*Quality of Life as a criterion for decisions regarding marketing authorization, therapy and reimbursement*

# Measuring Quality of Life : lessons from the past – prospects for the future

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# Überblick

- Where have we come from ?
- Where are we now ?
- What are the key issues ?
- A way forward

# Starting point

- No health care system can provide treatment for all patients, with all conditions, for all time
- Our collective capacity to innovate, our rising expectations of what health care can provide and steady extension in our life expectancy, place increasing demands on limited health care resources
- We need a decision-making framework in which to consider how best to use those resources

# Value and health

- The value of health underpins and guides all aspects of healthcare decision-making
  - the delivery of health care to individuals,
  - the formulation of health policies and programmes for society,
  - the development of new health technologies
- It is central to the assessment of both effectiveness and cost-effectiveness
- And paradoxically, it provides the common language that links clinicians, health economists and regulatory decision-makers

# Health outcomes

- How we choose to measure health outcome expresses a judgement about what WE think matters and what does not
- Exclusions do count – they all carry an imposed value of zero
- Hence the need to standardise the way that we quantify health outcomes and the growth in interest over the past 25 years in the what we call "quality of life" measures

# IQWiG and value

Economics deals with the ***value*** of resources used and of the outcomes produced. Given the use of money as the currency of value in the marketplace, the term *value* is often misunderstood as something that necessarily has to do with money. It does not. It has to do with "the regard that something is held to deserve, its importance or worth".

# Measuring "quality of life"

- Health status measurement in its current form, dates back more than 35 years
- Motivation for development of these measures was the need to go beyond clinical parameters
  - Global / generic measures
  - Condition-sensitive measures
- Now generally referred to as measures of health-related quality of life (HrQoL)

# Principal generic measures

## PROFILE MEASURES

- Sickness Impact Profile (SIP)
- Nottingham Health Profile (NHP)
- SF-community
  - RAND MOS SF-36
  - SF-20 / 12 / 8 / 2
- WHOQOL

## INDEX MEASURES

- QWB
- Rosser-Kind Index
- 15-D
- HUI cluster
  - HUI II and III
- EQ-5D
- AQLQ
- YHL
- SF-6D

# Valuing "health"

- An issue that is not restricted to regulatory agencies or health economists
- It is a major issue for clinicians too
- Health economists makes life difficult by requiring that "utility" weights are used for QALY calculations
- Clinicians make life difficult by generally ignoring the issue altogether

# Karnofsky Performance Scale

| Description | Score |
|---|---|
| Normal | 100 |
| Normal activity ; minor signs / symptoms | 90 |
| Subnormal activity ; some signs / symptoms | 80 |
| Unable to work or to continue normal activities | 70 |
| Requires occasional assistance | 60 |
| Requires considerable assistance and frequent care | 50 |
| Disabled ; requires special care | 40 |
| Severely disabled ; hospitalised | 30 |
| Very sick ; hospitalised with active support treatment | 20 |
| Moribund | 10 |
| Dead | 0 |

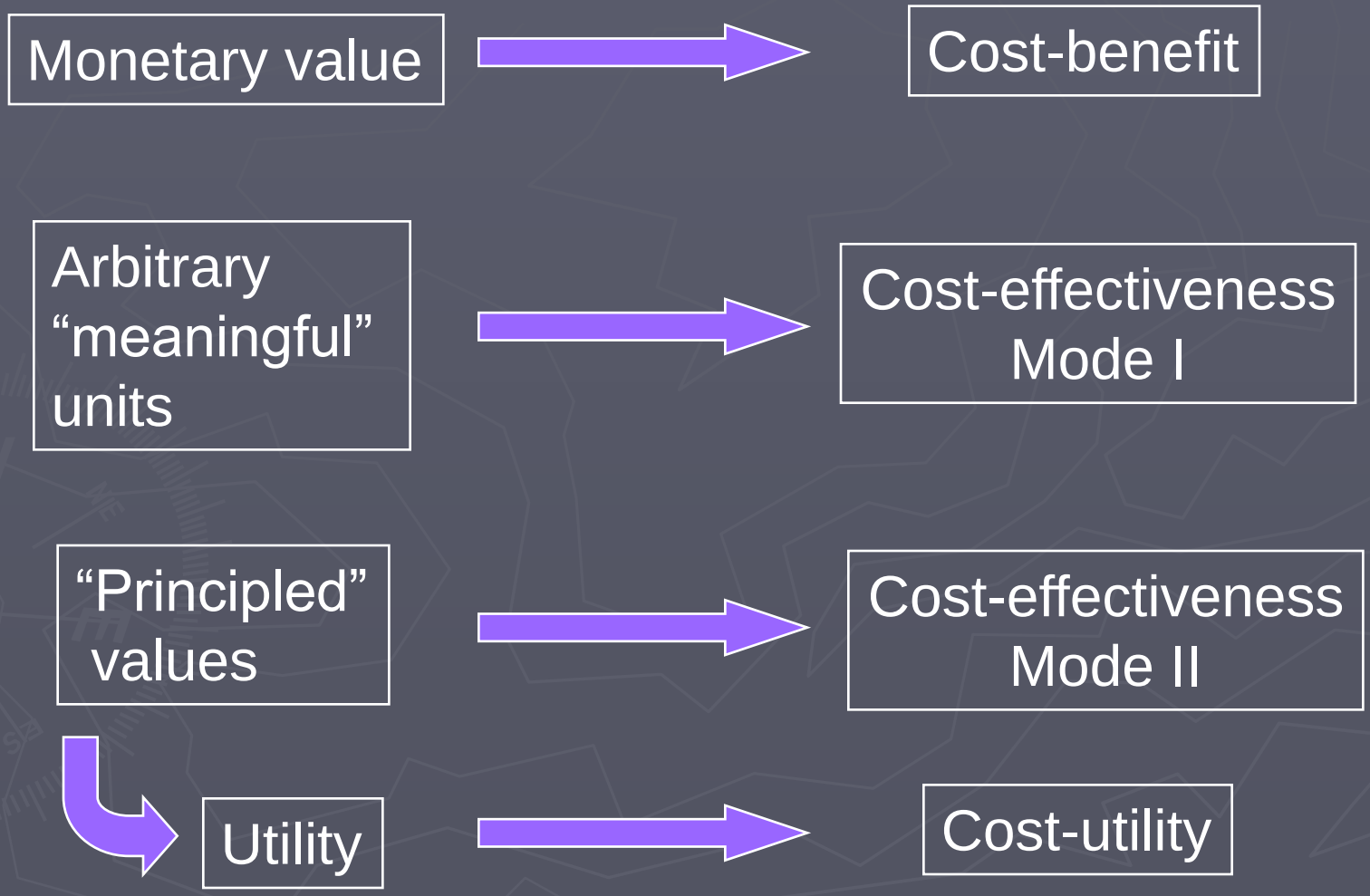
# FACT-L

| | PHYSICAL WELL-BEING | Not at all | A little bit | Some-what | Quite a bit | Very much |
|---|---|---|---|---|---|---|
| GP1 | I have a lack of energy | 0 | 1 | 2 | 3 | 4 |
| GP2 | I have nausea | 0 | 1 | 2 | 3 | 4 |
| GP3 | Because of my physical condition, I have trouble meeting the needs of my family | 0 | 1 | 2 | 3 | 4 |
| GP4 | I have pain | 0 | 1 | 2 | 3 | 4 |
| GP5 | I am bothered by side effects of treatment | 0 | 1 | 2 | 3 | 4 |
| GP6 | I feel ill | 0 | 1 | 2 | 3 | 4 |
| GP7 | I am forced to spend time in bed | 0 | 1 | 2 | 3 | 4 |

# Fundamental questions

- WHO should describe and value health ?
- HOW should health be described and valued ?
- HOW should such information be represented in aggregate ?

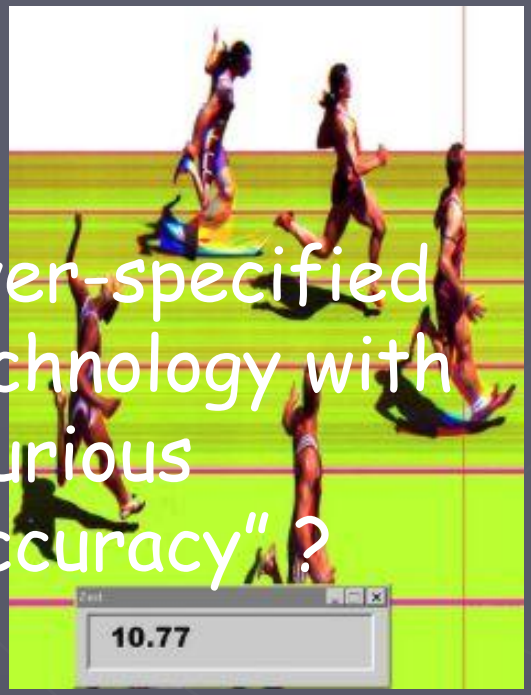
# Valuation metrics



# Valuation methods

- ordinal judgement
  - sorting
  - ranking
  - category rating
  - paired comparisons
- cardinal judgement
  - magnitude estimation
  - equivalence scaling
  - visual analogue scale (VAS) rating
- utility measurement
  - standard gamble
  - time trade-off
- 'revealed' valuations
  - legal awards
  - health insurance
- stated preference
  - willingness-to-pay

Over-specified technology with spurious "accuracy" ?





Complex measurement task requiring high degree of accuracy

# Key issues for debate

How should the value of health benefits be counted / valued in economic evaluation ?

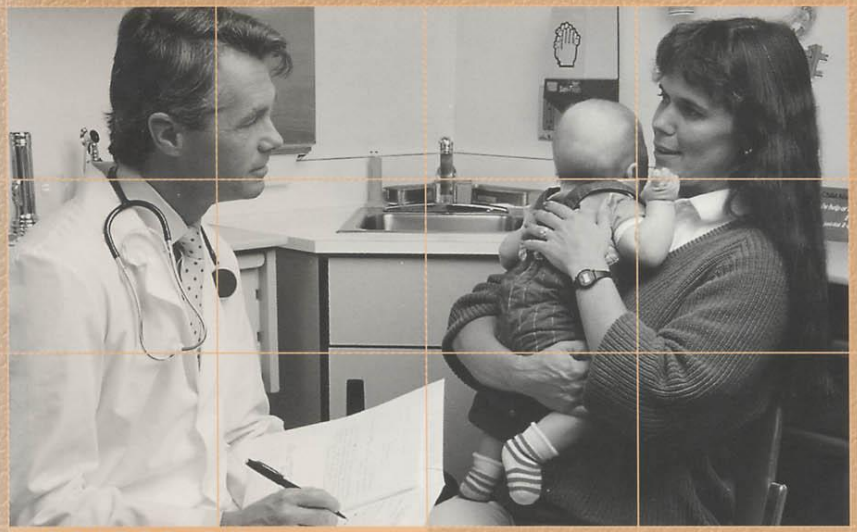
Should the requirements of economic evaluation dominate other uses of the (same) health outcomes data ?

Gold
Siegel
Russell
Weinstein

COST-EFFECTIVENESS in HEALTH and MEDICINE

OXFORD

# COST-EFFECTIVENESS in HEALTH and MEDICINE



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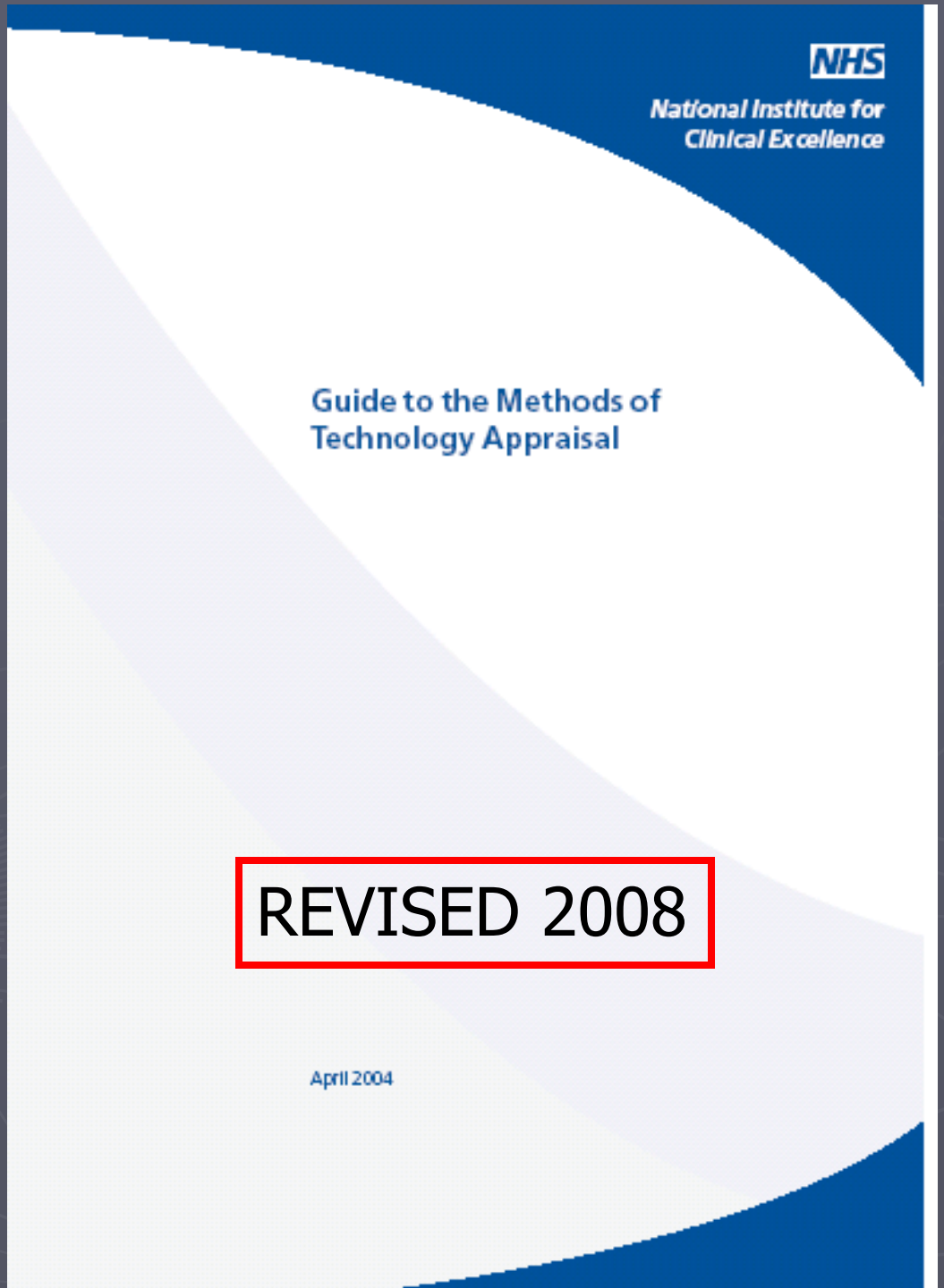

NHS
National Institute for Clinical Excellence
Guide to the Methods of Technology Appraisal
REVISED 2008
April 2004

| Element of health technology assessment | The Reference case |
| --- | --- |
| Measure of health benefits | QALYs |
| Description of health states for calculation of QALYs | Health states described using a standardised and validated generic instrument (#5.5.3) |
| Method of preference elicitation for health state valuation | Choice-based methods, for example time trade-off or standard gamble, not rating scale |
| Source of preference data | Representative sample of the general public |

| Element of health technology assessment | The Reference case |
| --- | --- |
| Measure of health benefits | QALYs |
| Description of health states for calculation of QALYs | EQ-5D |
| Method of preference elicitation for health state valuation | TTO |
| Source of preference data | Representative sample of the general public |

# What's the PROblem with the PRO ?

- The PRO is essentially the solution to a problem encountered by the US FDA
- That problem centred on the issue of "validating" quality of life claims made in patient information leaflets and in other promotional material
- The crux of the problem was
  - HOW DO WE DEFINE QUALITY OF LIFE ?
- The PRO solution has not resolved this

# What is a PRO?

- *A PRO is a measurement of any aspect of a patient's health status that comes directly from the patient (i.e. without the interpretation of the patient's responses by a physician or anyone else).*

Draft Guidance for Industry on Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims. US FDA, 2006

Patients may be the source, but virtually ALL such responses are coded using a value system that modifies those responses

# FDA draft guidance is wholly silent on the issue of VALUATION

# A warning from the past

- The present situation in the UK in which a "standard" methodology is applied as the base (reference) case analysis relies upon a measurement system (EQ-5D) that has evolved over more than 2 decades
- Some of us were arguing for the standardised measurement of health status (HrQoL) more than 30 years ago
- Why do you need more time ?

# Decide what's best for YOU

*what question are you seeking to answer*

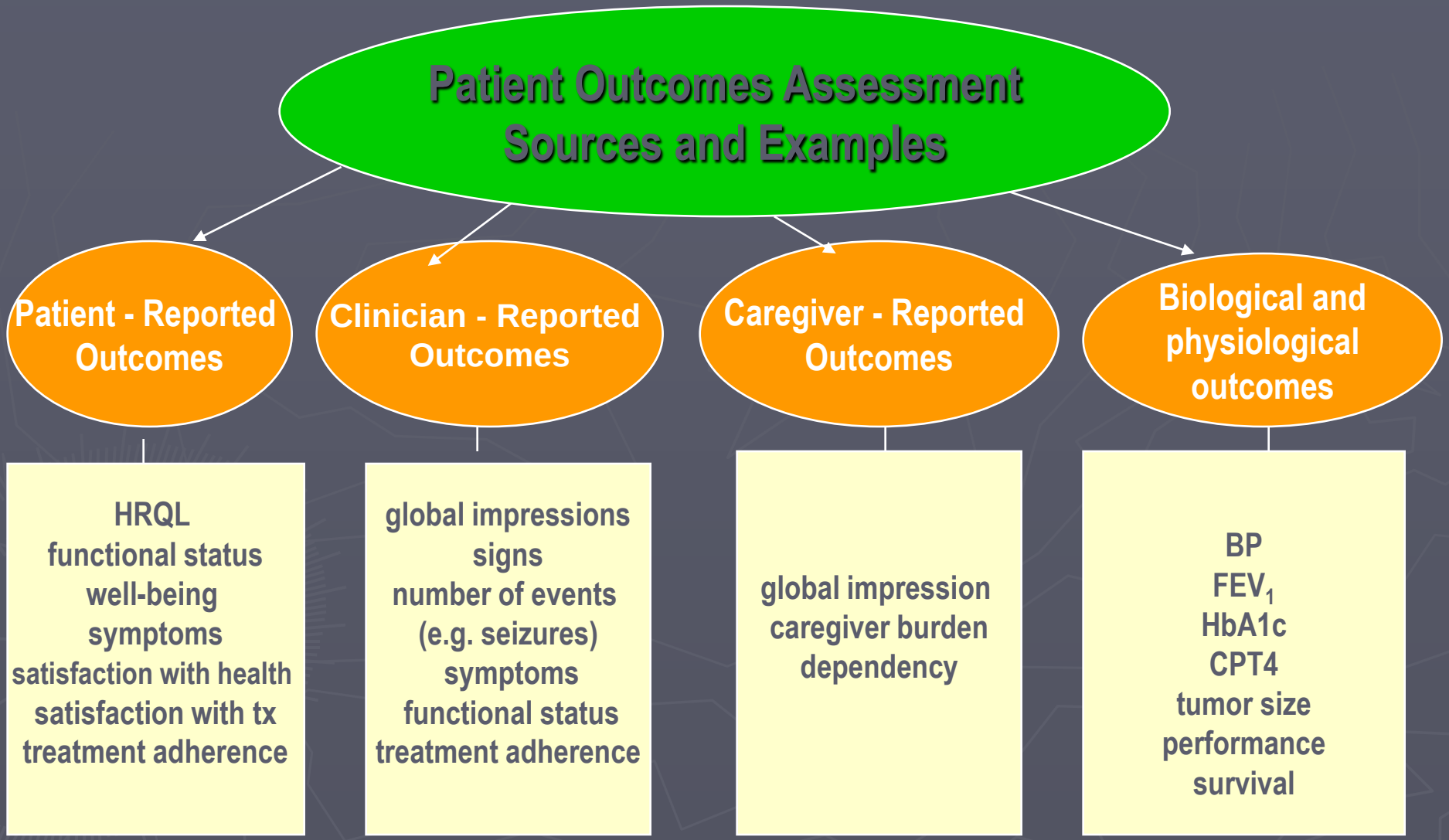
- Health economists should adopt methods that are "fit for purpose" and appropriate for the healthcare system that they wish to advise
- Cost-utility analysis methods may be appropriate where social decision-making is the dominant application
- Other methods of valuing outcomes may be more appropriate where behaviour in a health market operates differently or to inform other forms of decision

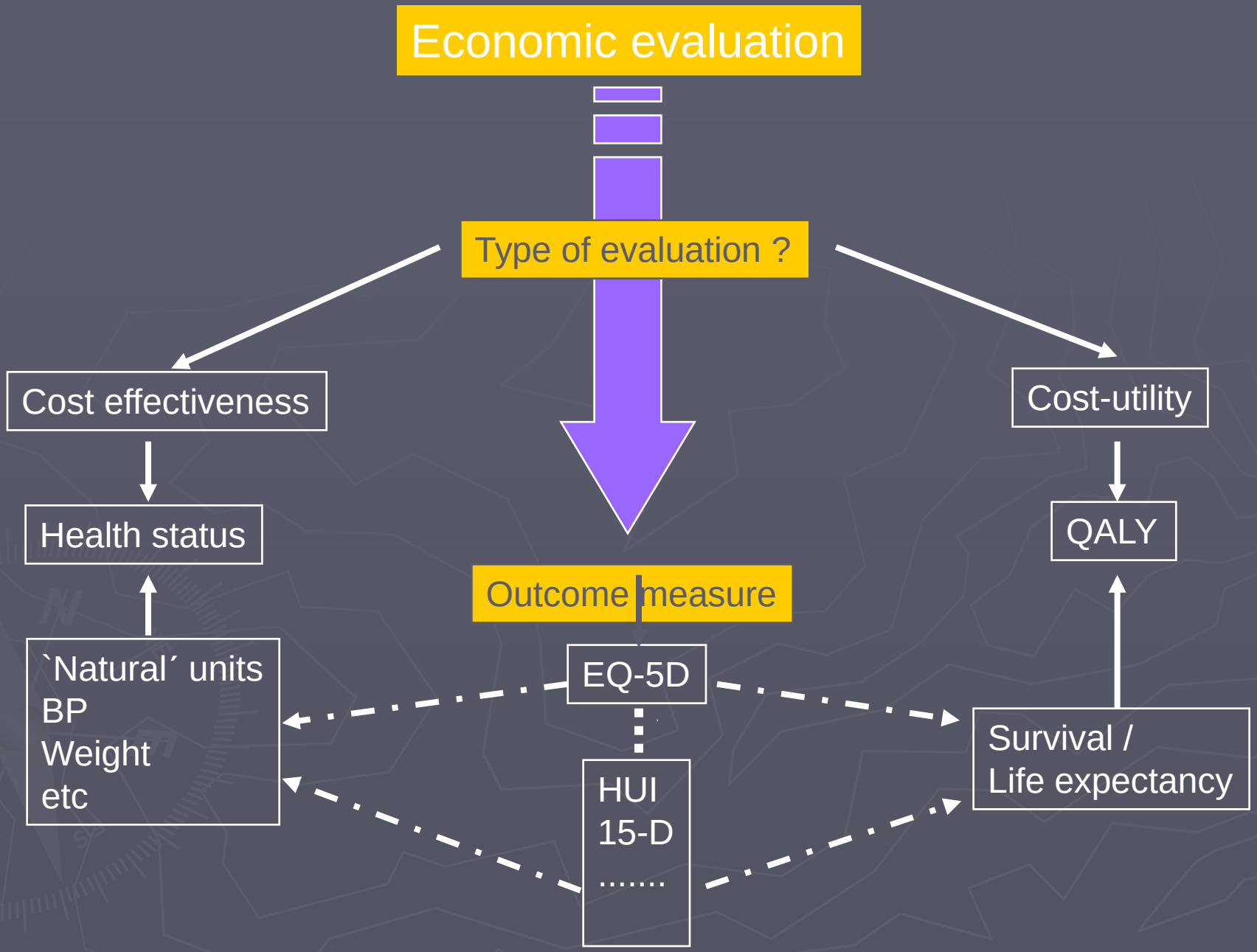
# And the future ?

- Doing nothing is NOT an option
- We do not have the luxury of waiting for a new methodology to come along and gain acceptance - the issues facing Society are NOW and current methods may not be perfect but they are available and can help
- We should at least invest in finding out how far those methods can provide real assistance and what (if anything) needs to be done to improve them

The best way to influence the future is to write it yourselves

# Mk I FDA / PRO model




Economic evaluation
Type of evaluation ?
Cost effectiveness
Cost-utility
Health status
QALY
Outcome measure
`Natural´ units
BP
Weight
etc
EQ-5D
HUI
15-D
.....
Survival /
Life expectancy