Estimating sample size for qualitative research in clinical outcome assessment research: one size does not fit all!

IP23

Helen Kitchen, MSc
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Introduction to the panel

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- Specialist Lead, Clinical Outcomes Assessment, DRG Abacus

Kathryn Lasch, PhD

- Executive Director, Patient Reported Outcomes, Pharmerit International

Helen Doll, PhD

- Strategic Lead, Quantitative Science, Clinical Outcomes Solutions

Katy Benjamin, PhD

- Director, HEOR – Patient Reported Outcomes, Abbvie Inc.

Objective

The panel today will discuss the theoretical underpinnings and practical considerations for estimating sample sizes for qualitative research studies that are intended to support clinical outcome assessment (COA) development & validation

Importance of collecting qualitative data from **patients** is widely recognized

- **Generalizability** is a key consideration when planning study designs
- Sample sizes should be **representative** of target patient population
- **Representation**: Patients in the study sample reflect the diversityheterogeneity of patient characteristics in the target population (although the **distribution** could vary)

How do we sample for qualitative research for COA validation?

Probability sampling vs non-probability sampling

- Random vs non-random

Qualitative research is exploratory; non-probability sampling is appropriate & includes[1]:

- Convenience: pre-defined group, continues until a set number of subjects are enrolled
- Purposive: participants intentionally selected to represent pre-define relevant traits or conditions
- Quota: ensures inclusion of people who may be underrepresented by convenience or purposeful sampling
- Snowball: participants refer others who they know may be eligible
- Case study: a single participant

Few practical guidelines currently exist for sample size estimation in COA validation

[1] Luborsky and Rubinstein (1995). Sampling in Qualitative Research. Rationale, Issues, and Methods. Res Aging, 17(1), 89-113.

Types of qualitative studies & sample size estimation

Concept elicitation

- ISPOR Task Force Part 1: No rule can be provided to determine either the sample size or number of iterations needed to reach saturation in PRO instrument development
- Lasch et al (2010): 10-12, depending on sample homogeneity.
- Guest, Bunce, & Johnson (2006): 12-15 in a relatively homogenous sample

Cognitive interviews

- Willis (2005) has suggested that 7-10 interviews are sufficient to confirm patient understandability of an item.
- Leidy & Vernon (2008): Number needed is a function of the complexity of the instrument & the diversity of the population
- ISPOR Task Force Part 2: Recruit participants considered typical or generally representative of the target population, and a purposive sample of those who may have unique responses/perspectives or difficulty.

Clinical trial exit interviews

- von Maltzahn, Marshall, Arbuckle et al (2017) 20-30 for refining COAs through exit interviews dependent on indication, budget, perceived importance, & diversity
- Anthony el al (2017) used n=35 to explore whether outcomes associated with primary endpoint were clinically meaningful

Sample characteristics & size will vary depending on the target population and concept.

There is a lack of consensus within the field & little empirical research.

How can we determine sample size? What qualitative and quantitative methods are available to us?

Over to the panel!

- **Kathy Lasch** will present **qualitative** approaches
- **Helen Doll** will present recent advances in **quantitative** approaches
- **Katy Benjamin** will debate the **PROs and CONs** of these approaches
- **Helen Kitchen** will summarise **clinical & practical factors** influencing sample size

You're all invited to debate the methods and approaches discussed today!

Clinical & practical factors to consider

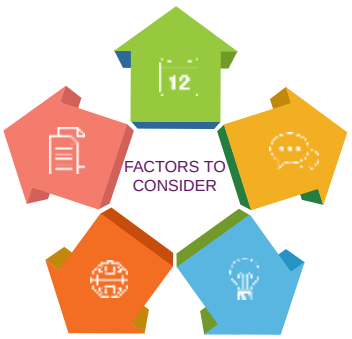
Helen Kitchen, MSc

Specialist Lead, Clinical Outcomes Assessment, DRG Abacus

Clinical & practical factors to consider in sample size estimation

RESOURCE CONSTRAINTS
Time relative to clinical development, budget

STUDY TYPE
Open-ended exploratory vs cognitive interview vs preference study vs exit interview



SUB-GROUPS
Number & type of sub-groups of interest including clinical & demographic groups

COMPLEXITY
Complexity of research question
Complexity & type of COA

PREVALANCE & AVAILABILITY
Prevalence of the condition in countries of interest
Availability of patients

?

Q&A