

German Cancer Research Centre
DKFZ Heidelberg Germany
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Health economic evaluation and high cost services: The need for reconsideration

Jeff Richardson
Emeritus Professor, Foundation Director
Centre for Health Economics Monash Business School
Monash University Australia
www.monash.edu/business/che

SUMMARY

Objective of economics

Best allocation of finite resources

BUT methods have mixed success

- Simple competitive markets ✓✓
- Social infrastructure - ??

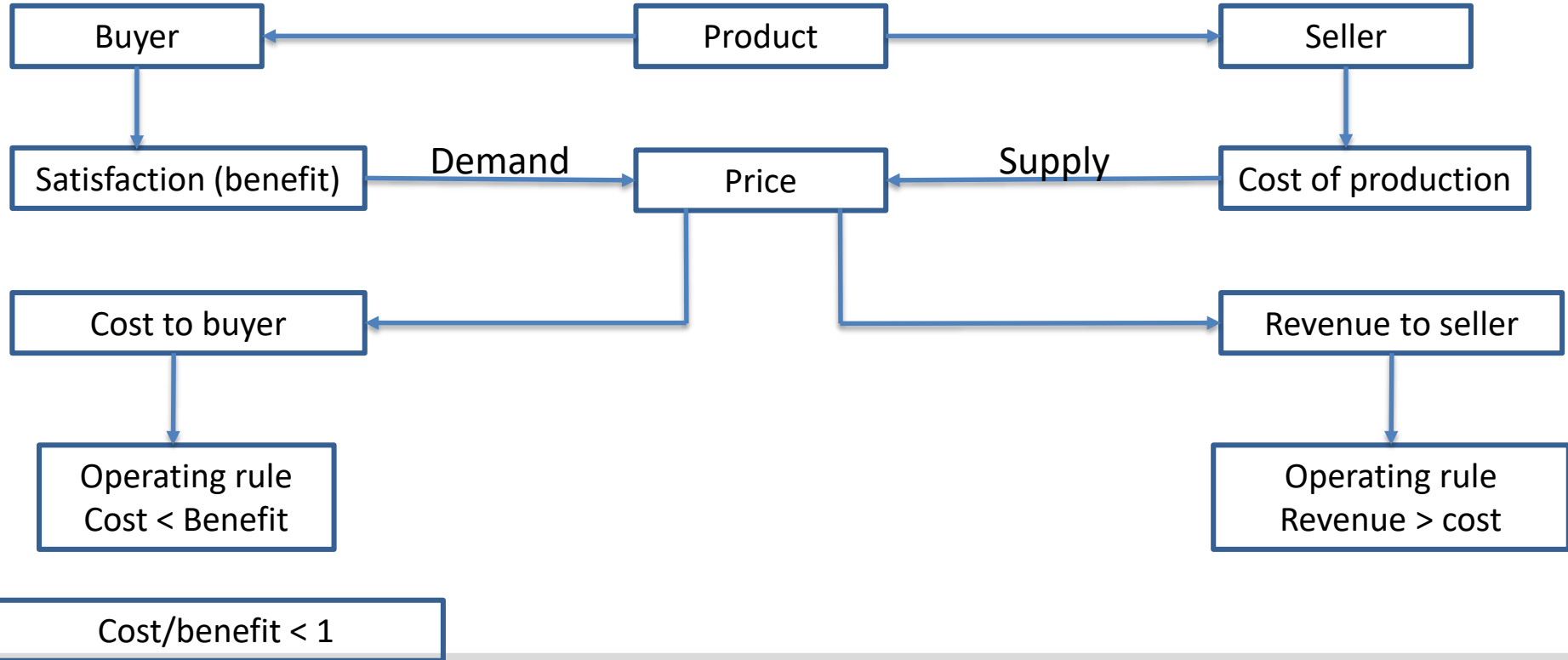
- Economic evaluation
 - ← Unsupported assumptions *wrt* values, motivations
- Empirical evidence
 - Need for revision of theory/practice
 - Fairness first paradigm
 - ie theory, methods commence with fairness

OUTLINE

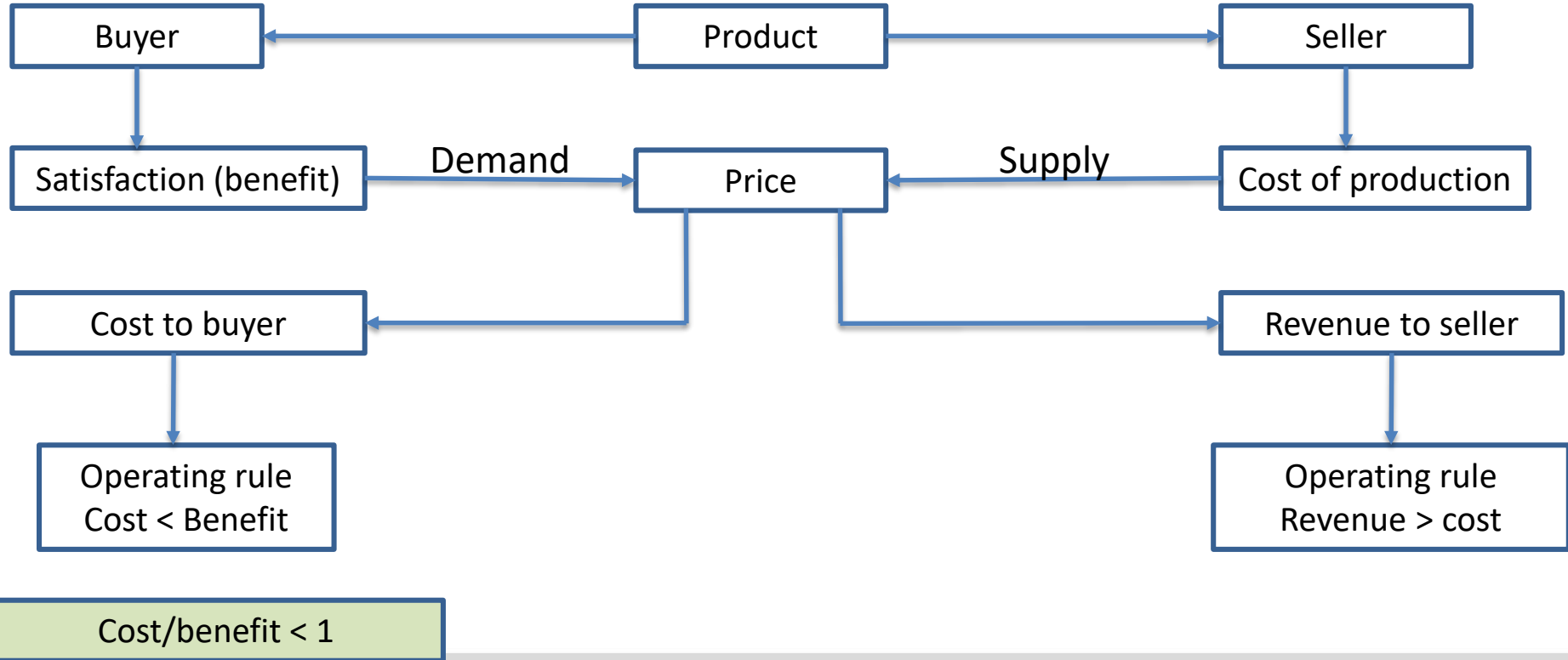
1. Welfare theory
2. Where economic evaluation theory fails
 - Utility maximisation
 - Expected utility theory
 - Fairness
3. Empirical evidence
 - Individual values
 - Sharing
4. Need for a paradigm shift

1. WELFARE THEORY

WELFARE THEORY: THE FOUNDATION OF EVALUATION THEORY

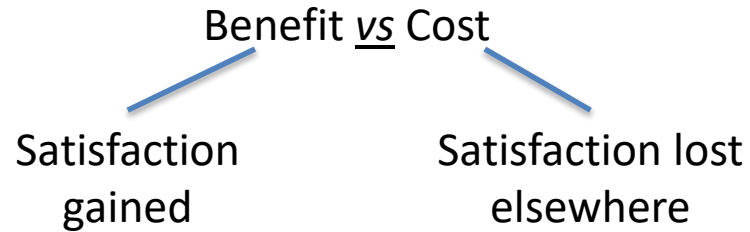


WELFARE THEORY (Summary)



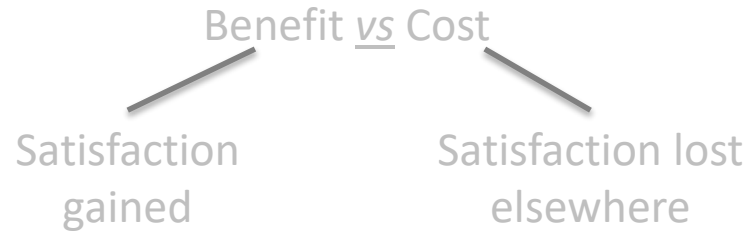
BUYER PERSPECTIVE

- Key Element:
Direct comparison by buyer



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Direct comparison by buyer



- Key to efficiency = choice (consumer sovereignty)
 - a) The buyer is in the best position to judge net benefit and choose
 - b) Resources gravitate to the products preferred by buyer
- Consequence: preferences (utility) maximised

SOCIAL PERSPECTIVE

- All individuals maximise utility, U .

Therefore

Social welfare $W=f[U_1 \dots U_n, \text{only}]$
= 'Welfarism'

A FEW PROBLEMS WITH WELFARE THEORY

PROBLEM 1 MOTIVATION

Motivation

Is utility the only motivation??

BUT

Behaviour ← habit/duty/religion/conformity/
marketing, etc

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‘Solution’

Revealed preference criterion

If chose X then by definition

you prefer X to alternatives

Choice identifies utility

PROBLEM 2 RISK

Outcomes

Subject to risk

Utility ignores risk

'Solution'

People maximise expected utility (EU)

$$EU = \sum p_i U_i = EUT$$

EUT=Expected Utility Theory

Claim: p_i =takes account of risk attitude

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Health Economics

Outcomes *st* risk

Use standard gamble to assess utility

Utility \leftarrow gamble, takes account of risk attitude

PROBLEM 3 DISTRIBUTION

Social Welfare

$f(U_1 \dots U_n)$... ignores distribution of utility

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Social Welfare
'Solution'

$f(U_1 \dots U_n)$... ignores distribution of utility

Potential compensation principle (Kaldor Hicks)

'If gain to A > loss to B'

then compensation is possible

= Better outcome

PROBLEM 3 DISTRIBUTION

Social Welfare
'Solution'

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Potential compensation principle (Kaldor Hicks)
'If gain to A > loss to B'
then compensation is possible
= Better outcome

Conclusion

If utility maximised
= 'Utilitarianism'

RESULT

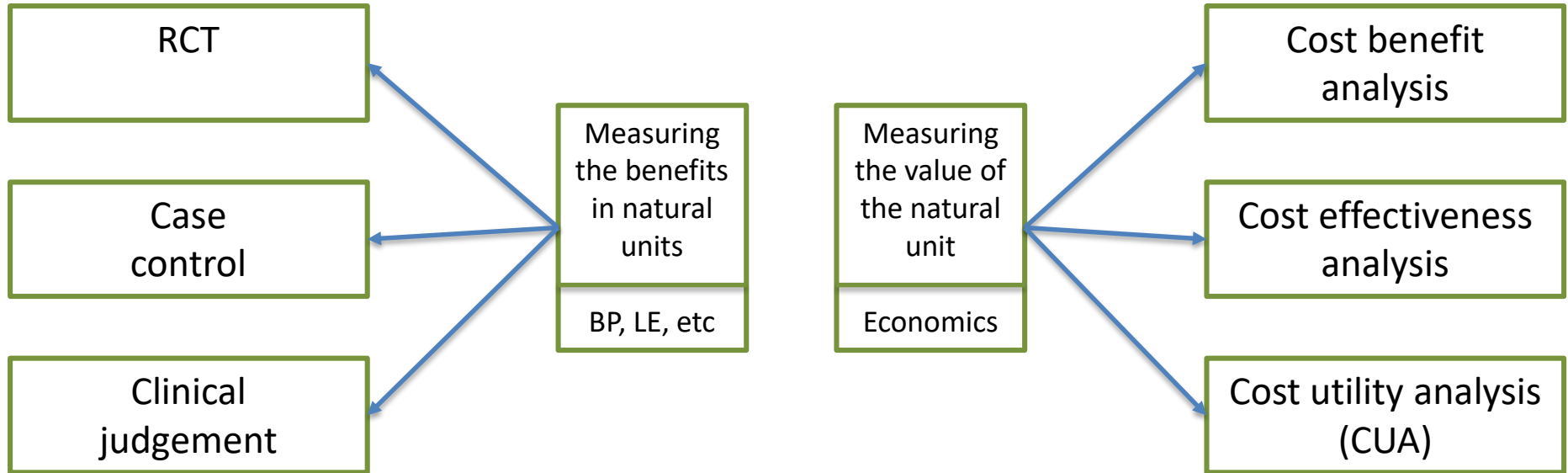
- A logically complete system
- Assumptions → most 'efficient' (ie maximising) outcome
- Important caveat
 - Welfare theory permits an 'equity-efficiency trade-off'
BUT Rules for efficiency clear, explicit
No rules /guidelines for equity
 - Implies 'efficiency focused' paradigm

1(a) Adaptation to Health Economic Evaluation

A PROBLEM

- Welfare theory
 - Key to efficiency = the direct comparison of benefits/costs by buyer
- Health economics evaluation
 - Health authority makes comparison (input from)
- Response: retain the key equation: $\text{cost/benefit} < 1$
- But benefit \nleftarrow individual choice
 \leftarrow estimated benefit

THE EVALUATION FRAMEWORK



COST UTILITY ANALYSIS

- Benefit = QALYs
- QALY = (life years)*(utility) = utility of benefit
= Quality adjusted life year
 'utility' = strength of preference
- Decision criterion
 Minimise cost/QALY
 → maximum QALYs from a budget

CHANGING THE GOALPOSTS

- Benefit \leftarrow individual comparison cost vs benefit
- Now \leftarrow estimated benefit
- Changes violate assumptions of welfarism

Response

- QALY = measure of 'health'
- Other assumptions OK
- Welfarism \rightarrow Extra Welfarism

A NEW, COMPLETE SYSTEM

- QALYs = best estimate of (duration weighted) utility or 'health'
- Min cost/QALY \rightarrow max QALYs
- Assumptions
 - Total health/QALYs \uparrow
 \rightarrow social welfare \uparrow

2. WHERE ECONOMICS FAILS

THEORY AND IMPLEMENTATION

- Implementation – imperfect methods
 - eg Measuring utility: seriously defective (EQ-5D)
- Theory = ‘foundations’ of evaluation methods
 - ← problematic assumptions = focus below
(bad theory → measurement irrelevant
or ambiguous use)

PROBLEM 1: INDIVIDUAL MOTIVATION

- Is maximising utility the only motivation?
 - Habit/duty/religion/conformity/marketing ??

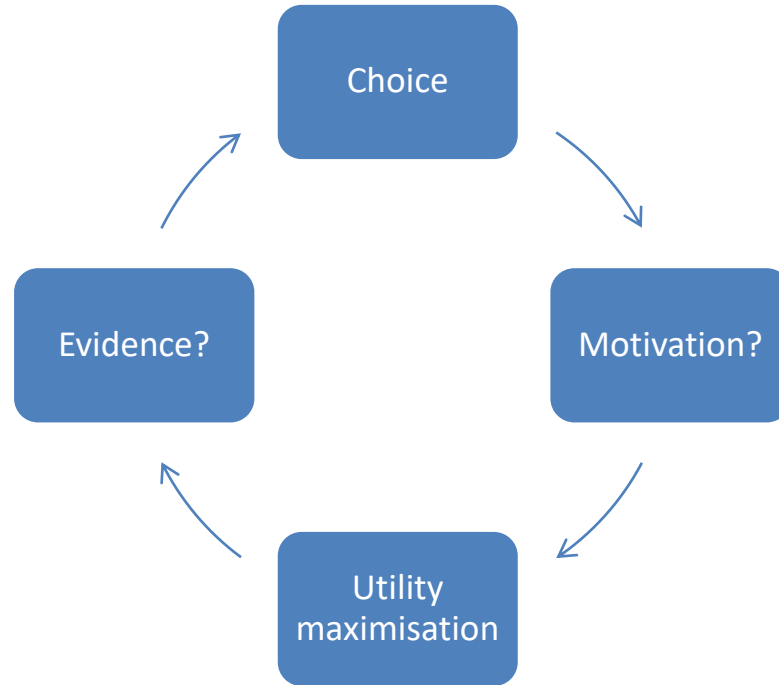
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 - Revealed preference criterion
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- **Criterion behaviourally barren**

THE REVEALED PREFERENCE TAUTOLOGY



FAILURE OF REVEALED PREFERENCES

- If Utility maximisation → harmful outcome (eg ignorance)
 - Choice → regret (individual)
 - Choice rejected by paternalistic policy

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- If Utility maximisation → harmful outcome (eg ignorance)
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 - Choice rejected by paternalistic policy
- **Collective decision making eg NHS**
 - Revealed (individual) preference not possible

CONCLUDE

- CUA ~~↔~~ empirical evidence of individual motivation
- Motivation ← behaviourally barren tautology
- Behavioural economics = a response
- Health economics unaffected (to date)

PROBLEM 2: EXPECTED UTILITY THEORY

People maximise expected utility ($EU = \sum p_i * U_i$)

... Empirically wrong (Allais 1953; Schoemaker 1982 *ff*) ... largely ignored

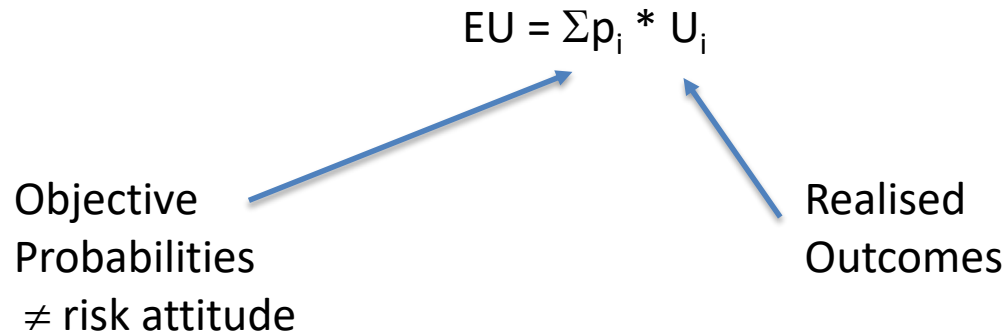
→ Omission of utility of risk per se (thrill/dread of etc)
(Von Neumann and Morgenstern)

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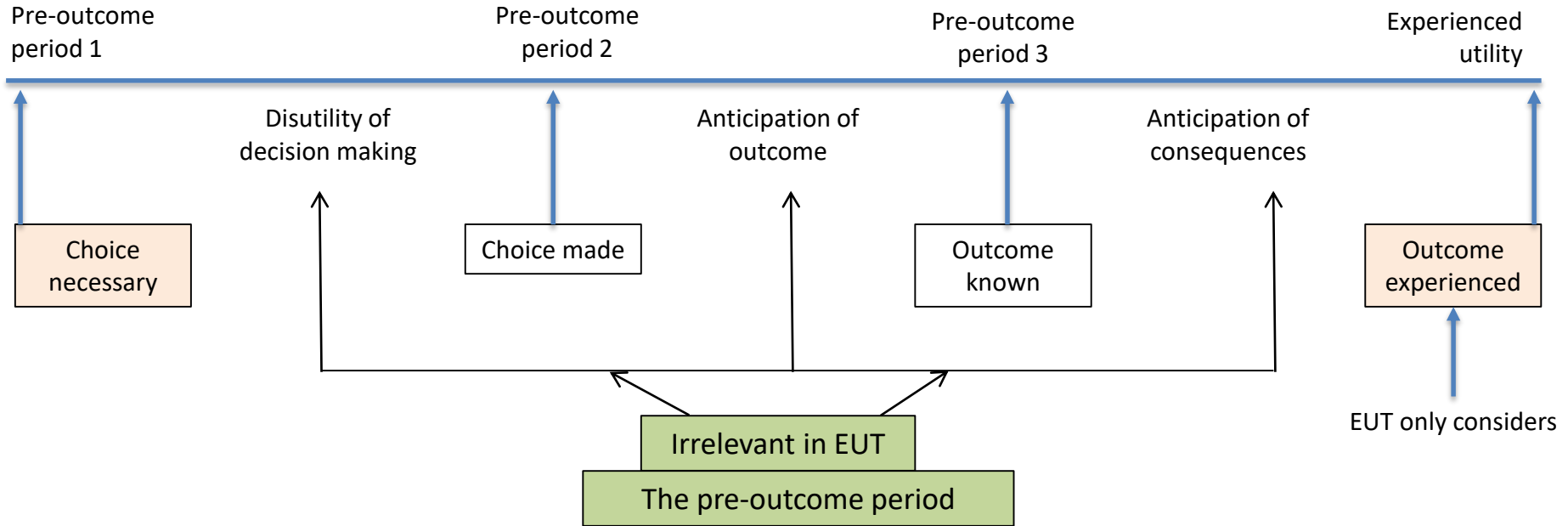
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EUT: THE OMISSION OF A TIME DIMENSION

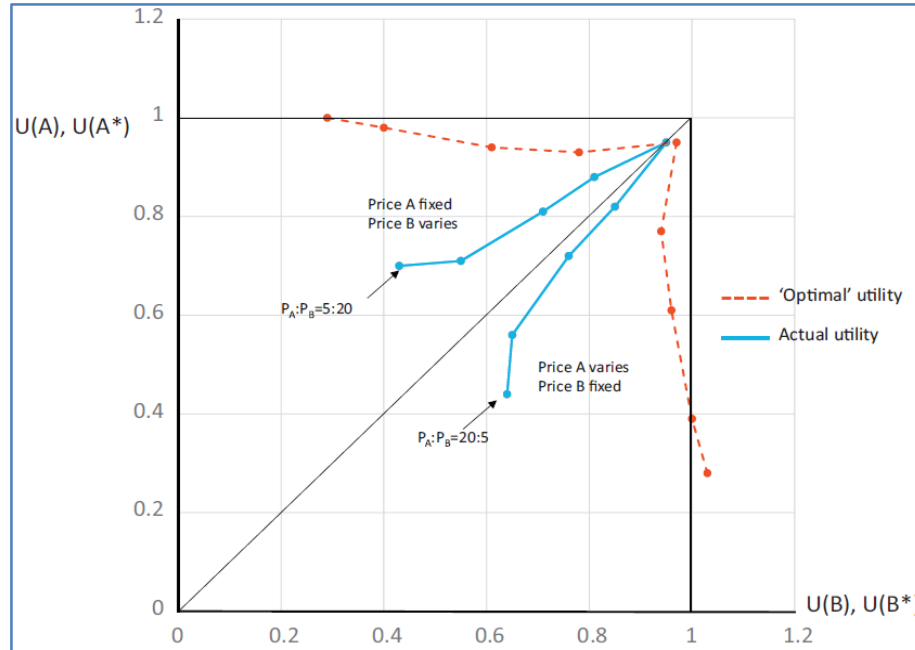


CONCLUDE

- CUA ignores emotions/behaviours in pre-outcome period

RECENT TEST OF CHOICE AND EUT

One of 2 illnesses will occur
 Service A, B \rightarrow QoL \uparrow
 Purchase insurance
 as $P(A)$, $P(B)$ varies



Plot
 Combination of U_A, U_B
 which

- Maximises EU
- - - 403 respondents select

- Conclude: Economic evaluation ignores risk

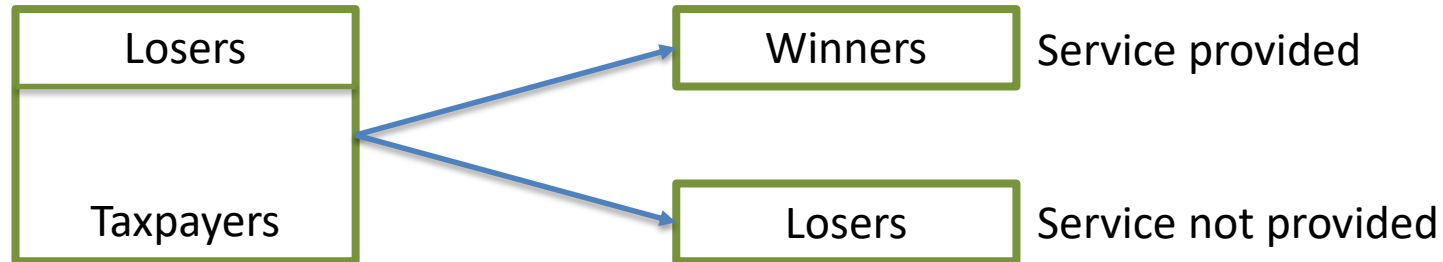
Richardson et al 'Uncertainty and the Undervaluation of services for severe health states in CUA', Value in Health (on line 2017)

PROBLEM 2: SOCIAL PREFERENCES

- Do people want maximum QALYs
 - Maximisation ignores distribution
 - 4 people: $(5+5+5+0) > (3+3+3+3)$
15 QALYs > 12 QALYs

PROBLEM 2: SOCIAL PREFERENCES

- Do people want maximum QALYs
 - Maximisation ignores distribution
 - 4 people: $(5+5+5+0) > (3+3+3+3)$
 - 15 QALYs > 12 QALYs
 - CUA → winners/losers



JUSTIFICATION FOR NON-PROVISION TO LOSERS

- Rhetorical ... more QALYs ('health') better than less losers ... lose!
- Ethical ... utilitarianism: an assumed goal
- Evidence of population support ... na



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3. EMPIRICAL EVIDENCE: INDIVIDUAL VALUES

SURVEY EVIDENCE FROM AUSTRALIA (n=455)

Which ethical principle

- Australians are not hedonic utilitarians

‘Action producing happiness is always right’

agree 22.8%

disagree 57.4%

‘Maximising happiness is more important than any other principle’

agree 14.3%

disagree 65.9%

SURVEY EVIDENCE FROM AUSTRALIA (n=455)

- There is a strong commitment to 'duty', 'role in community' (solidarity/communitarianism)

'I must fulfil duties even if it makes me less happy'

agree 92.0%

disagree 8.0%

'Having duties is a natural part of being a member of society'

agree 95.0%

disagree 5.0%

DUTY = LONG RUN SELF INTEREST ??

‘People help others only because they gain something personally’

agree	18.2%
disagree	60.7%

CONCLUDE

- Personal motivation
 \neq pure self interest
- Social motivation therefore:
 unlikely to be the sum of individual self-interest
- Task: what personal motivations are relevant to social decisions

EVIDENCE FROM ANTHROPOLOGY

Behaviour ← social role/social inter-relations

- Social behaviour
 - Motivation
 - Reciprocal altruism ('weak reciprocity')
 - Help others expect reciprocal treatment
 - Strong reciprocity
 - Punish others for selfishness in absence of self interest

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 - Ultimatum game: Personal loss to punish unfair behaviour
 - Dictator game: Share with others at personal loss; no possible penalty

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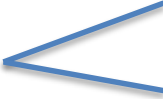
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 - 'Sharing is a core feature of human society' (Kameda 2002)

3b. EMPIRICAL EVIDENCE

Allocating The Budget: Results from 4
surveys

SIMILAR METHODS

- Web based allocation exercises
- Fixed budget:
 allocate between  low cost QALY ... CUA includes
 higher cost QALY ... CUA excludes
- Budget rises, sharing possible

Sharing Survey 1

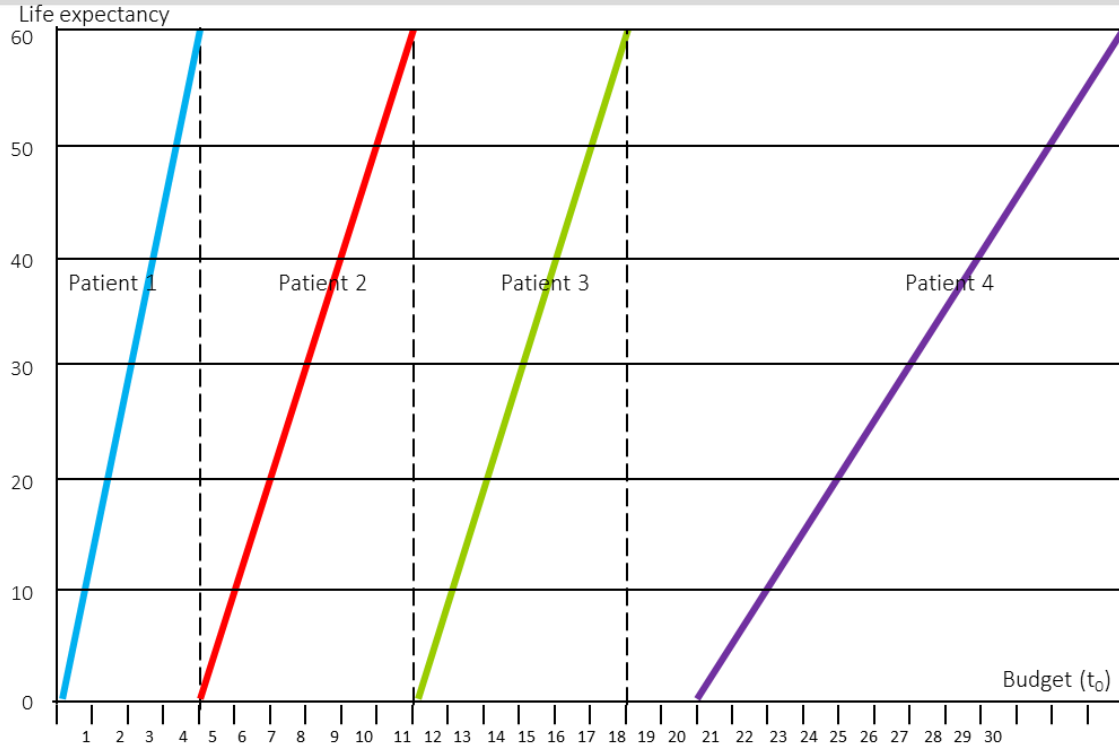
MAXIMISING HEALTH VERSUS SHARING: MEASURING
PREFERENCES FOR THE ALLOCATION OF THE HEALTH
BUDGET

Richardson J, Sinha K, Iezzi A, Maxwell A
Social Science and Medicine 2012 75(8):1351-1361

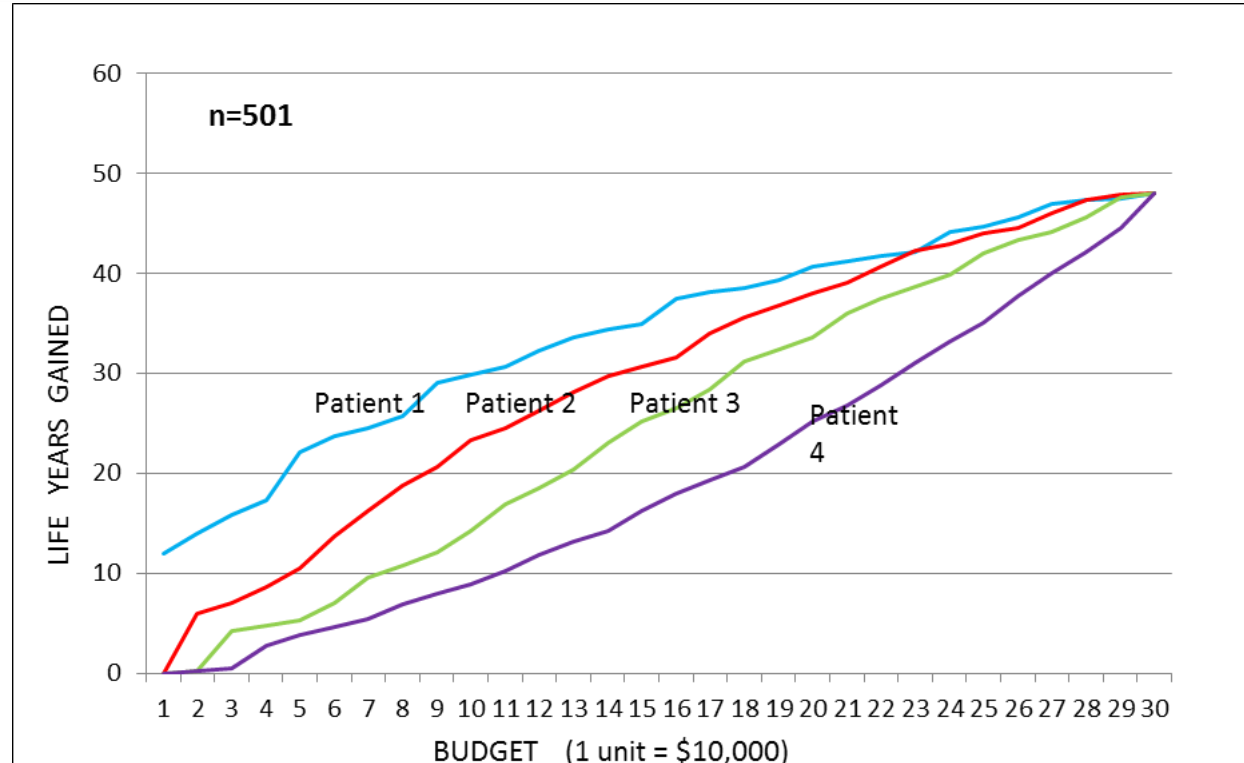
WEB BASED ALLOCATION EXERCISE (n=532)

Patient 1	12 yrs	12 yrs	12 yrs	12 yrs	12 yrs	12 yrs	12 yrs	12 yrs	12 yrs	12 yrs	12 yrs	12 yrs	12 yrs
Patient 2	8 yrs	8 yrs	8 yrs	8 yrs	8 yrs	8 yrs	8 yrs	8 yrs	8 yrs	8 yrs	8 yrs	8 yrs	8 yrs
Patient 3	6 yrs	6 yrs	6 yrs	6 yrs	6 yrs	6 yrs	6 yrs	6 yrs	6 yrs	6 yrs	6 yrs	6 yrs	6 yrs
Patient 4	4 yrs	4 yrs	4 yrs	4 yrs	4 yrs	4 yrs	4 yrs	4 yrs	4 yrs	4 yrs	4 yrs	4 yrs	4 yrs

CEA AND LIFE YEARS ALLOCATED



SURVEY RESULT



CONCLUSION, SHARING SURVEY 1

- Cost is relevant
But
- Sharing with most costly treatment immediate

SHARING 2 LIFE EXTENSION

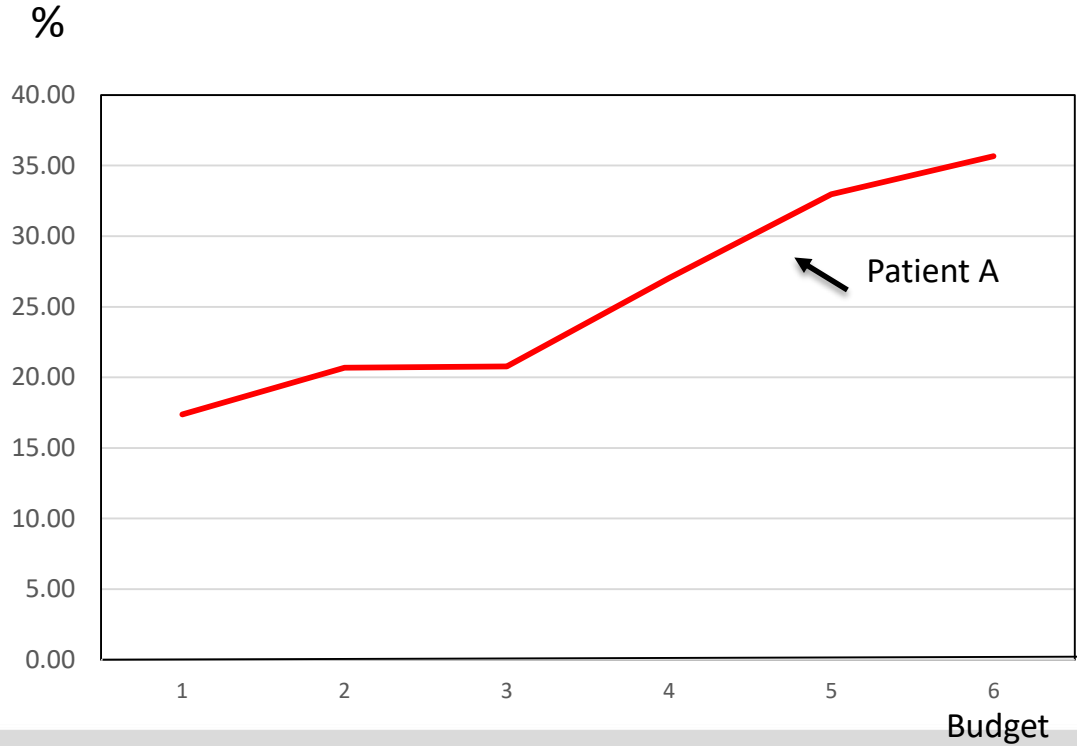
Sharing and the provision of “cost ineffective” life extending services to less severely ill patients

Richardson, Iezzi, Maxwell *Value in Health* 2018 (in press)

DESIGN

	A	B
Life expectancy	10	2
Cost/LY	2,000	1,000
Budget = progressively increases		
n=430		

PERCENT OF INCREMENTAL LIFE YEARS GIVEN TO PATIENT A: LIFE EXPECTANCY LONGER; COST/QALY HIGHER



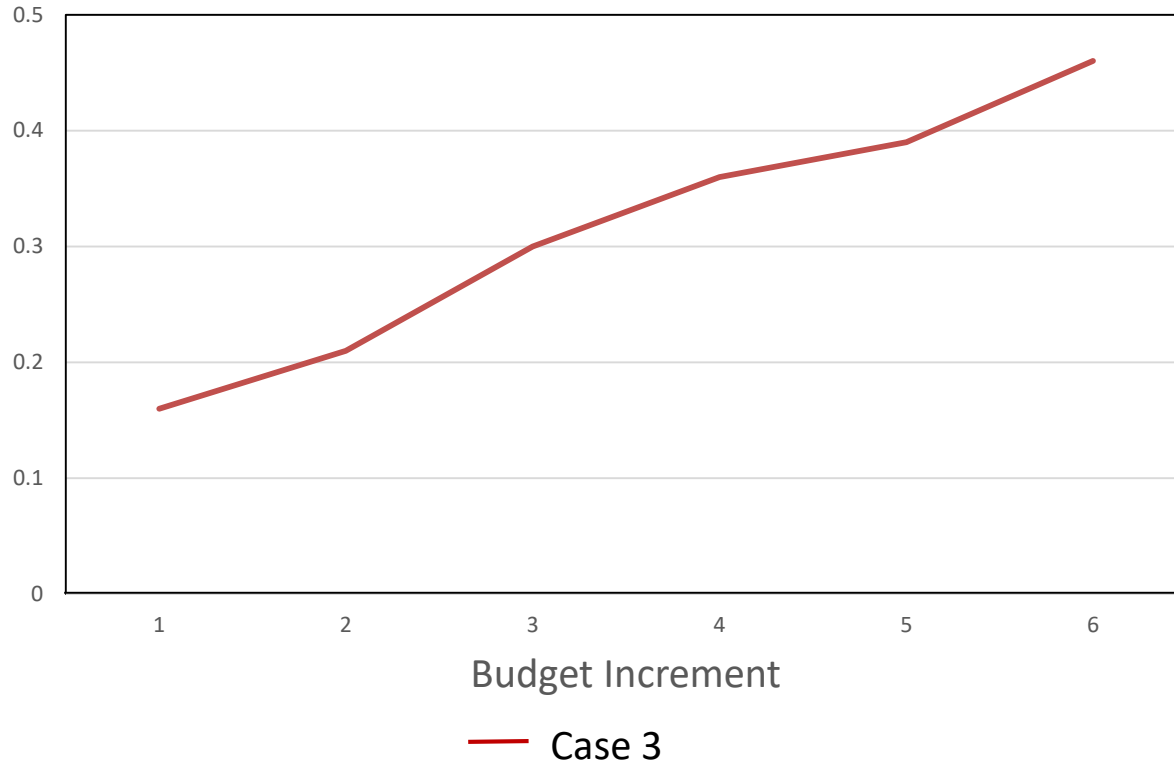
SHARING 3 QoL

Sharing in a communal health scheme when services improving the quality of life are not cost effective and patients are not severely ill

Richardson, Iezzi, Maxwell
Medical Decision Making 2018 (under review)

SHARING QUALITY (n=203)

% share of
Budget to
Patient A:
 $\text{cost}/\text{QoL}=3 \times \text{B}$
QoL 50 vs 30



SHARING SURVEY 4: Orphan Products

Sharing in a communal health scheme when services improving the quality of life are not cost effective and patients are not severely ill: Results of a population survey

Richardson, Iezzi, Maxwell

PharmacoEconomics 2017; online 2016

SURVEY (n=432)

- Allocate a budget
 - Illness A: 5 patients (no treatment – die; budget $\uparrow \rightarrow$ QoL \uparrow)
 - Illness B: many patients (budget $\uparrow \rightarrow$ QoL \uparrow)
- Cost varied: \uparrow QoL A = 20, 15, 10, 5, 2 x Cost \uparrow QoL B
- Size Group B varied: n = 100, 300, 600

TRADE-OFF

- Budget to A → less for B
- Small benefit/\$ vs large benefit \$
- Small total benefit vs large total benefit

TRADE-OFF

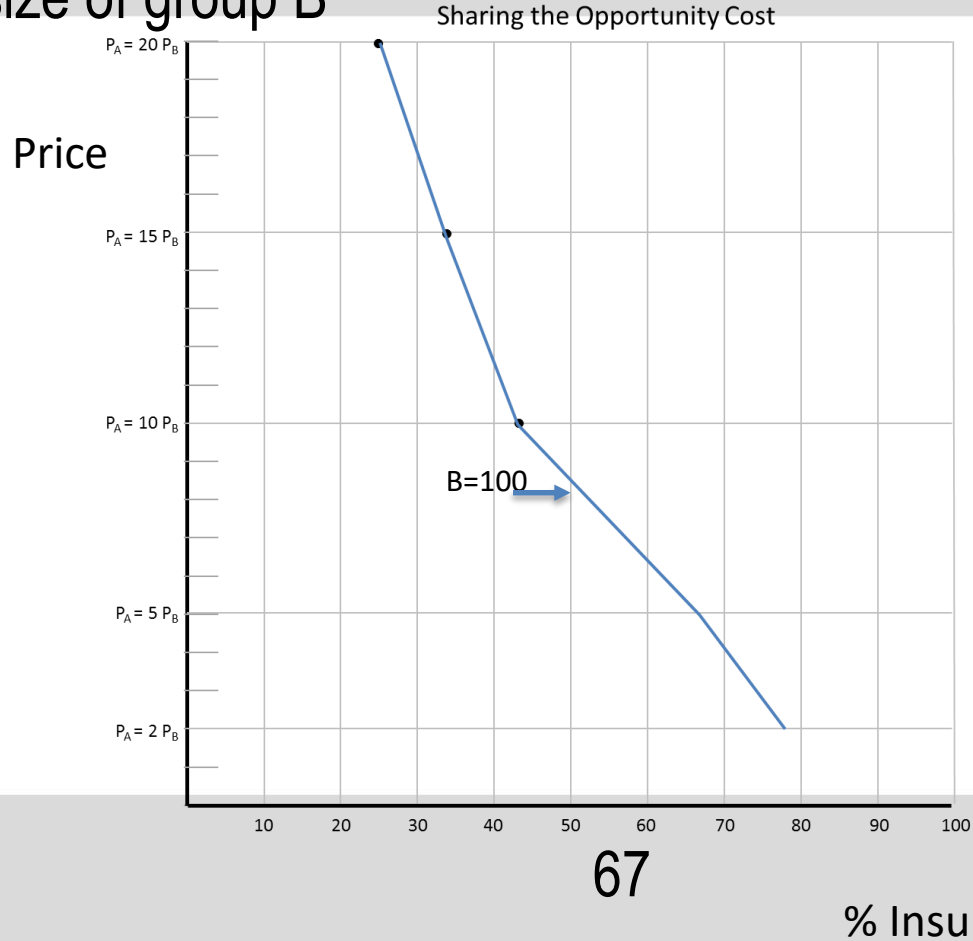
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Sailor at Sea Hypothesis

- Small numbers in group A \rightarrow low loss/person B
- Urgent benefit A vs non urgent effect B
- Hypotheses
 - Immediate sharing (CUA \rightarrow no budget for A)
 - Number of B \uparrow \rightarrow loss/person B \downarrow
 - \rightarrow sharing \uparrow
 - Cost A \uparrow \rightarrow sharing \downarrow

ALLOCATION TO HIGH COST PATIENT (B)

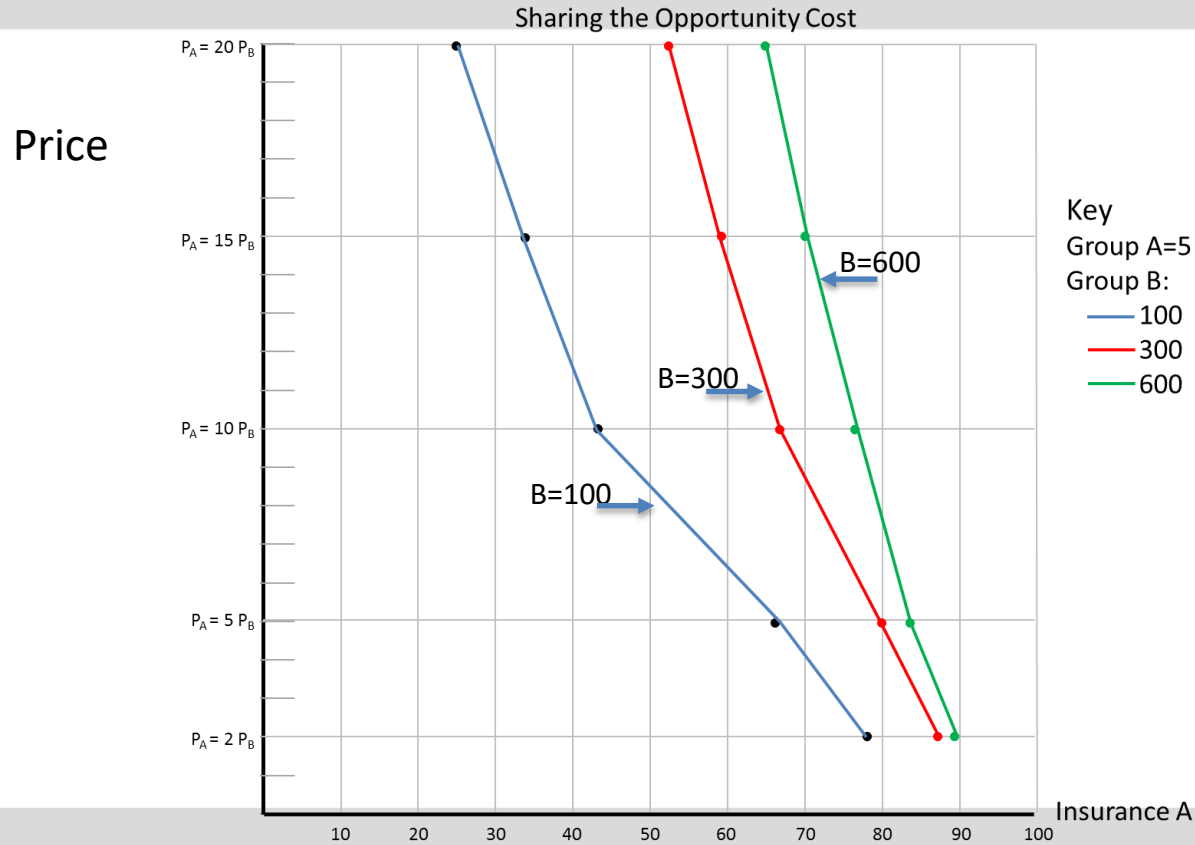
Price and size of group B



Group A=5

Group B: _____100

INSURANCE A BY PRICE A AND SIZE OF GROUP B



CONCLUSION SHARING STUDIES

- Design to date prevents sharing
- Sharing allows
 - Partial treatment of high cost/QALY services
 - In exchange for small loss for less severe patients
- Rationing ← intensity of care



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4. FAIRNESS vs EFFICIENCY PARADIGMS

REASONS FOR CHANGE

1. Motivation as a citizen in a social context
≠ motivation as an individual (Aristotle)

Individual, utility maximisation

- a) An inadequate explanation of behaviour
- b) (Wrongly) extrapolated to social context

2. Utilitarianism: excludes individuals
rejected by public
never empirically supported

REASONS FOR CHANGE FROM ECONOMIC THEORY (Cont)

3. Exclusion of patients

- Violates medical practice
- Violates social preferences

4. Community support

- Sharing
- Other fairness variables in literature

TWO PARADIGMS

- Extra Welfarism (Present theory)
 - Focus: Services (← simple theory of a market)
 - Objective: Maximise efficiency of service mix
 - Rationing: Exclude services

- Communitarianism
 - Focus: Patients
 - Objective: Universal entitlement
 - Rationing: Intensity of care

TWO PARADIGMS

Attribute	Present (Extra Welfarism)	Communitarianism
Analytical Focus	Maximisation	Optimisation (Fairness)
Social objective	Max utility	Fair sharing
Criterion for funding	Cost/QALY < threshold, T	Presumed entitlement
*Exclusions	Yes Cost/QALY >T	No (except extreme cases)
*Caveat	Ad hoc adjustment for undefined equity	Systematic adjustment for cost effectiveness
Funding formula	If criterion met, then 100% funding	Level of treatment varies =f[<i>fairness variables, cost, effectiveness</i>]
*Role of cost	Pivotal: max benefit ← min cost	Secondary: alters allocation, ie the intensity of care
Ethical basis	Utilitarianism	Communitarianism satisfaction of community preferences

UNRESOLVED ISSUES

- Challenges (hopefully) for future research
- Agreement/quantification of fairness
- Who makes the decisions?

SOLUTIONS TO 'UNRESOLVED ISSUES' EXIST

Agreement/quantification of fairness

- Empirical Ethics
 - Ultimate authority ← society
 - ✗ historical assumptions

HYPOTHETICAL WEIGHTS w^* INCORPORATING SEVERITY AND SHARING⁽¹⁾

Percent of population	Initial Severity	1.0	0.8	0.6	0.4	0.2
	Sev ^{0.5}	1.0	0.89	0.72	0.63	0.45
N	N ^{-0.434}	(Sev ⁻⁵)(N ^{-0.434})(Share)Y				
0.001	20	20	17.8	15.4	12.6	8
0.01	7.3	7.3	6.5	5.6	4.6	2.9
1.0	1	1	0.89	0.72	0.63	0.45
10	0.37	0.37	0.33	0.27	0.23	0.17

(1) $w^*=1/w$, where w = the threshold weight in equation 2

WHO MAKES DECISIONS

- Statutory (or advisory) body
(like Reserve Bank, Bureau of Statistics)
- Composition ... see McKie et al
Focus Group → mixed composition

CONCLUDING COMMENT

Could economists be fundamentally wrong for so long?

YES Evaluation theory \Rightarrow empirical error learning

Wrong allocation formula

\Rightarrow stock exchange crash

\Rightarrow bridge collapse

\Rightarrow contradictory observations

– Result

- Errors invisible
- None to challenge economists' authority

– Alternative

- 'Empirical Ethics': Population values *s.t.* ethical critique
- Ultimate arbiter: (laundered) social values



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Thank You



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Vielen Dank