

A comparison of public and decision-maker stated preferences for pharmaceutical subsidy decisions

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Refusal of funding angers cancer victim

AFTER battling cervical cancer herself, Kim Lambert will pay the \$460 to protect teenage daughter A.J. with Australia's world-first cancer vaccine if she has to.

But she is annoyed it may come to that. The vaccine, known as Gardasil, has been refused a place on Australia's immunisation program for the time being because of cost.

The Pharmaceutical Benefits Advisory Committee said it made the decision because the vaccination program, which would have cost taxpayers about \$625 million in its first four years, was not value for money.

"I can't understand why Kim Lambert, of Ipswich, is a 15-year-old daughter hoping this would become a school vaccination program quickly as possible."

"It's a vaccination that just blows my mind. I wouldn't approve of it."

"In the long term, cervical cancer costs a lot of money. It's a vaccination that has never been used before."

Ms Lambert will pay \$460 for the radical hysterectomy of the uterus, cervix and the vagina.

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by the Australian Bureau of Statistics and the Master 1970s and 1980s," he said.

Red tape delays new medicine for asbestos victims

Leanne Edmiston

A DRUG proven to improve the quality of life and longevity of those with mesothelioma has yet to be approved for the Pharmaceutical Benefits Scheme, despite being considered standard treatment by medical experts.

The drug, medical oncology, has been used elsewhere but then

will go longer — one chap had 30 treatments."

Malignant mesothelioma is a cancer of the outer covering of the lung or abdominal cavity and is frequently associated with past exposure to asbestos.

The disease can be dormant for up to 25 years before symptoms develop, but it is very aggressive and painful.

Most people given chemotherapy in the 1970s.

Prominent scientist Ian

Breast cancer drug cleared for PBS

A DRUG costing breast cancer victims up to \$70,000 a year is to be subsidised by the Federal Government.

Health Minister Tony Abbott said Cabinet had agreed to place the breast cancer treatment Herceptin on the Pharmaceutical Benefits Scheme from October 1.

This would cost the

Government \$400 million over the next four years, and was expected to help about 2000 Australian women every year.

About 2500 Australian women die of breast cancer every year, and about 14,000 women are diagnosed with breast cancer.

Survival rates from breast cancer in Australia have been improving, but

the incidence has been rising.

Mr Abbott said studies had shown that women who had been able to use Herceptin had a 30 per cent greater survival rate.

"This is a good example of the fact that we are prepared to list very expensive drugs if we believe they are genuinely cost-effective," he said.

news.com.au/couriermail

... yesterday, with some even offering to donate money to the organisation.

Subsidy for Viagra rejected

IMPOTENCE treatments have been swept off the Howard Government's drug subsidy list amid concerns for the financial viability of the \$2.4 billion Pharmaceutical Benefits Scheme.

Health Minister Kay Patterson not only rejected Pfizer's application for Viagra to be listed on the subsidy scheme yesterday, but also removed a long-time listing for Viagra.

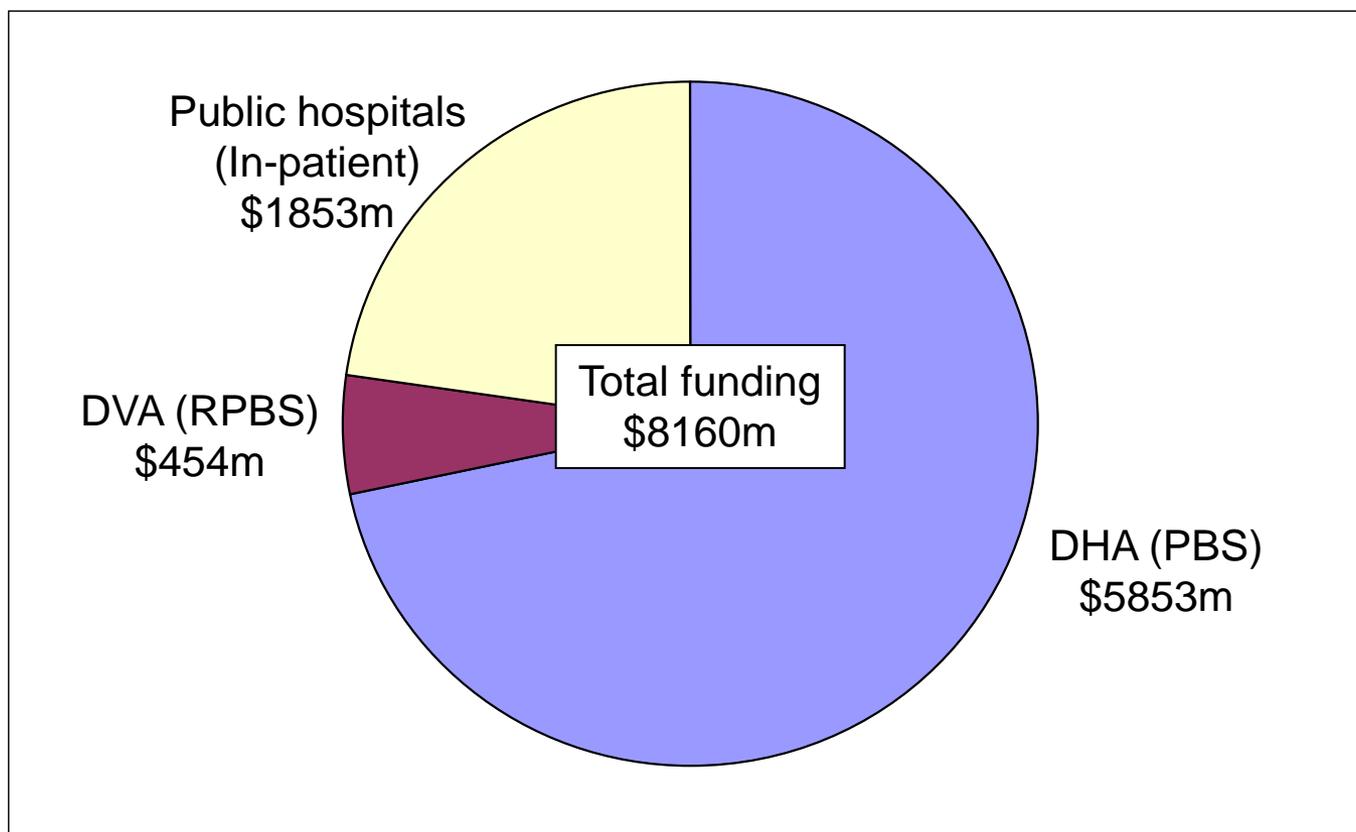
Why consider public preferences for pharmaceutical subsidy recommendations?

- Judgments are social as well as scientific in nature (Rawlins 2005)
- “Accountability for Reasonableness” framework for fair priority-setting: “Relevance” requires that the rationales for limit-setting decisions should appeal “to evidence, reasons, and principles that are accepted as relevant by fair-minded people...” (Daniels & Sabin 2002)
- Public consultation is consistent with the principles of procedural justice
- The public agree their preferences should be considered for “non-technical” aspects of priority-setting (Wiseman & colleagues 2003)

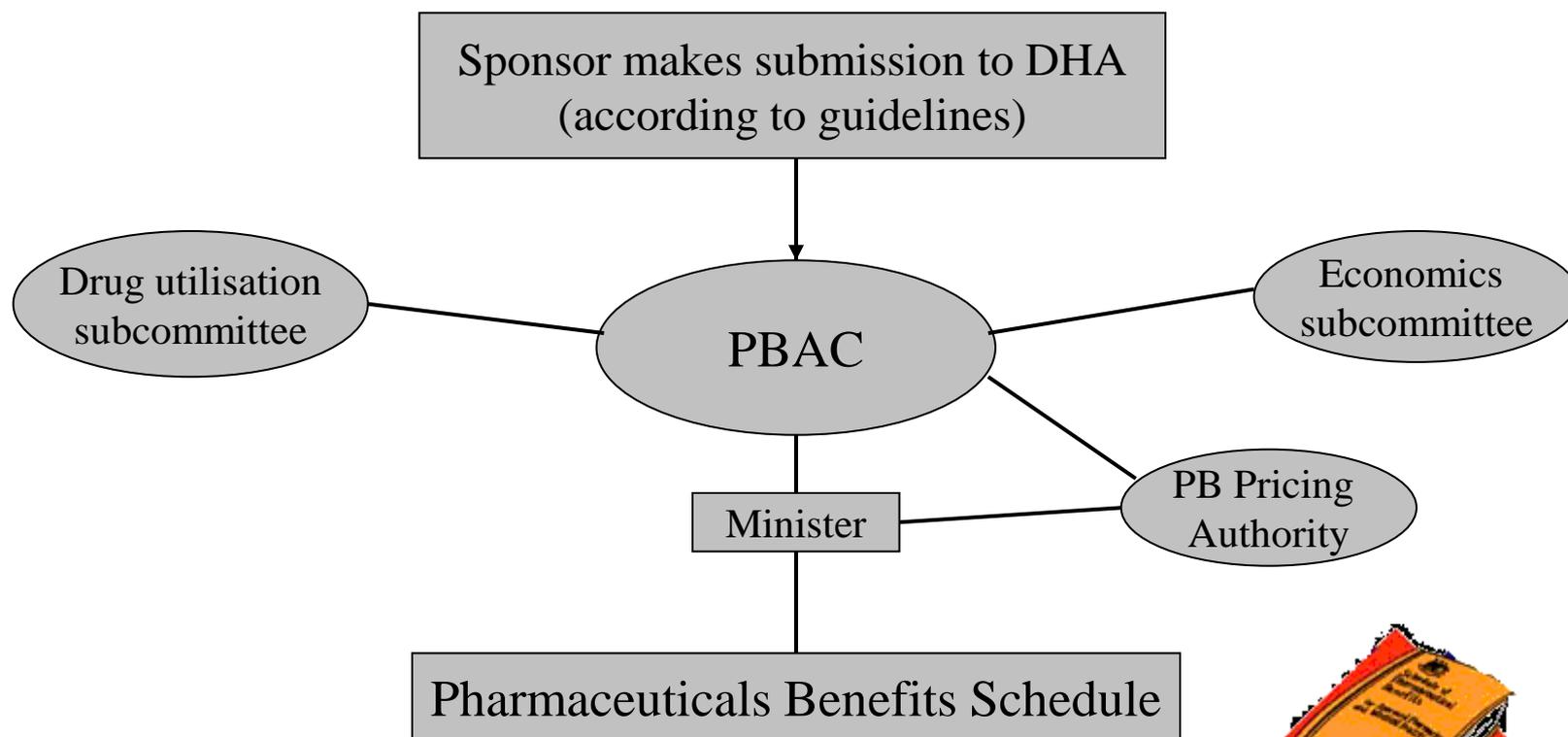
The Australian Pharmaceutical Benefits Scheme (PBS)

- Aims to provide reliable and affordable access to a wide range of necessary medicines
- Government subsidised, national “positive list”
- Patient contribution up to \$32.90 (general) \$5.30 (concession), plus any brand/therapeutic group premium

Government funding of expenditure on prescription medications 2006/7 (\$million)



Listing pharmaceuticals in the PBS



PBAC decision criteria

- *National Health Act 1953* – comparative effectiveness, cost and cost-effectiveness
- PBAC weighs these against other factors (Guidelines 2008)
 - e.g. Financial implications
 - Uncertainty
 - Equity
 - Available alternatives
 - Severity of condition
 - “Rule of Rescue”

Empirical evidence from past PBAC decisions

- Unlikely to reject $< \$42,000$ or recommend $> \$76,000$ per LYG (1991-6 data, 1998/9 values) (George & colleagues 2001)
- $\$69,897$ per QALY was highest ICER at which a drug was recommended (1994-2003 data) (Henry & colleagues 2005)
- Clinical significance, cost-effectiveness, cost to government, severity of disease significantly influenced decisions (1994-2004 data) (Harris & colleagues 2008)

What do the public think should be considered?

- Studies in the context of priority-setting in health care suggest criteria related to equity and fairness are important e.g. age, severity of illness, distribution of gain
- Cost or cost-effectiveness little explored
- Few studies specific to pharmaceutical subsidy context

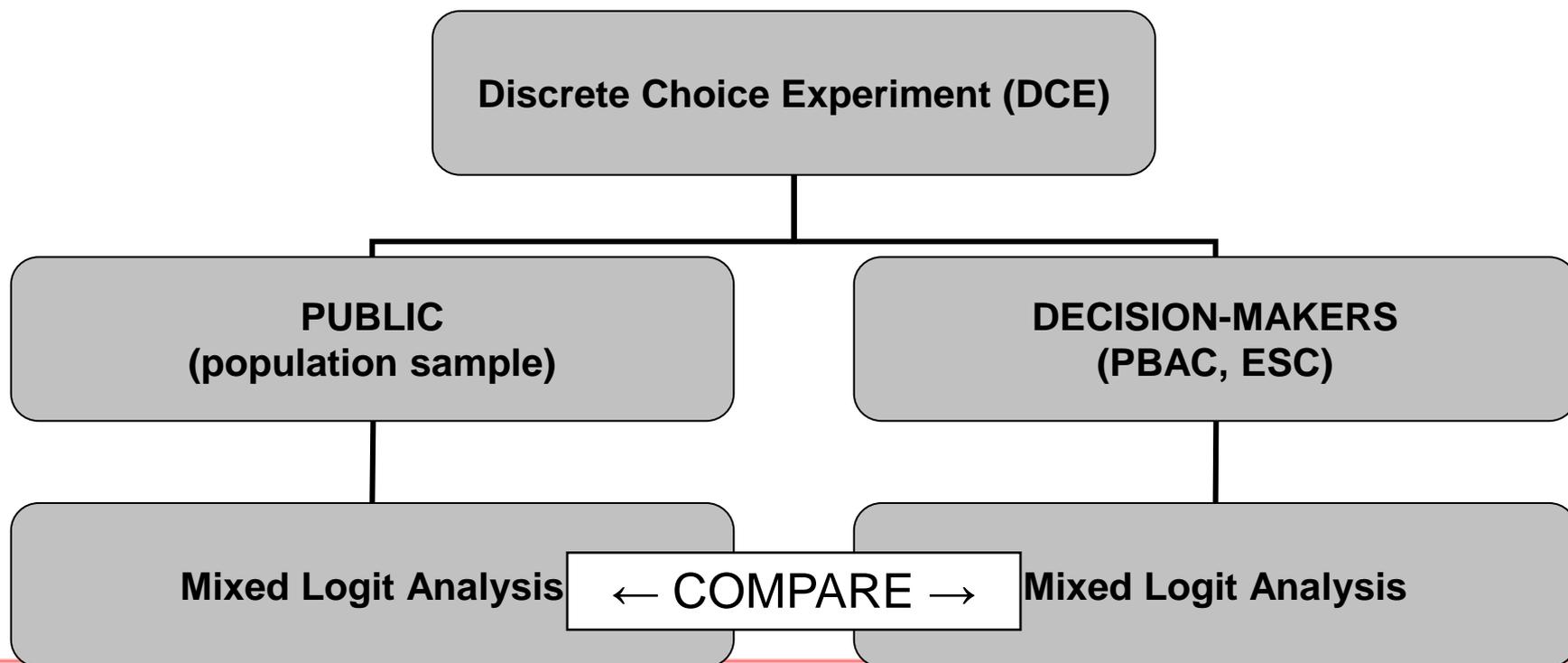
No previous research has directly addressed the consistency between public and decision-maker preferences

Aim

To evaluate the consistency of public and decision-maker preferences for the public subsidy of pharmaceuticals in the Australian context

...Specifically, to compare the relative importance of gains in survival, QoL, chance of response success and government costs, and the impact of severity of illness on preferences

Overview of methods



DCE – 3 severity of illness scenarios

Scenario	With current treatment	
	Expected survival:	Expected health state:
“A”	Short (3 months)	Poor (Extreme pain/discomfort)
“B”	Long (15 years)	Poor (Extreme pain/discomfort)
“C”	Long (15 years)	Moderate (Moderate pain/discomfort & moderate anxiety depression)

DCE Attributes and Levels

Attribute	Attribute levels		
	Scenario A	Scenario B	Scenario C
SUCCESS	60%, 90%	60%, 90%	60%, 90%
SURVIVAL	3 months, 1 yr, 10 yrs	15 yrs, 20 yrs, 25 yrs	15 yrs, 20 yrs, 25 yrs
QoL	No Pain/ Discomfort (1.0) Moderate Pain/ Discomfort (0.80) Extreme Pain/ Discomfort (0.26)	No Pain/ Discomfort (1.0) Moderate Pain/ Discomfort (0.80) Extreme Pain/ Discomfort (0.26)	No Pain/ Discomfort & Moderate Anxiety/ Depression (0.85) Moderate Pain/ Discomfort & No Anxiety/ Depression (0.80) Moderate Pain/ Discomfort & Moderate Anxiety/ Depression (0.73)
COST	\$1,000; \$10,000; \$100,000	\$1,000; \$10,000; \$100,000	\$1,000; \$10,000; \$100,000
UNCERTAINTY (decision-makers)	Low; High	Low; High	Low; High

Experimental design

- Separate design process for each scenario
- Fractional factorial, main effects design used
- Two generic alternatives (pharmaceutical A/B)
 - First alternative from orthogonal main effects plan
 - Second alternative using Shifted Design Technique (Bunch & colleagues 1996)
 - D-efficiency 100% for public scenario designs, 97.2% for decision-maker scenario designs
- Resulted in 27/48 choice sets for public/decision-makers

Pilot studies

Indicate ability to respond to choice sets, distinguish between attribute levels

An “opt out” option of “Neither” included following feedback from pilot studies to reduce risk of non-response and better reflect current practice

Sample introductory text (public scenario A)

Two new prescription medications have been developed. They are suitable for use in a group of 5,000 adults per year who all have the same medical condition. The average age of the adults is 50 years. With current treatment, which is effective in all adults, the adults will on average live for another **3 months** with **extreme pain/discomfort**. The two new medications vary in terms of the chance that the treatment will be successful, and the average length of life and quality of life the adults will have after successful treatment. The average additional cost to the government per person treated also varies, and this cost applies regardless of whether or not treatment was successful.

The government has a limited budget and only one of the medications can be funded from money raised via public taxes. Each person will receive the chosen medication; that is, either medication A or medication B. If neither medication is funded, or if the new medication is unsuccessful, the adults will still be able to receive the current treatment, which you can assume to have no additional costs.

Sample choice set (public scenario A)

Medication A		Medication B
90%	Chance of success	60%
1 year	Survival	10 years
No pain/discomfort	Quality of life	Extreme pain/discomfort
\$100,000	Additional Cost (per person treated)	\$10,000
Please tick one box. I would prefer the government to fund:		
Medication A <input type="checkbox"/>	Neither <input type="checkbox"/>	Medication B <input type="checkbox"/>

Sample choice set (decision-maker A)

Pharmaceutical A		Pharmaceutical B
60%	Chance of success	90%
10 years	Survival	3 months
Moderate pain/discomfort (Utility weight 0.80)	Quality of life	No pain/discomfort (Utility weight 1.00)
\$100,000	Incremental Cost (per person treated)	\$10,000
High	Uncertainty	Low
Please tick one box. I would prefer the government to fund:		
<i>Pharmaceutical A</i> <input type="checkbox"/>	<i>Neither</i> <input type="checkbox"/>	<i>Pharmaceutical B</i> <input type="checkbox"/>

Data collection

Ethical clearance obtained

Self-administered questionnaire, mailed to:

Public

- The “resident” (age \geq 18 yrs) at a quasi-random sample of 1,000 households identified from electronic white pages

Decision-makers

- 37 current or past (since 2000) members of PBAC and/or ESC

Mixed Logit (MXL) analysis

Gaining popularity for analysis of DCEs:

- Accommodates preference heterogeneity
- Permits error correlation between alternatives
- Permits error correlation across multiple choice responses from each respondent

(For other examples of MXL analysis of DCEs in a health care setting see e.g. Hall et al 2006, Howard & Salkeld 2008, Johnson et al 2000, King et al 2007, Kjaer & Gyrd-Hansen 2008; Lancsar et al 2007)

Model estimation in NLOGIT 4.0

Alternatives j= pharmaceutical A, pharmaceutical B:

$$V_j = \beta_0 + \beta_1 \text{CSURV} + \beta_2 \text{CSTATE} + \beta_{\text{succ}} \text{SUCCESS} + \beta_{\text{surv}} \text{SURVIVAL} + \beta_{\text{qwt}} \text{QALYWEIGHT} + \beta_{\text{cost}} \text{COST} + \beta_{\text{uncert}} \text{UNCERTAINTY} + \varepsilon_j$$

β_0	– alternative specific constant representing choice of new pharmaceutical (A/B)
CSURV, CSTATE	– initial survival / health state associated with current treatment (effects coding)
SUCCESS, SURVIVAL, QALYWEIGHT, COST	– attribute levels (continuous coding)
UNCERTAINTY (decision-makers)	– attribute level (effects coding)
ε	– error term

Utility for “neither” alternative normalised to zero

Random parameters

COST parameter fixed *a priori*, remaining attribute parameters and constant specified as random

All random parameters were specified with the individual heterogeneity weights following a standard normal distribution

Error correlation introduced across each group of 27 (public) or 48 (decision-maker) choice responses

Models estimated using 1,000 simulated Halton draw replications

Response

- 161 (16.3%) public provided 4333 valid observations
- 11 (29.7%) decision-makers provided 526 valid observations
- “Neither” pharmaceutical was chosen for 14% and 12.9% of choice sets by the public and decision-makers respectively

Public respondent characteristics (n = 161)

Characteristic	Category	N (valid%) or Mean (SD)	
Age (yrs)		Mean 53.55 (SD 14.47)	
Gender	Female	93	(58.5%)
Education	Degree / Professional qualification	84	(52.5%)
Household income (\$)	Up to 25,000	21	(13.9%)
	25,001-50,000	30	(19.9%)
	50,001-75,000	34	(22.5%)
	75,001-100,000	28	(18.5%)
	100,001-125,000	23	(15.2%)
	>125,000	15	(9.9%)
State	NSW/ACT	34	(21.5%)
	Victoria	34	(21.5%)
	Queensland	43	(27.2%)
	South Australia	21	(13.3%)
	Western Australia	23	(14.6%)
	Tasmania	3	(1.9%)
Accessibility/Remoteness Index of Australia (ARIA) Code	Highly Accessible	132	(83.5%)
	Accessible	20	(12.7%)
	Moderately Accessible	5	(3.2%)
	Remote or Very remote	1	(0.6%)
Private health insurance			
	Hospital cover	Yes	115 (72.3%)
	Extras cover	Yes	93 (66.0%)

Decision-maker respondent characteristics (n=11)

- 9 current / past PBAC members
- 5 current / past ESC members
(3 were in both categories)

Estimated model - public

Variables	Unit	MXL	
		β	σ
Random parameters			
CONSTANT	new	-4.7419 ***	2.9567 ***
SUCCESS	1%	0.0195 ***	0.0132 ***
SURVIVAL	1yr	0.1238 ***	0.0524 ***
QALY WEIGHT	0.1	0.6012 ***	0.2521 ***
Fixed parameters			
COST	\$1,000	-0.0086 ***	
CURRENT SURVIVAL	short	0.4577 ***	
CURRENT HEALTH STATE	poor	0.4072 ***	
df		4321	
Pseudo-R ²		0.4208	
AIC		1.2775	
Log Likelihood		-2756.80	

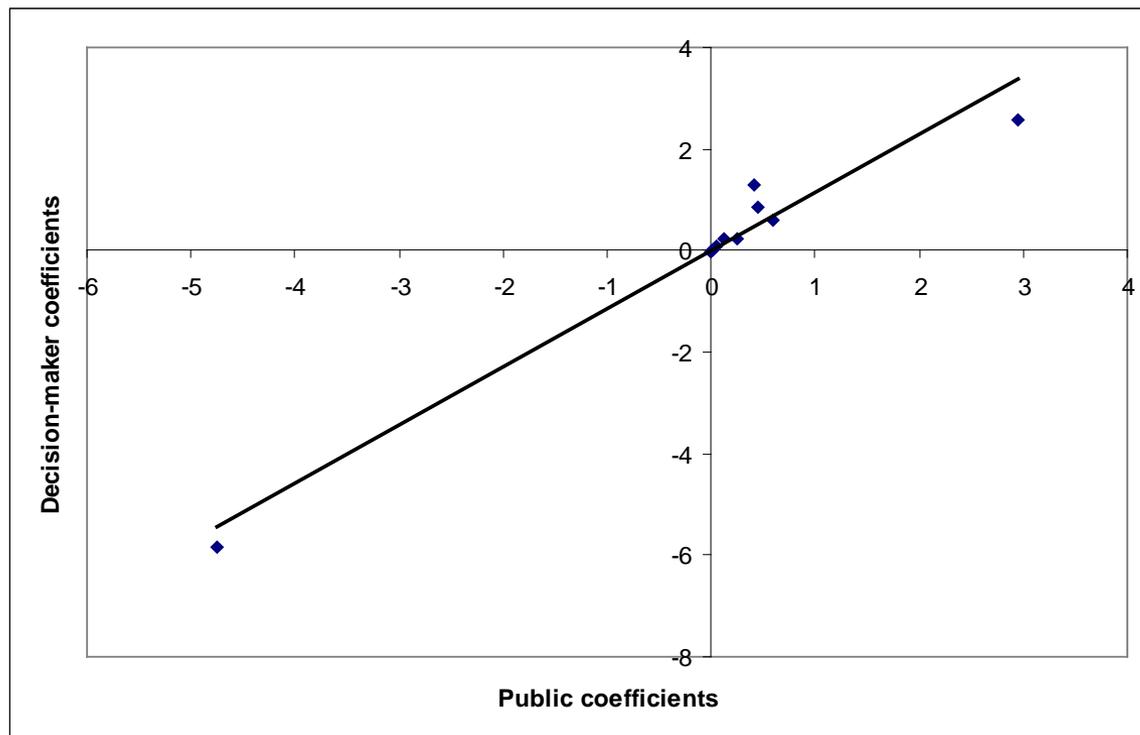
*** p<0.0001

Estimated model – decision-maker

Variables	Unit	MXL	
		β	σ
Random parameters			
CONSTANT	new	-5.8283 ***	2.5662 **
SUCCESS	1%	0.0262 ***	0.0080 ns
SURVIVAL	1yr	0.2309 ***	0.0726 *
QALY weight	0.1	0.5898 ***	0.2151 ***
UNCERTAINTY	high	-0.2663 *	0.3087 **
Fixed parameters			
COST	\$1,000	-0.0270 ***	
CURRENT SURVIVAL	short	0.8372 *	
CURRENT HEALTH STATE	poor	1.3080 ***	
df		512	
Pseudo-R ²		0.5062	
AIC		1.1344	
Log Likelihood		-285.34	

*** p<0.001 ** p<0.01 *p≤0.05 ns not significant (p>0.05)

Comparison of public and decision-maker preferences - scatter plot



$R=0.981, p<0.001$

Willingness for the government to pay

Gain	Willingness for the government to pay for gain						Ratio public / decision-maker WGTP
	Public			Decision-makers			
	Mean	95% CI		Mean	95% CI		
		Lower	Upper		Lower	Upper	
1% chance of success	\$2,271	\$1,751	\$2,790	\$969	\$512	\$1,426	2.3
1 year survival	\$14,417	\$12,048	\$16,786	\$8,552	\$5,957	\$11,147	1.7
0.1 QALY weight	\$70,005	\$59,176	\$80,834	\$21,844	\$13,676	\$30,011	3.2

Implications of findings

- Suggests a high level of agreement between public and decision-maker preferences
 - Relative importance of success, survival, QoL, cost
 - Preference to treat severe illness
- Confirms public (and decision-makers) are willing to limit the price government should pay for health gain from pharmaceuticals

Finding of consistency requires confirmation

- In a larger more representative public sample
- May be limited by differences in public & decision-maker instruments
- In a design including interaction effects

Future Research

- Are the aggregate stated preferences of individual committee members a valid indicator of the actual decision-making preferences of the group as a single decision-making entity?
- There is a need for a structured research program (methodological and applied) to elicit public preferences for subsidy decisions

Conclusion

- The findings of this DCE provide an early indication of consistency between public and decision-maker stated preferences for pharmaceutical subsidy decisions
- Further research into the consistency of public preferences with both decision-maker stated preferences and actual decisions is required