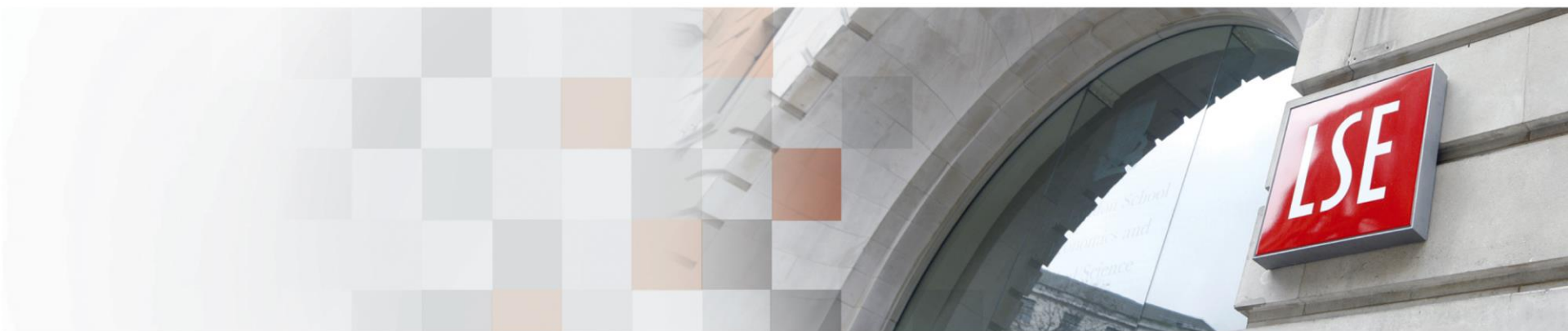


Patient engagement and health economics

Panos Kanavos
London School of Economics
Brussels, 1 June 2016



THE LONDON SCHOOL
OF ECONOMICS AND
POLITICAL SCIENCE ■

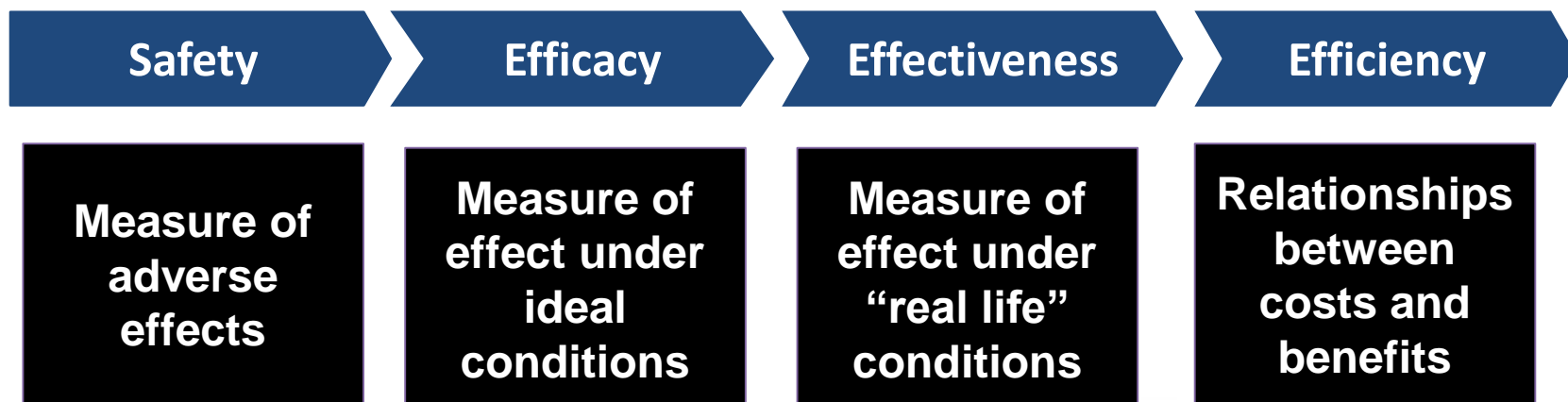


- HTA and value assessment
- The role of patients in providing evidence
- Why patient input to HTA is important
- Why patient organisations need support to participate in HTA
- Whose preferences should we use in health care decision-making?

What do decision-makers want?



- Safety and Efficacy are only the *first* steps to provide evidence for a new drug; Effectiveness and Efficiency need to be proven



- Efficacy does not imply effectiveness and effectiveness does not imply efficiency
- Safety and efficacy are the competence of regulators, effectiveness and efficiency are the competence of payers/insurers
- Use of Health Technology Assessment to assess value: evidence-based information for decision-making

Formal Use of HTA



Europe (not exhaustive)



The Americas



*



**varies by health plan*

Asia and Oceania



What kind of judgements are we making with clinical benefit assessment and/or cost effectiveness analysis?

1. Scientific judgements

- Reliability of the evidence-base
- Appropriateness of sub-groups
- Generalisability
- Capture of quality of life
- Handling uncertainty

2. Social value judgements

- Severity of disease
- End of life interventions (“rule of rescue”)
- Age
- Health inequalities
- Social value judgements taken into account, **but** there is lack of appropriate metrics

Level of Patient Involvement



- Varies from extensive (eg NICE in the UK) to very limited (eg CDR in Canada)
- Many HTA agencies allow patient submissions of evidence
- Other examples of involvement include:
 - scoping of the assessments (eg. choice of comparators, outcomes to be considered)
 - commenting on draft reports
 - appeals against recommendations

Why patient input to HTA is important

What do patients want from HTA?



- That the impact considered is broader than the health service
- That the impact of an illness and its treatment on the patient and family is understood
- That illness is given a priority to reflect its burden
- That a true reflection of a therapy's value is assessed
- That the assessors accept that all evidence has been generated with a particular view in mind

That the impact considered is broader than the health service



- Burden on the economy
 - Staying in the workplace
- Burden on social services
 - Staying independent
- Burden on families and friends
 - Staying active and mobile

Different perspectives



The Patient

- Accurate diagnosis
- Appropriate treatment without delay
- Symptom control
- Information and communication
- Some control over what is happening

The Physician

- Accurate pathology and markers
- Prognostic and predictive information
- Participation in clinical trials
- Good outcomes for patients

The Health Economist

- Benefits that can be measured objectively
- Certainty about the level of benefit
- Accurate costs associated with delivering a treatment
- Budget control

The evidence that patients and carers provide is set in the real world; it is ‘experiential’ evidence



- Saying you were sick five times each day is less meaningful than explaining that this means you cannot manage to go to work, or that it happens so quickly that you cannot make it to the toilet and have to clean up after yourself
- Recording that the fatigue caused by existing treatments is so severe that it means you have to lie down all day and so cannot look after your children
- Explaining that a pill is more acceptable than an intravenous treatment not just because it means less trips to hospital but because it allows you to continue living a more normal life
- Describing the effect a treatment has on your daily life – such as, ‘it means I don’t have to stay within 50 metres of a toilet’

- Only patients and their caregivers know precisely how a disease impacts on daily life and how specific treatments or management strategies can influence its quality

Why patient organisations need support to participate in HTA

HTA is not straightforward




$$1+1 = [(27/3)/3]-1$$

Nor easy to navigate



Patient evidence and quality of life

- * evidence from an online survey on QoL
- * evidence from face-to-face interviews replicating EQ-5D-5L

Research questions



- Are there differences between patients and population preferences due to adaptation of patients to health states?
- Are there aspects of patient QoL that are not captured by generic QoL tools such as the EQ-5D-5L?
- Whose valuations should we take into account in decision-making?

Results of the online survey

(1,031 administered surveys; 767 completed surveys;
response rate 74%)

Patient characteristics & QoL



Table 1. Patient employment and demographics (n=767)

Age (mean, SD)	50 (14.13)
Age at diagnosis (mean ,SD)	39 (17.5)
Gender, Female (n,%)	591 (77%)
Marital status (n,%)	
Single	174 (23%)
Married or cohabiting	513 (67%)
Divorced	50 (7%)
Separated	15 (2%)
Widow	15 (2%)
Employment status (n,%)	
Employed	312 (40.7%)
Unemployed	27 (3.5%)
Temporary sick leave	58 (7.5%)
Permanent work disability	19 (2.5%)
Retired	83 (10.9%)
House wife/ husband	182 (23.8%)
Student	30 (3.9%)

Table 3. All patient QoL and Disability
Mean (SD) or n (%)

EQ5D Utility	0.62 (0.27)
Utility loss	0.24 (0.27)
EQ5D VAS	63 (23.35)
Barthel index	18.2 (3.25)
Independent (20)	175 (54%)
Mildly dependent (15-19)	119 (37%)
Moderately dependent (10-14)	19 (6%)
Severely dependent (0-9)	12 (4%)

Disease specific characteristics & QoL



Table 5: Characteristics and HRQOL results of patients and caregivers across sample main disease areas
(Average figures)

	All sample	BC	RA	MDS	EDS	Blood cancer	Asthma
Patient age	50	53	47	65	35	57	49
Caregiver sample	77	5	2	11	3	7	2
Caregiver age	54	57	54	63	44	55	45
Patient Satisfaction	6.5	7.7	6.4	8.1	4.6	8.2	7.0
Caregiver Satisfaction	6.4	6.8	3.0	7.7	5.0	7.4	6.0
Patient Utility	0.62	0.70	0.60	0.70	0.33	0.50	0.74
Patient Utility loss	24%	15.2%	27.6%	16%	53.1%	35.2%	12.3%
Caregiver Utility	0.72	0.73	0.81	0.73	0.76	0.88	0.44
Caregiver utility loss	14%	14%	6%	12.7%	10.2%	0%	42%
VAS patients	63	69	67	67	45	53	63
VAS caregivers	69	81	85	79	68	71	61
Barthel index	18.34	19.59	18.06	18.84	15.29	16.93	18.83
Zarit scale	35	27	32	32	36	29	34

Patient Evaluation of EQ-5D-5L tool

“Are there any aspects of your illness, which have had a big impact on your health, that were not captured by the EQ-5D-5L?”

- Yes (n=359, **51%**)
- Please tell us what they are (n=325, **46%**)

	Fatigue	Side Effects	Cognitive problems	Relationships /social life	Family issues	Co-Morbidities	Sleep	Fear for future	Work issues	Financial issues
All sample (n=325)	19.5%	9%	6.5%	6.2%	5.3%	5%	4.6%	3.5%	3%	2.2%
BC (n=83)	21.7%	20.5%	9.5%	3.6%	4.8%	1.2%	2.4%	3.6%	1.2%	2.4%
MDS (n=30)	40%	13.3%	3.3%	-	3.3%	-	3.3%	6.6%	3.3%	-
Blood cancer (n=11)	9%	18%	9%	9%	18%	9%	-	18%	9%	-
KC (n=8)	37.5%	12.5%	-	-	12.5%	-	-	-	-	-
RA (n=20)	15%	5%	-	5%	-	-	5%	-	5%	-
AS (n=6)	33%	-	33%	33%	-	-	-	-	16.6%	-
PsA (n=4)	25%	-	-	-	-	25%	-	-	50%	-
EDS (n=27)	30%	-	15%	11%	7.5%	7.5%	3.7%	-	7.5%	3.7%
Asthma (n=15)	-	-	-	7%	7%	-	7%	-	7%	-
ET (n=9)	11%	-	-	22%	11%	11%	-	-	-	11%
RLS (n=13)	7.7%	7.7%	7.7%	-	-	-	70%	-	-	-
HMS (n=6)	83.3%	-	16.6%	-	-	16.6%	-	-	-	-
HIV (n=3)	-	66.6%	-	33.3%	-	-	-	-	-	-

2. Results of the face-to-face interviews replicating Euroqol process (611 interviews; 2 diagnoses; breast cancer and rheumatoid arthritis)

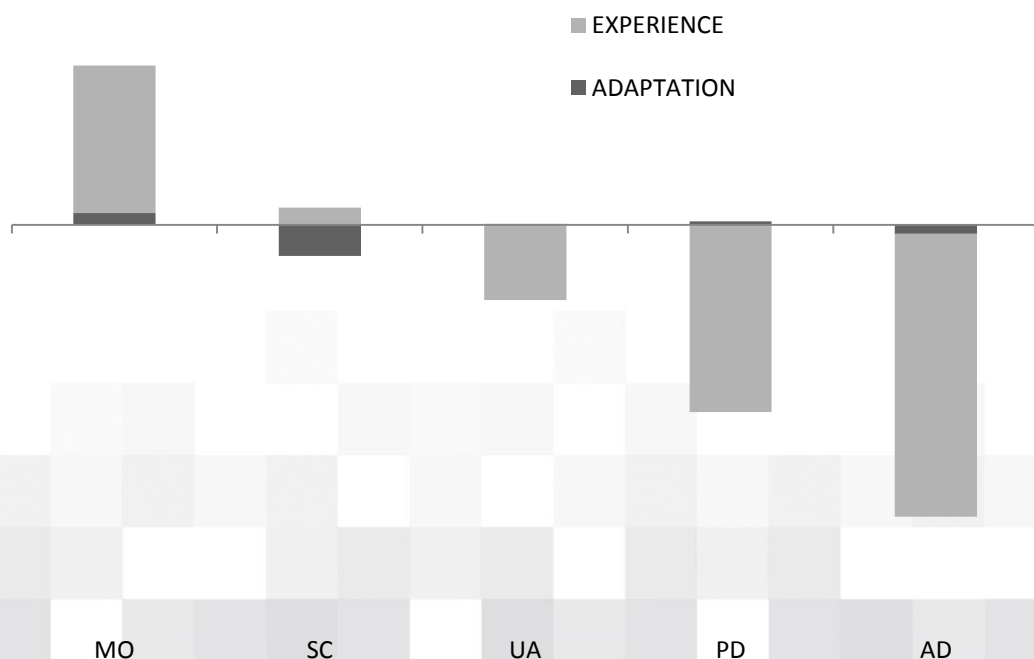
Are there differences?

	G : P	Ra : Bc	G : Ra	G : Bc
11112	✓	●	✓	✓
12111	✓	●	✓	✓
11221	✓	●	✓	✓
13122	●	●	●	●
11421	●	●	●	●
13313	●	●	●	●
21315	✓	●	✓	✓
23242	✓	●	✓	✓
32314	✓	●	●	✓
54231	●	●	●	●
33253	✓	●	✓	✓
45233	●	●	●	●
34515	✓	●	●	✓
45144	●	●	●	●
24553	✓	●	✓	✓
55424	●	●	●	●
52455	●	●	●	●
43555	●	●	●	●
55555	✓	●	✓	✓

Findings:

1. No differences between patient groups
2. (10/19) Statistically significant Differences between general population and patient groups

Do the preferences towards health related quality of life differ between the general population and defined patient groups?



1. Differences exist because patients have experience with the impaired
2. Effects of adaptation on hypothetical health states valuation are trivial
3. Significant differences on three health dimensions:
 - mobility,
 - pain or discomfort,
 - anxiety or depression.

Advantages of using patient value set



- We found statistically significant differences between patients' valuations and general public valuations on three health dimensions: mobility, pain or discomfort, and anxiety or depression.
- Adaptation in the case of HRQoL no longer possess a normative problem, as the effects of adaptation on hypothetical health states valuation are trivial
- It seems that a "veil of ignorance" is too thick
 - differences in preferences stem from patients being more able to accurately imagine "non-tangible" dimensions of health states (anxiety or depression and pain or discomfort)
- Patients consider problems related to mobility less problematic, while problems related to pain/discomfort and anxiety/depression are seen as more problematic. Largest disagreement is on anxiety/depression dimension.

Policy implications



1. Patient value set does differ from general population values set and has na impact on priority setting.
2. A shift from general population value set to patient value set would result in **lower priority** given to health states/diseases where the problems are connected to
 - ❖ Mobility dimensionand in **higher priority** given to health states /diseases where the problems are connected to
 - ❖ Pain / Discomfort,
 - ❖ Anxiety / Depression
3. Eliciting patient preferences is far more difficult than eliciting preferences from general population. Before such a shift can happen, further studies are needed to address:
 - whether mapping from general to patient population is possible
 - whether differences between both sets equal in various settings (countries)