



Assessment, analysis and interpretation of Patient-Reported Outcomes (PROs)

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This course is prepared by

Anna Brown, PhD ab936@medschl.cam.ac.uk

Jan Stochl, PhD js883@cam.ac.uk

Tim Croudace, PhD tjc39@cam.ac.uk

(University of Cambridge, department of Psychiatry)

Jan Boehnke, PhD boehnke@uni-trier.de

(University of Trier, Department of Clinical Psychology and Psychotherapy)

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Tim Croudace

1. HEALTH-RELATED QUALITY OF LIFE



Trends and Notions

- Patient based outcome measures are increasingly used
- A range of instruments are available e.g. mental health
- Several reviews of patient reported outcome measures existed, even in 2000: multidimensional measures of functioning of quality of life (cf. symptom measures). Patients perspective includes satisfaction.
- Psychopathology – Syndromes / Severity of Illness
- Psychological Signs – Screening situations
- Relevant populations? Clinical groups
- Health Status measures – Health Care Needs
- Healthy populations – Bartlett and Coles (1998)
- Proxy measures
- General profiles vs Disease specific measures
- Lancashire quality of life profile vs Quality of life in Depression scale



Coming to terms ...

- Patient assessed health outcomes programme
- Patient report health instruments
- Health outcomes

- Health care epidemiology / RCTs
 - Patient perspective: comfort vs cure
 - Patient satisfaction: intervention or services
 - “Patient reported health outcome measures offer a method of assessing the outcomes of care or interventions in terms of: Symptoms / Functioning / Subjective Well-Being or QoL”



Instruments = Measures

- Terms used interchangeable
- Also, in psychometrics
 - Psychological Assessment = Test
 - Test Theory = Psychometric Theory
- A wide variety, when considered on these grounds
 - Number of items/ dimension(s) covered/ mode of administration/use of proxies/ completion time/ populations/training requirements/potential applications



The term PROM

- **Patient Reported (health) Outcome Measures ...** has been used to refer to a variety of:
 - Questionnaires
 - Interviews
 - And other methods of assessing the health of the patients, as seen from the patient's perspective.
- The common feature being that the patient themselves assesses their situation [subjective]



Professional versus Personal

- Seeking areas where objective and subjective evaluations may diverge
- Patient centred
- Subjective evaluation
 - Of Needs
 - Or Outcomes
- Including broader notions than symptoms
 - E.g. satisfaction
- Including general functioning and well-being



Whose views count(ed)?

- Patients should have a say
 - Their perspective and report should be valued
 - Should be allowed to express their opinions
 - Their perspective should be prioritised
-
- Why? They place a greater emphasis on non-clinical (holistic) aspects of treatment than healthcare professionals.



Patient (preferences)

- Self assessment may differ substantially from the judgement of their doctor or healthcare staff
- Preferences for toxic care differ too (taking chemotherapy for little probability of cure, or prolongation of life)
- Observers are poor judges of patient opinion
- Patients opinions vary from expectations of both staff and relatives
 - e.g. observers underestimate the importance of pain/nausea



MRC Methodology underpinning Patient Reported Outcome Measures

- “PROMS are an assessment of health status and health-related quality of life that comes directly from the patient”.
- “Lord Darzi’s NHS Next Stage Review indicates that they will be increasingly used in the evaluation of health care technologies and healthcare services, and they also contribute to regulatory decision making.”
- “Ensuring the validity and robustness of PROMS is therefore vital in enabling measures to have maximum impact on research outcomes and health care decision-making.”
 - www.mrc.ac.uk/Fundingopportunities/Highlightnotices/PROMs/index.htm



WHO Def'n of Health (1948)

- Not merely absence of disease
- A state of complete physical, mental and social well-being
- ... many other definitions of both health and QoL have been attempted, often linking the two, and frequently mentioning **happiness** and **satisfaction** with life



different things to different people

- It is clear that “QoL” means different things to different people and takes on different meanings according to area of application
- Narrow – aspects affected by disease or treatment
- Broad – including indirect consequences of disease, hence health-related quality of life



HRQoL is still a loose definition

- “In the absence of any agreed formal definition most investigators circumvent the issues by describing what they mean (by QoL) and letting the items (questions) in the questionnaire speak for themselves”
- In accordance with current usage, irrespective of the semantic arguments
 - QoL and HRQoL regarded by us as similar or largely overlapping constructs



Contexts

Patient Reported Outcomes Quality of Life

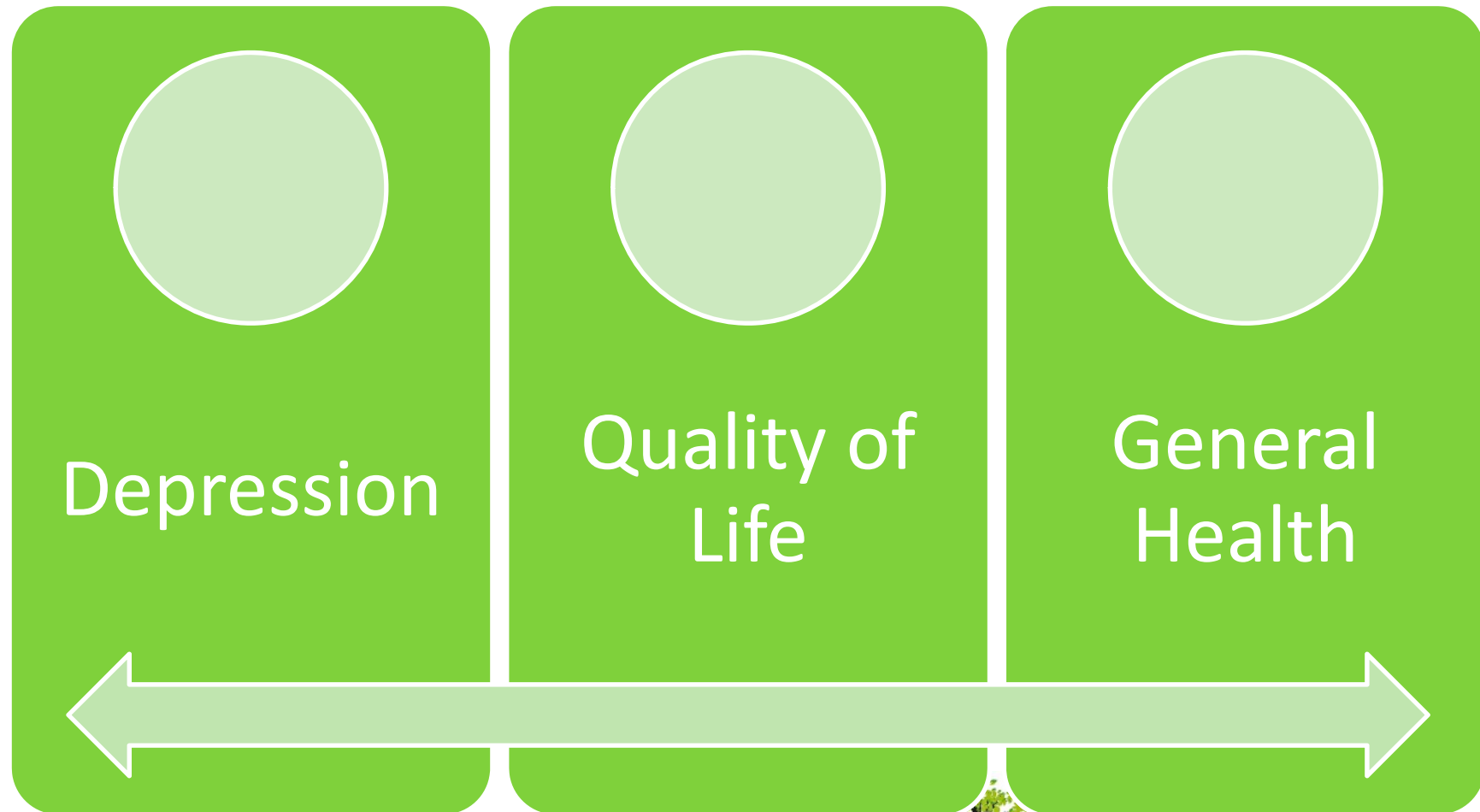
	P	
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Q	O	L

PROMs

- PRO / PROMs
 - A current catch-phrase
- QoL
 - A standard endpoint in RCTs
 - *Versus* the only endpoint in palliative therapy
 - Comfort versus Cure
- Benefits limited v Side-effects



In mental health multi-item scales are already prevalent



5 reasons for the adoption of quality of life measures in mental health

1. Interventions directed towards comfort v cure
 2. Interventions to reduce morbidity are complex
 3. QoL assessments take satisfaction into account
 4. Consistent with WHO's more comprehensive definition
 5. Political correctness
- + they are positively regarded by patients / users
 - + useful in evaluating multidisciplinary interventions
 - + if brief, are inexpensive and easy to introduce



Alliteratively Ps

- **P**atient:
 - Syndrome
 - Symptom/Severity
 - Satisfaction
- **P**erson (healthy)
 - Screening symptoms
 - Subjective signs (psychological distress)
 - Senstations
- **P**roxy
 - PS. Spiritual aspects ...?



One unifying and non-controversial theme throughout all approaches ...

- Is that the concepts can be assessed only by subjective means
- Proxy assessments are only usually employed if the patient is unable to make a coherent response. For example, those with:
 - mental impairment
 - The very young, very old or most severely ill or disturbed

NEVERTHELESS

many individual concepts (e.g. emotional functioning) lack a formal agreed definition that is universally understood



Randomized Controlled Trials

- A key methodology for evaluation of therapies
- Traditionally considered in terms of relatively objective outcomes: mortality & morbidity
 - cure
 - biological response
 - survival
- More recently greater emphasis on subjective indicators/self-report (including single items)
 - Physical and Social Functioning
 - Emotional functioning / adjustment
 - Psychological Distress / Anxiousness / Low Mood / Fatigue
 - Pain e.g. headache
 - Other symptoms: nausea/vomiting (toxicity side-effects)



Why measure QoL? [in RCTs]

- Reasons for including QoL items depend on trial type: curative vs. palliative, rehabilitation
- Cancer/HIV/AIDS: Treatment of fatal diseases can and often does result in limited gains in cure or prolonged survival
- + therapeutic interventions in these areas frequently cause serious side effects and functional impairment
 - Therapies that are indistinguishable in terms of **clinical assessment** (efficacy/safety) may produce substantial changes in Quality of Life.
 - There are numerous examples in which QoL have had an unexpectedly important role in the interpretations and conclusions of RCTs
 - *The cure might be worse than the disease (in some cases)*



Which trials should assess QoL?

- QoL may be the main endpoint [incurable disease, palliative care, seriously ill]
- Treatments expected to be equivalent in efficacy and a new treatment would be deemed preferable if it confers QoL benefit(s)
- A new treatment may show a small benefit in cure or survival but this might be deemed preferable if it confers QoL benefits
- Treatments may differ considerably in their short term efficacy, but if the overall failure rate is high, then QoL issues should be considered



QoL can predict ...

- Medical Decision Making / Prognosis
- There may be some prognostic importance of PROMs (in RCTs, from baseline measures)
- It is not clear in these circumstances whether QoL scores reflect an early perception by the patient of disease progression, or whether quality of life status in some way influences course of disease ...



Timing and Technological Developments

- PRO movements has invigorated clinical epidemiology with psychometric research
- Technologies developed in education testing translated into health assessment and healthcare evaluation
- Similar concerns: item-level data, binary/ordinal ratings, multiple constructs/latent dimensions
- Desire to shorten while retaining validity and not compromising reliability; also longitudinal use....



Looking ahead

- To be suitable for potential applications ...
- PROMs must have evidence of:
 - **Reliability** – are scores reproducible (internally consistent?)
 - **Validity**- whether an instruments measures what is intended
 - **Responsiveness**- whether an instrument is responsive to important changes in health



Book by Fayers & Machin “*The assessment, analysis and interpretation of PROs*”

- A practical guide to PRO & QoL
- A non-technical intro to less familiar methods
- Assuming some statistical familiarity, but avoiding unnecessary statistical detail
 - Based on experience in Cancer RCTs – but the issues that arise are common to QoL assessment in general

