incorporation of patient perspectives into the design of quantitative preference elicitation studies.

> Most stated-preference methods involve rating, ranking, or making a choice between hypothetical options presented as questions in a survey. Parallels can therefore be drawn with considerations for the development and implementation of Patient-Reported Outcomes (PROs) and other Clinical Outcome Assessments (COAs).

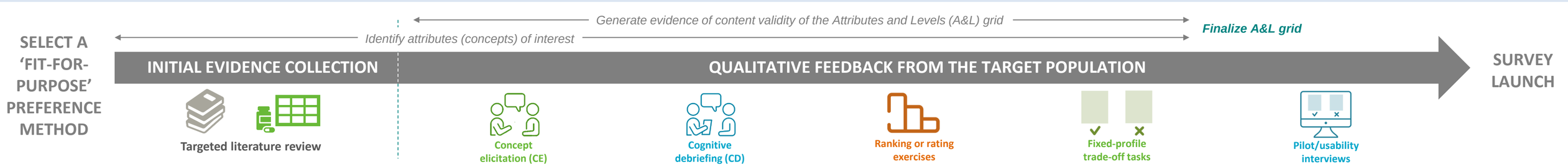
> This poster outlines recommendations and considerations regarding the design and testing of patient preference surveys, from a COA-developer perspective.

Concluding perspectives

> To maximize the utility and validity of patient preference studies, in-depth qualitative pre-testing is recommended to ensure that surveys are fit-for-purpose in the specific context-of-use (i.e., decision context).

> Standard and endorsed research methods utilized in the development and validation of PRO/COA measures can be adapted for use in the design and execution of preference studies.

> As perspectives on the design and utility of Patient Preference studies continue to emerge from regulatory agencies, HTA bodies, and other key decision-making stakeholders, clearly-defined expectations and practical guidance for developing preference studies to inform decisions across the MPLC are needed.



STEP 1: SELECTING A 'FIT-FOR-PURPOSE' PREFERENCE METHOD

> Research teams must firstly evaluate if a stated-preference method is appropriate to the specific research question. Such methods are most valuable in the context of 'preference-sensitive' decisions.[5]

> While the discrete choice experiment (DCE) is the most applied preference elicitation method, alternative preference methods may be more 'fit-for-purpose' depending on the specific needs of decision-makers, and practical considerations (e.g., sample feasibility, timelines).[6]

STEP 2: IDENTIFYING ATTRIBUTES (CONCEPTS) OF INTEREST

Targeted literature and evidence reviews

> The first step in regards to evidence generation should involve a targeted literature review, to generate a preliminary evidence base in a population reflective of the population of interest and any pre-identified attributes (concepts) of interest.

> This review may involve a variety of sources depending on availability and the specific decision context:

- ***Clinical trial literature/product labels;***
- ***Real-world evidence*** *(e.g., surveys and registry data);*
- ***Qualitative evidence*** *(e.g., interviews, patient blogs and forums);*
- ***Patient-facing materials/resources;***
- ***Expert clinical input;***
- ***Previous Patient Preference studies.***

> As well as informing attributes and levels of importance, targeted reviews can also be used to identify and evaluate measures of relevant psychological constructs for understanding individual patient preferences.

STEP 3: FEEDBACK FROM THE TARGET PATIENT POPULATION

The value of a dedicated 'qualitative pre-testing phase'

> The total number of attributes to include in a patient preference study is finite and research teams have to make decisions on which attributes to include. Therefore, attribute selection is a potential source of experimental bias.

> Optimal attribute selection involves a mixed-approach evidence generation phase, involving targeted literature reviews, qualitative interviews in the target population, and multi-disciplinary input (including expert advice).

> The development history and rationale for attributes, levels, imagery, and supporting materials should be documented in a clear decision tracking matrix.

> Qualitative testing of survey content (such as A&L grids and upfront training/information) is considered critical in facilitating evidence-based design.

Considerations for the qualitative sample

> Interview samples are determined based on the research question and context.

- *Sample sizes must be sufficient to demonstrate achievement of* ***conceptual saturation*** *at the sample level (and if applicable, at the sub-group level);*
- *Research teams should select the most optimal* ***recruitment approach*** *to meet the specific needs and application of the study findings, and be aware of how recruitment approach may introduce untoward bias;*
- *Similar to COA development, any preference studies planned within a regulatory context are likely to require* ***confirmation of diagnosis,*** *to confirm enrolment of the specific population of interest;*
- *Samples should be* ***reflective of the target population*** *and consider* ***diversity*** *in perspectives and experiences with respect to disease presentation, treatment, demographics, sociocultural background, and health literacy.[3,7]*

Concept elicitation (CE)

> CE interviews involve open-ended questioning to elicit spontaneous qualitative data. In the context of PP studies, CE is a valuable technique to explore treatment experiences, perspectives, attitudes, and priorities.

> Evidence generated is valuable to contextualize perspectives on attributes/levels, provide confirmatory evidence to attributes identified in a preliminary literature/evidence review (Step 2), or identify attributes in the absence of existing evidence from the patient perspective.

Cognitive debriefing (CD)

> CD interviews involve a series of targeted probed questions (utilizing 'think aloud' techniques) intended to elicit evidence on comprehension and relevance of materials. This technique is widely-endorsed by regulators in the context of COA development.

> In the context of a preference study, CD interviews can be adapted to inform A&L grid design, by supporting the following:

- ***Attribute and level comprehension*** *– particularly important for attributes reflecting clinical trial AE data and/or complex efficacy/survival outcomes;*
- ***Attribute relevance, importance, impact/influence on treatment choice;***
- ***Level increment meaningful differentiation/meaningful change;***
- ***Attribute independence;***
- ***Appropriate communication of benefit, risk, or harm;***
- ***Potential limitations*** *(e.g., grounding effects or implausible combinations).*

Ranking or rating exercises

> Attribute ranking or rating exercises (e.g., most important/least important) are valuable to understand the relevance of selected attributes, and elicit preliminary insights regarding relative attribute importance.

> These exercises may be conducted in CE interviews (undefined range for spontaneous attributes) and CD interviews (defined range for pre-identified attributes) to help inform decisions regarding attribution selection for the final survey.

Fixed-profile trade-off tasks

> Qualitative interviews provide an opportunity to test assumptions in regards to potential choice card design, format, and task burden (e.g., number of attributes).

Pilot/usability interviews

> Piloting comprises a soft launch of a survey with a small subset of the full participant sample, to check that the survey and data collection work as expected within conditions that the survey is expected to be completed.

> Patient preference surveys are commonly administered online – therefore, testing should be performed using browsers and devices (e.g., tablet, laptop, mobile phone) that participants are expected to use.

> Observation and interviewing of participants can be used to identify difficulties in navigating and responding to the survey, issues with visual presentation, and overall burden associated with survey completion which could lead to missing or inaccurate data.[3]

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