

# Choosing Outcomes Across a Population: Deliberative Approaches

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# Core outcomes

“minimum set of outcome measure that must be reported in all RCTs in a given health condition”

(Boers, et al., 2014)



# Why core outcomes?

- Enable decisions based on most **important** outcomes (& measures)
- Prevent **bias**
- Facilitate comparison & **synthesis**
- Reduce **waste**



# How to identify outcomes?

- Include patient advisors
- Traditional qualitative research (e.g., focus groups)
- Stated preference methods
- Consensus process (Delphi, nominal group technique, deliberative methods)



# Patient *reported* outcomes

- Direct assessment of patient experience
- *PROs* may be important to patients
  
- Important outcomes can also be observed
- Some patients cannot report outcomes



# Patient *selected* outcomes

- Patients may identify what matters
- But not aware of all possible outcomes (especially new products)
- Medical terms vs. describing experience

Glucose > 120 mg/dl  
Increased prolactin levels  
Prolongation of the QT interval  
Extrapyramidal symptoms

- Values may differ



# Patients make a difference

For example, the original OMERACT core set in rheumatoid arthritis was established without direct patient input; but focus groups were held at OMERACT 6 (in 2002), the first OMERACT meeting that patients were invited to attend. Supported by a previous email survey, fatigue and sleep were identified as missing from the OMERACT core set, which only included pain, function, joint counts, global assessments and a blood test. The experience of fatigue has been reported by a large proportion of people with rheumatoid arthritis, and it is often the most important problem for individual patients.

(Williamson, et al., 2012)





# Addressing diversity

- Vision not meeting requirement for driving
- Cataracts
- Glaucoma
- Needing eye surgery (for cataracts or high eye pressure)
- Needing medicine for controlling high blood pressure or cholesterol
- Infection (e.g., sinusitis)

Relatively more important to:

- 1) Whites
- 2) People with high SES

Yu T, Holbrook JT, Thorne JE, Flynn TN, Van Natta ML, Puhan MA (2015). "Outcome preferences in patients with noninfectious uveitis: Results of a Best-Worst Scaling Study." Invest Ophthalmol Vis Sci 56: 6864-6872.



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*Core Outcome Measures in Effectiveness Trials*

The COMET (Core Outcome Measures in Effectiveness Trials) Initiative brings together people interested in the development and application of agreed standardised sets of outcomes, known as '**core outcome sets**'. These sets represent the minimum that should be measured and reported in all clinical trials of a specific condition, and are also suitable for use in clinical audit or research other than randomised trials. The existence or use of a core outcome set does not imply that outcomes in a particular trial should be restricted to those in the relevant core outcome set. Rather, there is an expectation that the core outcomes will be collected and reported, making it easier for the results of trials to be compared, contrasted and combined as appropriate; while researchers continue to explore other outcomes as well. COMET aims to collate and stimulate relevant resources, both applied and methodological, to facilitate exchange of ideas and information, and to foster methodological research in this area.

**When searching the COMET database, please note that a systematic review is currently underway to identify eligible material, and we are continually updating the database as we identify eligible studies. Therefore, the records retrieved by any search might increase on a daily basis.**



# Delphi consensus method

Round 1:  
Topic generation

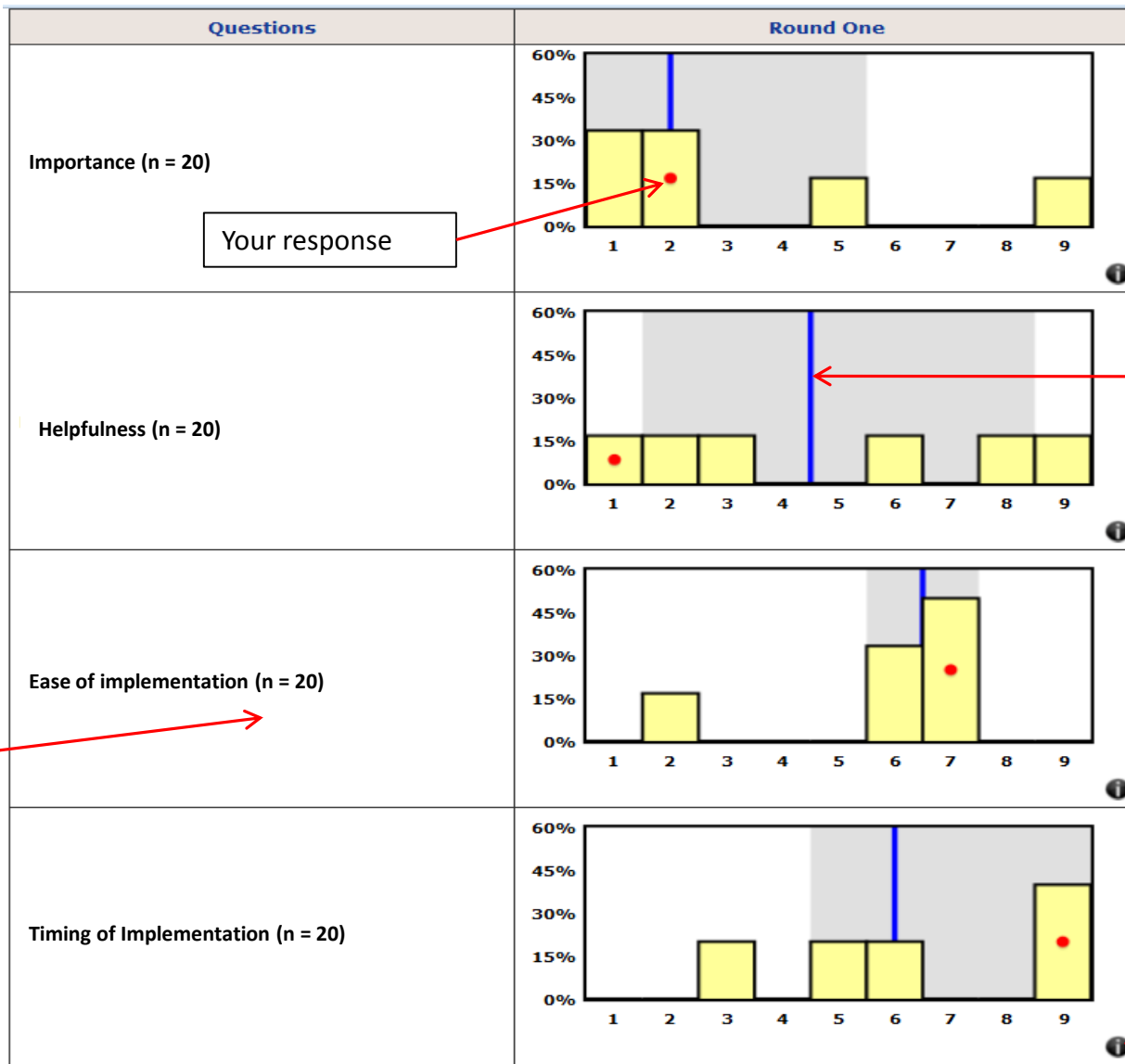
Round 2:  
Assessment

Round 3:  
Feedback / Discussion

Round 4:  
Reassessment

- Asynchronous and anonymous
- Large or small groups
- Combines quantitative and qualitative data
- Patients can propose outcomes
- Iterative





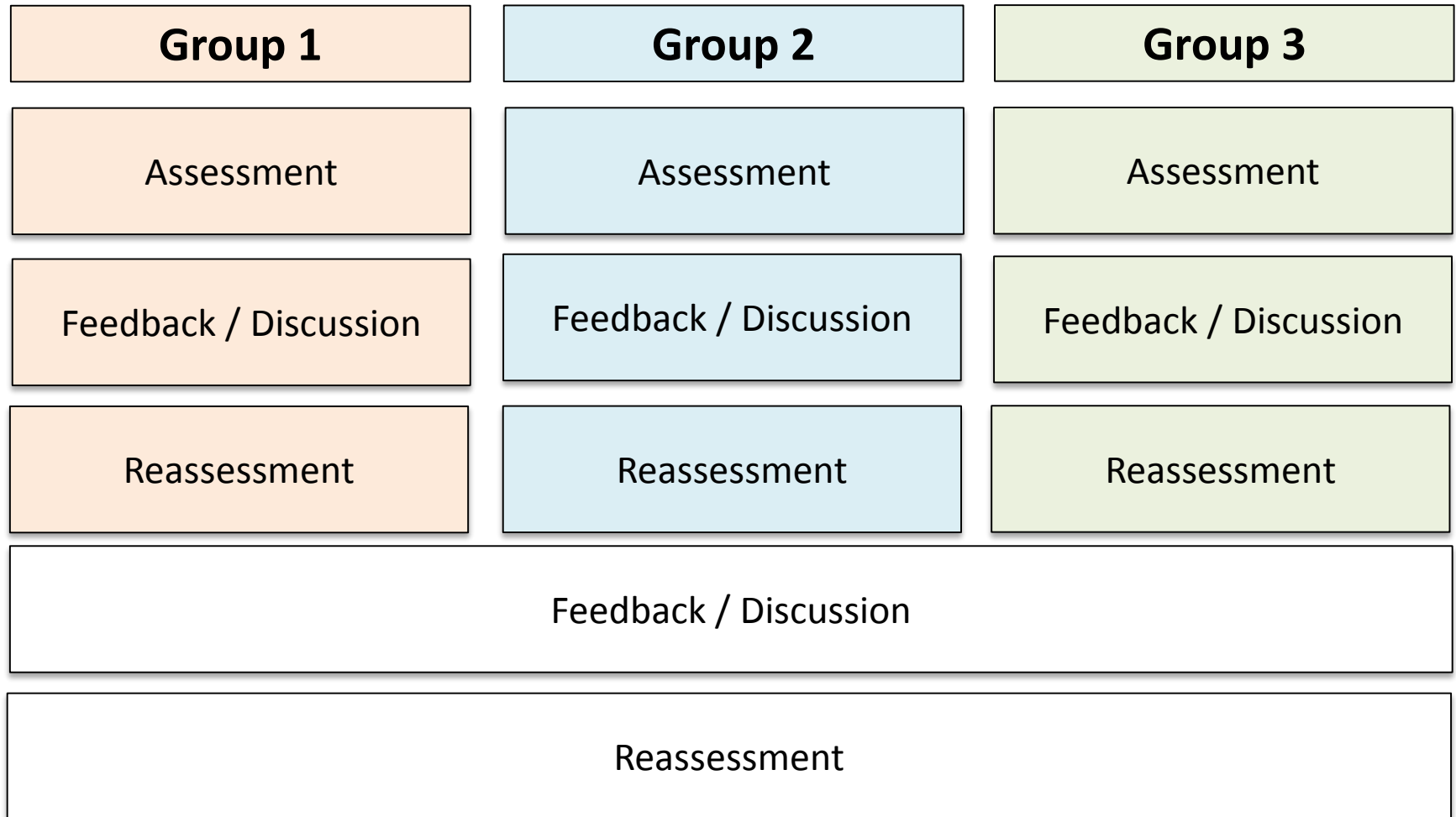
Group median

Hover over to see the response scale

ExpertLens Software, RAND Corp  
Thanks to Dmitry Khodyakov and Sean Grant



# Delphi with multiple groups



# Conclusions

- Identify important outcomes
- Explore areas consensus & disagreement
- Compare within and between groups
- Identify hypotheses for confirmatory research

