

## Plenary Session 3 Best Practices for PRO Instrument Development and Validation

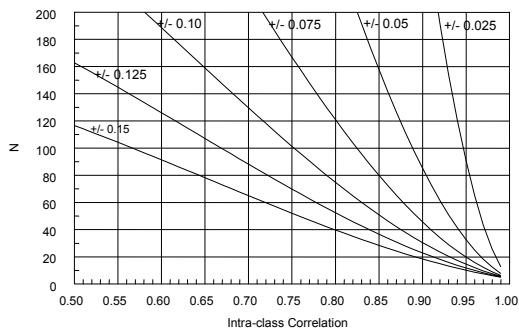
### Discussant

Nancy Santanello, MD, MS  
Merck Research Laboratories  
Department of Epidemiology  
June 29, 2006

## Patient Role in PRO Development and Validation

- PROs must be patient centered
- The content of measures must be relevant to the patient's experience
- Patient input essential to assure comprehension of questions, response options, and instructions
- Many older measures developed without patient input work well and are tried and true
  - Many of these measures are reliable and valid through application in patient populations
  - Should not re-invent new measures if the older measures work well
- Guidance should not be prescriptive as to indicate how many patients are needed for the various steps in development and validation – patient numbers for validation analysis depends on reliability

## Sample Size as Function of Reliability and Half-Widths of 95% Confidence Limits



Source: Streiner & Norman. Health Measurement Scales. New York: Oxford University Press, 1995 (Reliability: repeat administrations over a time interval where no true change should occur)

## Recall: Shorter Does Not Mean Better

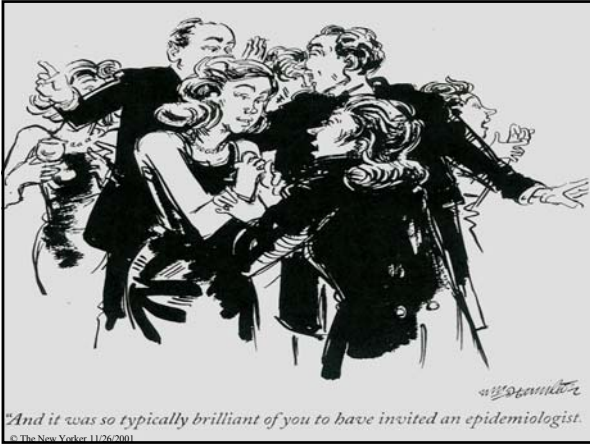
- Disease, frequency of events, ability to recall event, trial design, patient burden should drive recall period
- Cognitively determine recall period with patients – What makes sense? What do patients remember?
- Shorter recall:
  - Chronic or acute diseases with day to day or hour to hour variability: *asthma, sleep disorders, hot flashes, urinary symptoms, migraine symptoms, acute pain*
- Longer recall:
  - Chronic disease which with less variability (eg. COPD, chronic pain, cancer symptoms)
  - Infrequent episodes which are easily remembered (eg. hospitalization, ED visits, doctor visits, surgeries, AEs)

## Weighting

- Weighting is unnecessary when
  - Instrument has a fixed number of response options for all items
  - Items are of broadly similar relevance and importance
  - Items are reasonably highly correlated
- If weighting is used, the developer should provide evidence to support the weighting

## Validation: How Much is Enough?

- Agree that minor changes do not requiring field testing, only cognitive debriefing ... but what is minor?
  - Changing VAS or any question from vertical to horizontal
  - Slight changes to wording of instructions
  - Minor format changes – e.g. boxes to lines
  - Going from validated patient-administered paper to patient-administered e-PRO- how much more validation do we really need?
  - Translations of a validated PRO or e-PRO
  - ? Removing a domain - need to justify why



*And it was so typically brilliant of you to have invited an epidemiologist.*

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