

# **Individualised Patient Outcomes**

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# What are Individualised Patient-Reported Outcome Measures (iPROMs)?

- Like traditional PROMs, they are instruments (usually questionnaires) that measure health status and related concepts such as quality of life.
- Unlike traditional PROMs, they allow each respondent to individually define the domains and weights to be assessed within the questionnaire.
- Also called 'Patient Generated Outcome Measures'.

# Why do we need iPROMs?

- Traditional PROMs do not capture the uniqueness of individuals.
- Instead, they capture the individual's perception of his or her health/QoL through the lens of a standardised model of 'the good life' using pre-selected domains/weights.
- This is criticised because:
  - Many of the original PROMs such as EQ-5D, SF-36 were designed with little input from patients.
  - Individual definitions of health/QoL are posited to be highly heterogeneous and idiosyncratic, meaning that very few patients fit the 'average' definition.

# Other critiques

- Measures like the Oxford Hip Score which were developed using classical psychometric methods cannot be used to pinpoint individual patients who have had good or bad outcomes.
  - data is only usable at group level.
- The experience of completing and interpreting the traditional measures, especially generic measures, has been described as artificial and lacking face validity by many patients and clinicians.

# Individual level meaning is important!



# EQ-5D

- 5 domains: mobility, self care, usual activities, pain/discomfort, anxiety/depression.
- Responses on each domain are weighted and summed to produce an overall score where 1 = perfect health.
- **Mobility**
  - I have no problems in walking about
  - I have some problems in walking about
  - I am confined to bed

# Where do the EQ5D domains come from?

“... in 1987, with Rosser and Kind, I gathered together some interested researchers in Europe to see whether, by pooling our knowledge and expertise, we could identify a “common core” of some three or four key elements that most health status indexes contained, and that were presumably regarded as important in determining whether someone’s health was getting better or not. The goal of this “Common Core Group” was to identify an essential minimum data set, **not to produce a comprehensive measure containing every item that might be considered relevant by someone or other.** Being both multi-national and multi-disciplinary, and having in mind the different purposes to which such a measure might be put, our early discussions were difficult and often tense, but we stuck it out and slowly learned to understand and accept each other’s conceptual frameworks and modes of thought and expression. The miracle is that in so doing we did not fight shy of forthright argument, and often made quite severe criticisms of each other’s positions, a tradition which persists within the group, now known as the EuroQol Group. From that difficult gestation period emerged the EQ5D descriptive measure of health-related quality of life, which is now widely used worldwide, and is the most commonly used measure in QALY calculations for the National Institute for Clinical Excellence (NICE).”

*Discovering the QALY: or how Rachel Rosser changed my life. Alan Williams in personal histories in health research, ed. Adam Oliver. Nuffield Trust. London 2005.*

**What happens if you ask people which domains are important to QoL?**

Depends on who you ask.

# Self-generated domains: Children with Cerebral Palsy

- Family
- Friends
- Pets
- Hobbies
- Physical play
- Physical health
- School/education
- Religion
- Other play
- Travel

*Vinson et al. J Dev Phys Disabil. 2010;22:497-508.*

# Self-generated domains: older people in care homes

- Leisure activities
- Family
- Relationships
- Social life
- Independence
- Peace/contentment

*Hall et al. Assessing quality of life in older people in care home. Age and Ageing 2011.*

# Healthy older and younger people

Domain	Healthy young adults	Healthy older people
Family	62%	89%
Social & Leisure activities	95%	38%
Health	91%	83%
Living conditions	80%	21%
Religion	75%	7%
Independence	19%	16%
Finances	60%	25%
Relationships	86%	18%
Work	38%	5%

*Browne et al. Quality of Life Research, 1994.*

# Where do the EQ5D weights come from?

- Derived from studies of population preferences for different life states.
- Preferences are derived from discrete choice methods (e.g. Time Trade Off).
- Weights are the averages preferences for different life states, derived from the whole sample.
- Weights (also known as 'value sets') are available for a number of different countries.

# What does this mean in practice?

- Mobility = no problems (1)
- Self-care = no problems (1)
- Usual activities = some problems (2)
- Pain/discomfort = moderate (2)
- Anxiety/discomfort = moderate (2)
  
- UK average value = 0.689 (TTO method)
- US average value = 0.768 (TTO method)

# Heterogeneity of preferences?

Health state	Median	IQR
21111	0.950	0.869-1.000
12332	0.500	0.256-0.675
23222	0.525	0.375-0.725
32211	0.488	0.175-0.563
32323	0.025	-0.500-0.500

*Bansback et al. Canadian Valuation of EQ-5D Health States. PlosOne 2012*

# What happens if you let people weight the domains they have chosen?

**SEIQoL weights for different domains provided by healthy adults**

<b>Domain</b>	<b>Weight</b>	<b>Range</b>
Physical function	0.22	0.03-0.38
Social function	0.14	0.05-0.44
Emotional function	0.24	0.02-0.46
Living conditions	0.14	0.02-0.30
General health	0.26	0.09-0.54

# Heterogeneity (or variability) undermines nomothetic research

- “When data are averaged over all the subjects... composite data are regarded as though they were representative of one ideal subject... generality and variability are basically antithetical concepts. If there are major undiscovered sources of variability in a given set of data any attempt to achieve subject or principle generality is likely to fail”

*Sidman (1960). Tactics of scientific research. New York: Basic Books.*

# The idiographic tradition

- Idiographic tradition in the social sciences assumes that for many important phenomena (e.g. experiencing quality of life) individuals cannot be described using general rules because of the complexity of each life history.
- Autobiographical methods considered more appropriate.
- Introduced by Gordon Allport in 1930s and applied most intensively in the field of personality psychology.
- Adopted in 1960s by psychologists working within 'phenomenological' tradition (e.g. George Kelly, Carl Rogers).

# Which iPROMs have been tried in the past?

- Four measures identified by Patel et al that (i) let patients define domains and (ii) produce an overall score as opposed to profile.
- SEIQoL
- PGI
- Repertory grid
- Asthma Quality of Life Questionnaire

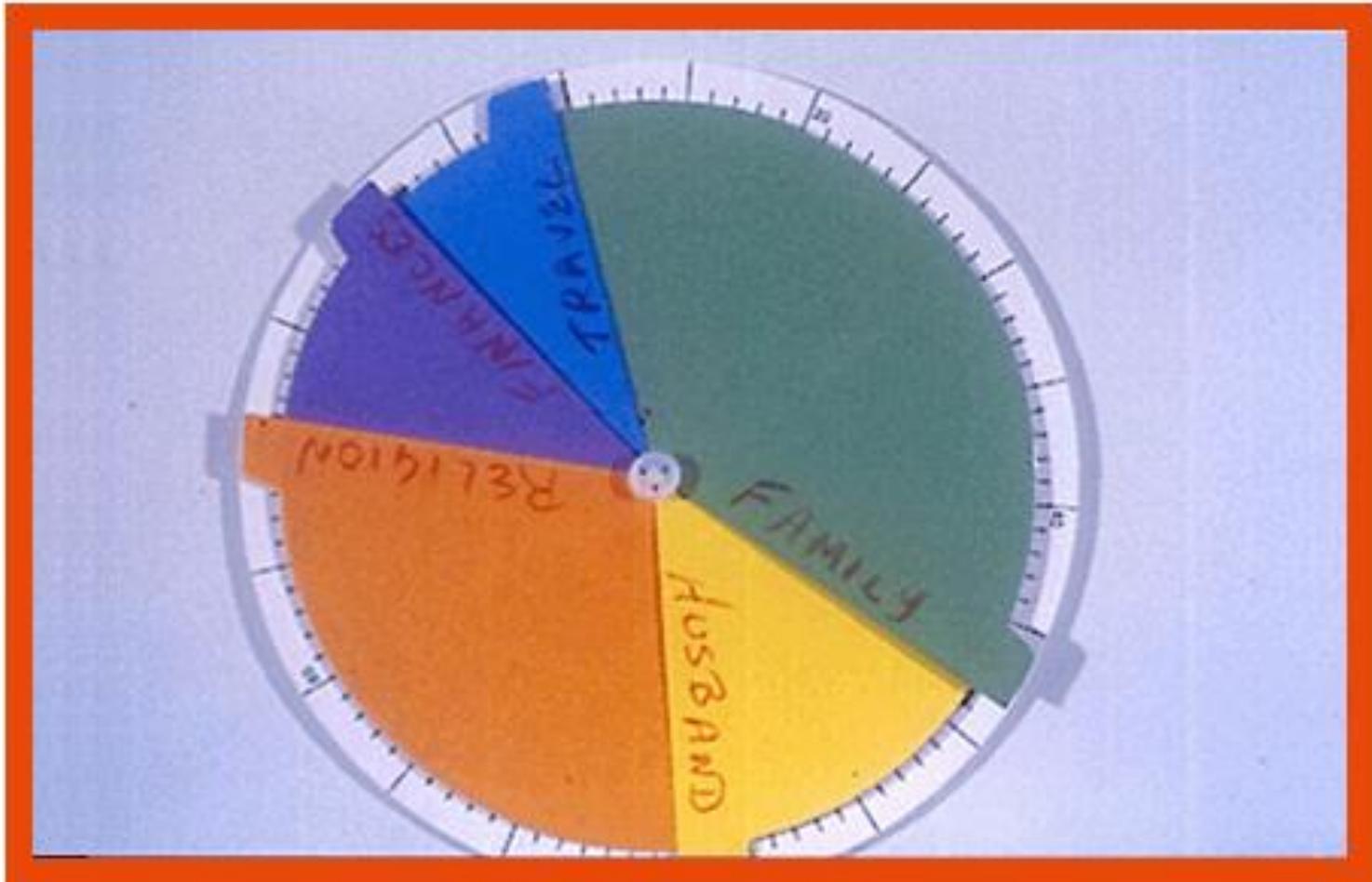
*Patel et al. Value in Health 2003;6:595-602.*

# Schedule for the Evaluation of Individual Quality of Life (SEIQoL): O'Boyle et al 1991

- Respondents nominate the 'top five' domains of most relevance to their quality of life.
  - Usually done in semi-structured interview
- Each domain is then rated on a 0-100 VAS anchored from 'worst possible' to 'best possible'.
- Each domain also weighted using a direct relative weighting method to produce weights that sum to 1.0.
- SEIQoL index =  $\Sigma$  (levels x weights): ranges from 0-100.

# Direct weighting method

图 3



# Patient Generated Index (PGI): Ruta et al 1994

- Patients asked to list the five most important domains affected by their condition. Also given a list of the areas most frequently mentioned by patients with the same condition.
- A sixth domain represents all other aspects of life that are not captured in the first five areas.
- Patients rate how their current condition matches their expectations in each of the six areas of life using a scale of 0 to 10.
- The six domains are weighted by distributing 12 points, these weights are then converted to a 0-1 scale.
- PGI index =  $\sum (\text{levels} \times \text{weights})$ : ranges from 0-10.

# Repertory Grid: Thunedborg et al 1993

- Complex technique based on in-depth interviews and factor analysis: adapted from a method developed by George Kelly to identify ways that individuals construe their experiences.
- Patients provide some domains (known as 'elements') and some are provided for them.
- Patients also provide 'constructs' and some are provided for them. Constructs allow the patients to tell us how they make sense of the construct (e.g. good/bad).
- The relationship between elements and constructs is then assessed and the scores are modelled using factor analysis.
- Produces a QoL index on a 0-100 scale.

# Asthma Quality of Life Questionnaire: Juniper et al 1991

- Four defined domains: physical activities, emotions, environmental stimuli, emotional function.
- All domains contain predefined items (ie questions).
- The physical activity domain also includes five individualized questions.
- Patients are asked to list activities in which they were limited by asthma in the past 2 weeks, and 26 activities are offered as probes.
- Patients then choose the 5 activities that are most important to them.
- Patients then rate the extent to which they have been limited in that activity during the past 2 weeks using a 7-point Likert scale.
- The AQLQ is a nonweighted instrument: all the items are equally weighted.
- Overall QoL score equals the mean score for all the items across all domains.

# Is there any evidence that iPRoMs are useful?

1. Can they improve 'group level' comparisons – e.g. detecting treatment effects, or quality of care differences between healthcare providers?
2. Can they improve clinical care at the individual clinician-patient level?

# Problems with group level use of iPRoMs

1. Practical issues (time, interviewer administration)
2. Profiles can't be used for comparative research, indices lose the rich idiographic material.
3. Weighting makes no difference (weighted/unweighted indices correlated  $> 0.95$ )
4. No more responsive than traditional measures.
5. Focus is on domains that are often distal in the causal pathway to intervention benefits.
6. Ignores improvements in modern psychometric methods (e.g. Rasch, IRT)
  - ✓ Use of qualitative research with patients to generate content.
  - ✓ Latent trait methods allow us to explore 'fit' between patient responses and the nomothetic model being used.

# Responsiveness of SEIQOL in hip replacement surgery

- Cohen's effect sizes (mean change/baseline SD) after hip replacement.
  - SEIQoL = 0.48
  - EQ5D = 1.3
  - Oxford Hip Score = 2.5

*Browne et al 2007, Report to DH.*

*O'Boyle et al 1992, Lancet;339:1088-91.*

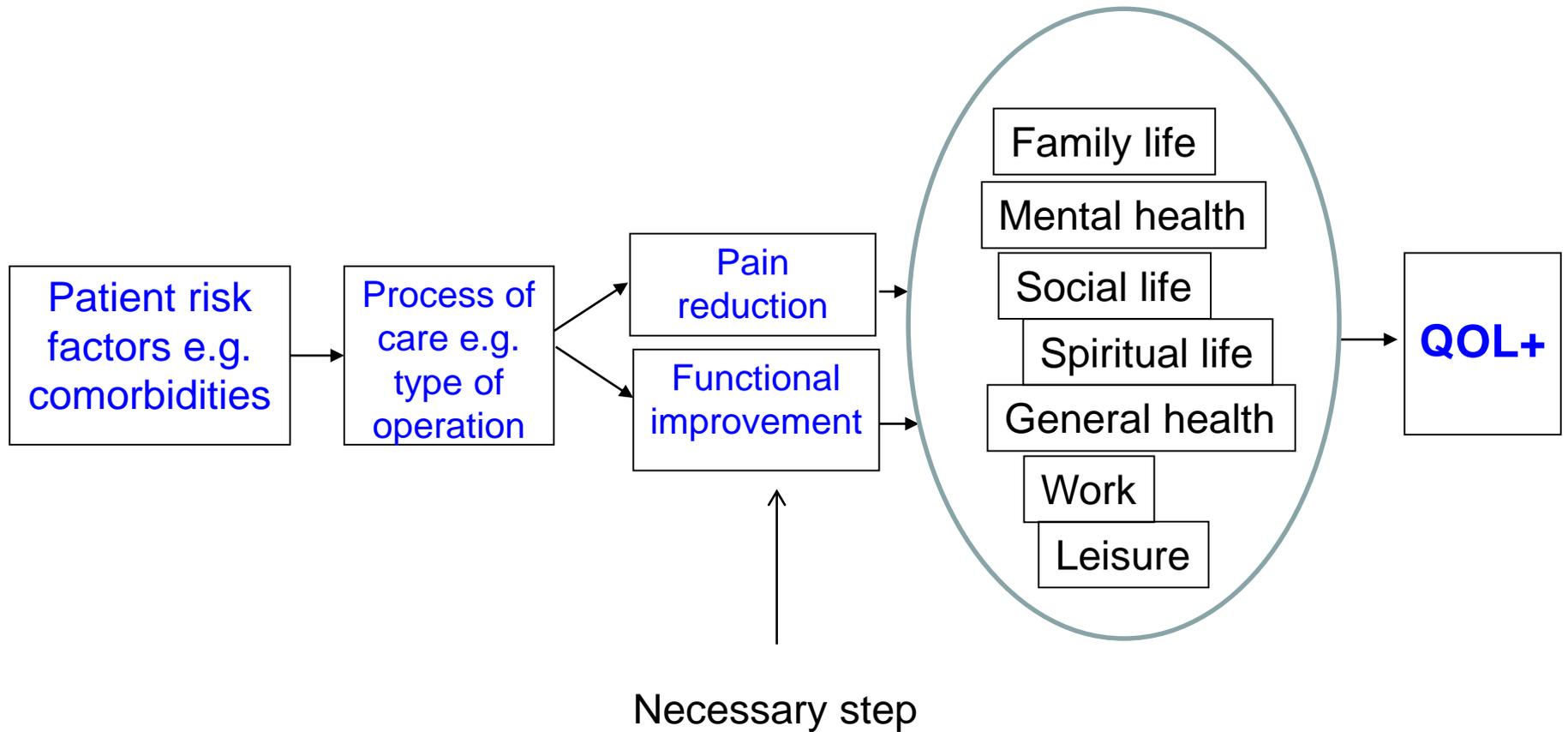
# Responsiveness of PGI

- Condition-specific instruments better able to capture change than PGI in low back pain, menorrhagia, suspected peptic ulcer, and varicose veins.
- PGI was comparable to SF-36 and better than EQ5D for patients being treated for obstructive sleep apnoea.

*Ruta DA, Garratt AM, Russell IT. Patient centered assessment of quality of life for patients with four common conditions. Qual Health Care 1999;8:22–9.*

*Jenkinson C, Stradling J, Petersen S. How should we evaluate health status? A comparison of three methods in patients presenting with obstructive sleep apnoea. Qual Life Res 1998;7:95–100.*

# Simplified causal pathway: QoL improvement after hip surgery



# What about individual patient-clinician level uses?

- There is a strong desire for PROMs that support individual patient care rather than only serve the needs of policy makers/purchasers.
  - Highlighting previously unrecognised health problems.
  - Measuring the effectiveness of different treatment plans.
  - Monitoring disease progression.
  - Stimulating better communication.
  - Promoting shared decision making.
- Traditional PROMs have been tried in this context but the evidence re effectiveness is mixed.

# **Does providing feedback on patient-reported outcomes to healthcare professionals result in better outcomes for patients? A systematic review.**

**Boyce and Browne, Quality of Life Research (2014)**

- 16/17 studies focused on individual level feedback.
- Only one study found a significant effect in favour of PROMs feedback: rest found no effect.
- Studies are methodologically weak.

“Qualitative research is required to provide a deeper understanding of the practical issues surrounding the implementation of PROMs and the methodological issues associated with the effective use of the information.”

## **The experiences of professionals with using information from patient-reported outcome measures to improve the quality of healthcare: a systematic review of qualitative research.**

Boyce, Browne, Greenhalgh. *BMJ Quality and Safety* (2014)

- Practical problems (technical support, workload)
- Negative attitudes (suspicion of managerial objectives, concerns about impact on patient-clinician relationship)
- Methodological concerns (validity, interpretability)
- Doubts about impact (cannot turn the data into practical solutions).

# Tentative evidence that iPROMs may be useful in some contexts

“...to fully understand [care home] residents' QoL, detailed interview-based instruments, administered by an experienced interviewer are needed.”

*Hall et al. Age and Ageing 2011;40:507-12.*

”It was found [in palliative care] that SEIQoL-DW provided an opening or 'window' onto participants' QoL as they travelled from their past to their future.”

*Crang et al, In J Palliat Nurs. 2008;14:90-7.*

”individual QOL appears to be largely independent from physical function in severely ill patients with ALS. Because of the characteristics of the scale, assessment of individual QOL may have an interventional value in palliative care.”

*Neudert et al. J Palliat Med. 2004 Aug;7(4):551-7.*

# Conclusions

- “... patient-generated outcome measures, although conceptually appealing, may not be appropriate as sole measures of HRQoL in a clinical trial setting... their most important role may not be in a clinical trial setting, but in the consultation process, where the practitioner can formulate and monitor therapeutic plans based on the life areas elicited by the patient.”

*Patel et al. Value in Health 2003;6:595-602.*