




# OUTCOMES RESEARCH APPLIED



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## Why are we doing this?

- “Health-related quality of life is the ultimate outcome of health care” (ISOQOL Vision)
- “The ultimate goal of health care is to restore or preserve health-related quality of life” (David Osoba)

# Types of Outcome Data

- Disease-focused -- biologic outcomes such as survival; event-determined measures
- Patient-focused -- HRQOL, symptom control, psychological; psychometrically-based measures
- Population-focused -- healthy days, economic impact; preference-based measures

## Where is HRQOL Data Used?

- Clinical trials - mostly phase III, some phase II, rarely phase I
- Descriptive studies - concurrent or retrospective
- Day-to-day clinical practice - still often informal
- Population studies - usually in form of QALYs

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## Purpose of HRQOL Assessment

- Screening
- Health-profile description
- Clinical decision-making
- Prediction of outcomes
- Preference (utility) assessment

# Clinical Decision-making - Settings

- Primary and secondary prevention
- Active treatment to cure a disease
- Active treatment to prolong life without cure
- Palliative treatment to control symptoms
- Supportive care
- Palliative care

# Health Care Decision-making

<u>Level</u>	<u>Target</u>	<u>Purpose</u>	<u>Locale</u>
1. Micro	Individuals	Individual benefit	Bedside, clinic, etc.
2. Meso	Groups	Group benefit	Institution, clinical trials
3. Macro	Communities, populations	Population benefit	Government

Adapted from Sutherland HJ and Till JE, Qual Life Res, 1993;2:297-302

# Users' Perspectives

- Does significance of HRQOL data differ with perspective of user?
- Micro - patient - relatives - physician, nurse, psychologist
- Meso - hospital administrator, financial officer, clinical trials group
- Macro - epidemiologist; health care policy maker
- If so, is there a sensible resolution?

## Finding A Common Ground

- Opinions of users of health care, i.e., patients and “patients-to-be”, are of the highest priority, since their ideas of what constitutes a meaningful change is paramount in deciding whether there is improvement or deterioration of HRQOL
- What do patients regard as a meaningful change in HRQOL?
- Does “meaningful” equal “clinically significant”?

## Patient's Perspective

- Minimal Important Difference (MID) or Subjectively Significant Difference (SSD)
- Determined by health transition questions (HTQs)
  - 0.5 on a 7-point scale (MID)
  - 6-8 % of the breadth of the HRQOL scale (SSD)
  - similar, with different questionnaires, in respiratory diseases, cancers and arthritis

# MID/SSD CONCERNS

- Reliability and validity of the methodology has been questioned
  - single items HTQs can't be tested for psychometric reliability
  - patients' memories can't be trusted
  - response shift may alter perceptions

# Concordance with Other Approaches

- MID/SSD roughly equal to small to moderate (0.2-0.5) effect size (ES)
- MID/SSD roughly equal to  $\sim 1$  standard error of measurement (SEM)
- All are  $\sim$  equal to 0.5 of a standard deviation (SD) of the scale score
- Is this concordance a coincidence, or is it a sign of validity?

## Conclusion?

- Which of these approaches is more readily acceptable and understandable?
- Depends on perspective of user
  - statistician would prefer 0.5 SD or 1 SEM
  - social scientist would prefer ES
  - physician would prefer MID/SSD
  - economist would prefer none of these!

# Advantages of the MID Approach

- Since patients and their health care personnel are the most likely users of HRQOL data, the MID/SSD has advantages in many situations
- It is easily understood by these users (the statistical approaches are not intuitive)
- It allows a calculation of the numbers of patients/people who benefit (and, thus, the NNT for one patient to benefit)



## Disadvantages

- Is the MID, i.e, 6-8% of the scale breadth the same for all scales?
- Does this number need to be ascertained for each questionnaire that is used?
- Is the same true for ES, SEM and SD?  
Probably not.



# Questions

- Can all investigators and clinicians understand the concept of MID and adapt to it?
- Is it a useful concept in economic and population studies?
  - can a QALY be defined in similar terms?
  - the meaning of small changes in QALYs is not certain, despite statistically significant change
  - determining the MID of changes in QALYs by standard gamble techniques is possible

## Questions - cont'd

- knowing the magnitude of a clinically meaningful change in QALYs should allow a calculation of the cost required for one person to benefit from a treatment
- would it help to bridge a gap between policy decision-makers and clinical-decision makers?
- attempt to derive a preference-based measure from the SF-36 (Brazier et al)

## Other Questions

- Will the use of computer adaptive testing (CAT) (based on IRT) require a re-calculation of the MID?
- Will the ES, SEM and 0.5 SD hold up in CAT?
- Will new definitions of meaningful changes in scores be required?

# HRQOL in Clinical Practice

- Still uncommon in routine oncology office
  - Acceptance of HRQOL by clinicians in clinical trials very good
  - For physicians, challenges include:
    - incorporating into clinic work flow
    - resource constraints for data collection and management
    - reimbursement for monitoring HRQOL
- (Donaldson MS, Proc ASCO 2004;23:790 Abstract 8264).

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- For patients, concerns include:
    - acceptability
    - response burden
    - data confidentiality
    - will data influence care?





- Way forward will require:
  - new attitudes re: function of medical records and patient encounters
  - new ways of organizing patient-clinician interactions
  - redesigned system of care that responds to patients needs



## Concluding Remarks

- Much better understanding of the meaningfulness of changes in scores now than 20 years ago
- Are there still more questions than answers?
- Agreement between disciplines on how to proceed may prove to be possible
- Reorganization of clinical practice will take time