

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

o health care system can provide treatment
or all patients, with all conditions, for all
me

ur collective capacity to innovate, our rising
expectations of what health care can provide
nd steady extension in our life expectancy,
ace increasing demands on limited health
are resources

ve need a decision-making framework in
hich to consider how best to use those

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-

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,

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

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

arnofsky 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

FACT-L

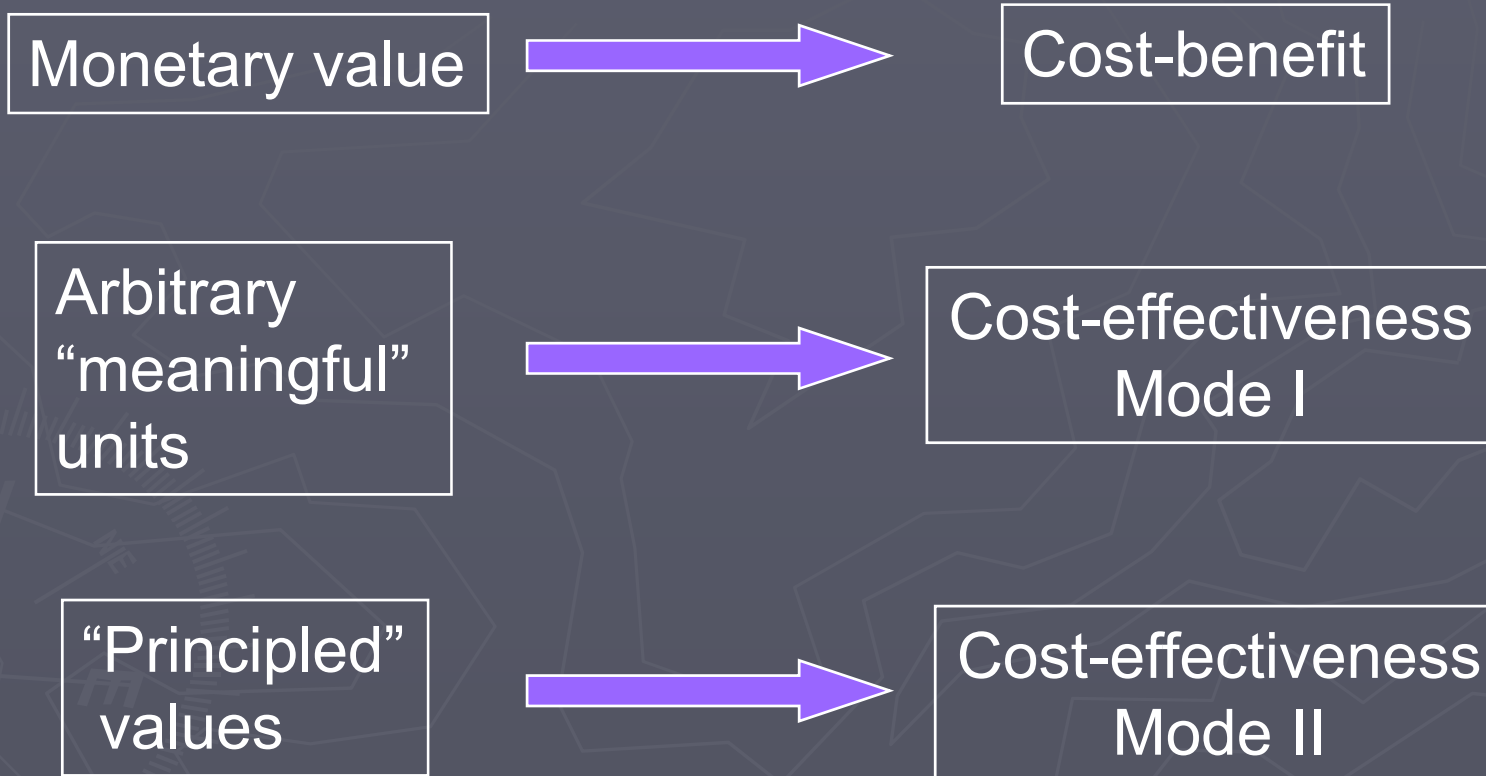
PHYSICAL WELL-BEING

	Not at all	A little bit	Some- what	Quite a bit	Very much
have a lack of energy	0	1	2	3	4
have nausea	0	1	2	3	4
because of my physical condition, I have trouble meeting the needs of my family	0	1	2	3	4
have pain	0	1	2	3	4
am bothered by side effects of treatment	0	1	2	3	4
feel ill	0	1	2	3	4
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

▶ utility measurement

- standard gamble
- time trade-off

▶ 'revealed' valuations

- legal awards
- health insurance

▶ stated preference

- willingness-to-pay



Complex measurement task requiring high degree of accuracy



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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

COST-EFFECTIVENESS in HEALTH and MEDICINE



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**Guide to the Methods of
Technology Appraisal**

REVISED 2008

April 2004

Measurement 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

Comparison of health technologies	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?

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 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

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

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

Urgent and current methods may not be

perfect but they are available and can help

We should at least invest in finding out how

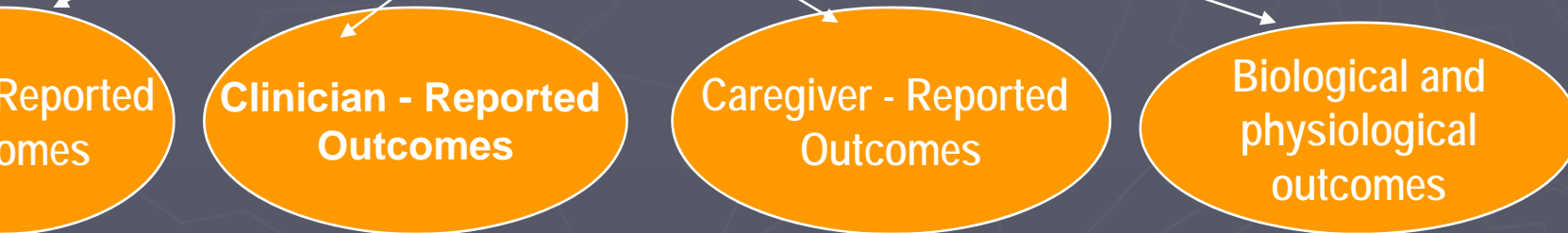
those methods can provide real assistance

and what (if anything) needs to be done to

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

Mk I FDA / PRO model

Patient Outcomes Assessment Sources and Examples



QOL
functional status
ability to perform
being
symptoms
ability to manage health
ability to manage health with tx
adherence

global impressions
signs
number of events
(e.g. seizures)
symptoms
functional status
treatment adherence

global impression
caregiver burden
dependency

BP
FEV₁
HbA1c
CPT4
tumor size
performance
survival

Economic evaluation

Type of evaluation ?

Cost effectiveness

Cost-utility

Health status

QALY

Natural units
Weight
c

Outcome measure

EQ-5D

Survival /
Life expectancy

HUI
15-D