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

Helen Kitchen, MSc
Specialist Lead, Clinical Outcomes Assessment

Introduction to the panel

Helen Kitchen, MSc
- 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 diversity/heterogeneity 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 & includes¹:

- 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


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
- 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 et 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?
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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
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Clinical & practical factors to consider in sample size estimation

**PREVALENCE & AVAILABILITY**
Prevalence of the condition in countries of interest
Availability of patients

**COMPLEXITY**
Complexity of research question
Complexity & type of COA

**RESOURCES 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

**FACTORS TO CONSIDER**

Q&A