# Health care policy evaluation: empirical analysis of the restrictions implied by Quality Adjusted Life Years

Rosalie Viney and Elizabeth Savage
CHERE, University of Technology, Sydney

Preliminary – not for quotation

# Health care policy evaluation

- Resource allocation decisions in health care increasingly based on cost-effectiveness analysis
  - Australia
    - Pharmaceutical Benefits Advisory Committee (PBAC)
    - Medicare Services Advisory Committee (MSAC)
  - United Kingdom
    - National Institute of Clinical Excellence (NICE)
  - Canada (Ontario), Denmark, Finland, Netherlands, Sweden
    - All have formal requirements for economic evaluation for reimbursement decisions for pharmaceuticals
  - United States
    - Discussion of a PBAC type process for new pharmaceuticals

# Example: Pharmaceutical Benefits Scheme

- PBAC required by legislation to consider cost-effectiveness (among other factors)
- Company seeking PBS listing for a drug must submit an economic evaluation
  - Evidence of clinical effectiveness
  - Estimate potential health gain from new drug relative to current treatment (measure of effectiveness)
  - Estimate additional cost of new drug relative to current treatment
    - proposed price for new drug
    - standardised "prices" for other health services
  - Calculate incremental cost-effectiveness ratio

# Examples of PBAC Decisions

**Incremental cost per additional Quality Adjusted Life Year gained "league table"**

| Submission | $/QALY | Recommendation |
|---|---|---|
| 1 | $4,690 | Recommend |
| 2 | $5,244 | Recommend |
| 3 | $8,570 | Recommend |
| 4 | $10,530 | Recommend |
| 5 | $13,121 | Recommend |
| 6 | $17,937 | Not Recommend |
| 7 | $21,225 | Recommend |
| 8 | $24,343 | Recommend |
| 9 | $133,337 | Reject |

Source: George B, Harris A and Mitchell A. Cost effectiveness and consistency of decision making: evidence from pharmaceutical reimbursement in Australia (1991-1996). *Pharmacoeconomics* 19:1103-1109 2001.

# Measures of health gain (effectiveness)

- Ideally want measures that are comparable across different diseases and treatments
- "Intermediate"/ "surrogate" (clinical) outcome not comparable
- Life years saved
- Quality adjusted life years saved (QALYs)

# Estimating health gain

- Decision tree framework
- Epidemiological information used to identify
  - disease states
  - outcomes of treatment
  - probability of different outcomes (proportion of a population experiencing the outcome)
  - survival (durations) associated with different outcomes
  - quality of life associated with different outcomes
- Estimate expected life years associated with each alternative (new treatment, current treatment)
- Not all years of life have the same value (quality)

# Quality Adjusted Life Years

- “Quality Adjustment” reflects valuation of poorer health states relative to “full health”
- QALYs calculated by weighting survival time associated with each treatment outcome by a QALY weight
- QALY weight of 1 for a year of life in “full health”
- QALY weights generally lie between 0 (death) and 1 (full health)
  - “Worse than death” health states permissible
- QALYs measure strength of preference for survival and quality of life and trade-offs between the two

# Example: QALY Weights

| | |
|---|---|
| AIDS, CD4 count range 0-50 | 0.79 |
| Arthritis, before treatment | 0.609 |
| Arthritis, after treatment | 0.647 |
| Cancer, breast, after surgery, first recurrence | 0.85 |
| Cancer, breast, after surgery, third recurrence | 0.3 |
| Cancer, prostate, metastatic, early progressive disease | 0.83 |
| Proctitis/cystitis after randiation therapy for prostate cancer | 0.9 |
| Stroke, severe, motor deficit | 0.03 |
| Stroke, mild cognitive deficit | 0.54 |

Source: Tengs, T and Wallace A. One Thousand Health-Related Quality of Life Estimates *Medical Care* 38(6) 583-637, 2000

UNIVERSITY OF TECHNOLOGY SYDNEY



CHERE CENTRE FOR HEALTH ECONOMICS RESEARCH AND EVALUATION

# Economic underpinnings of QALYs

- Health care can be characterised as lotteries over profiles of consumption and health

$$U = U\left(\left(h_1^1, c_1^1\right), \ldots, \left(h_T^1, c_T^1\right); p^1, \ldots, \left(h_1^m, c_1^m\right), \ldots, \left(h_T^m, c_T^m\right); p^m\right)$$

- Treatments modify the set of lotteries

- Individuals will have preference orderings over alternative treatments

- QALY model implies restrictions on form of utility function

# Outline

- Describe methods used to derive QALYS
- Outline the restrictions on preferences imposed by the QALY model
- Describe a discrete choice experiment designed to investigate preferences for health care
- Test restrictions on preferences implied by the QALY model

# QALYs

- Measure the value of a particular health state relative to full health, ignoring consumption
- Under the QALY model

$$U\left(h_1, h_2, \ldots, h_T\right) = \sum_{t=1}^{T} v\left(h_t\right)$$

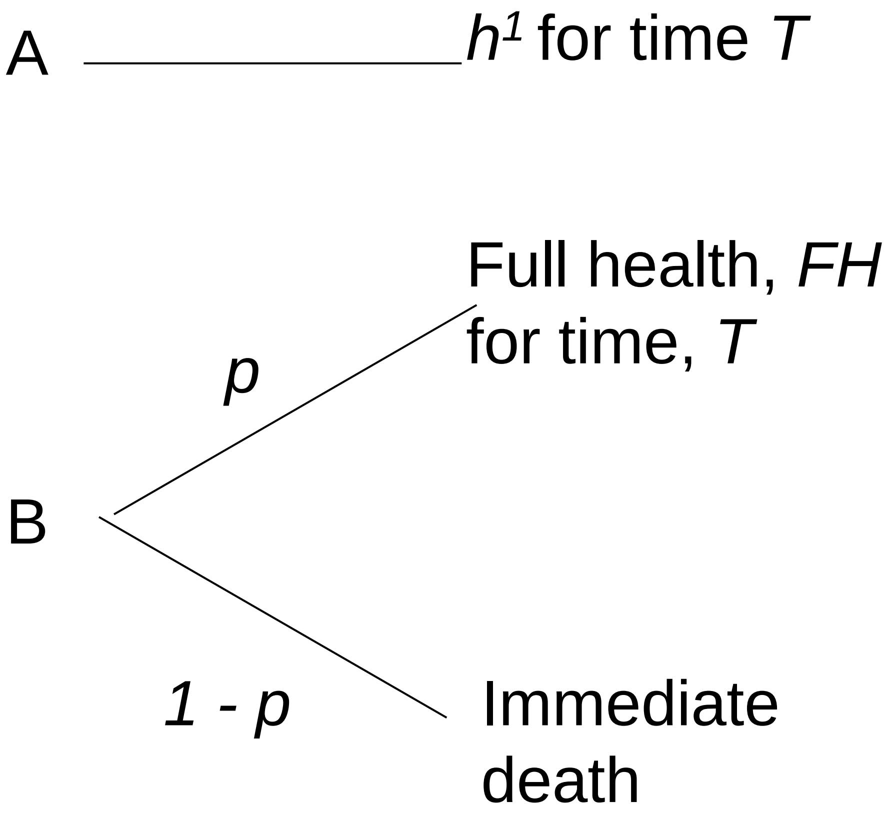
where $v(h_t)$ is the quality (utility) weight of health state in period $t$

- ie identical additive separable function in each period

# Quantifying QALY weights

- Involves experiments using standard gambles and time trade-offs
- These rely on restrictions on preferences over consumption and health status over time.

# QALY model: Standard gamble

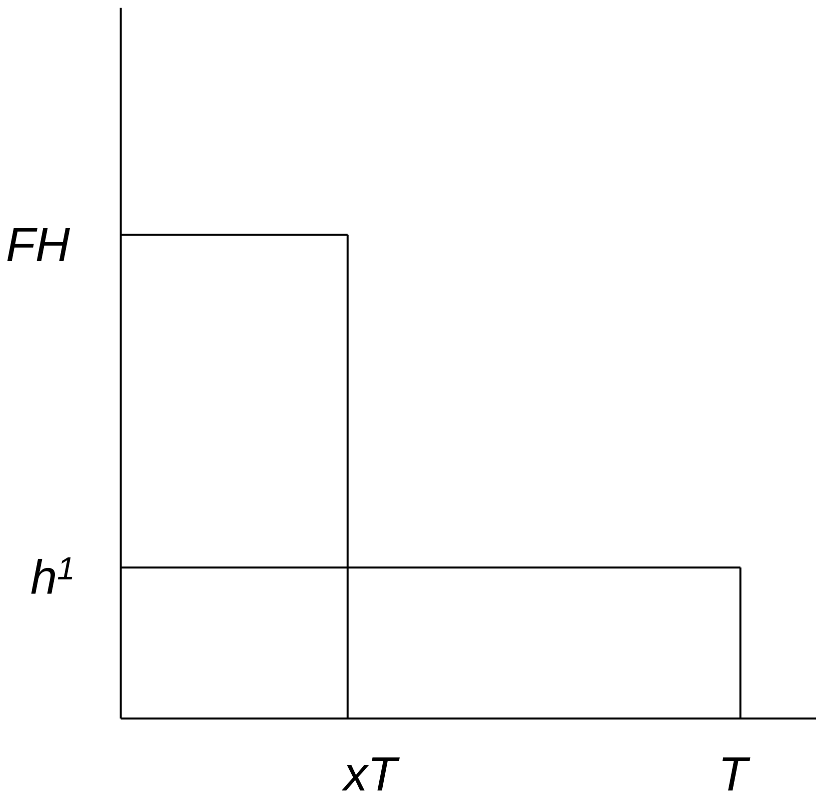



- Specify $h^1$
- Find p such that utility of the gamble, B, is equal to A - the certainty of health state $h^1$ for $T$ years
- ie

$$p.v(FH)T + 0 = v(h^1)T$$
$$\Downarrow$$
$$p = v(h^1)/v(FH)$$

- Normalise: $v(FH) = 1$

# QALY model: Time trade-off




- Find x such that utility of the health state 1 for time *T*, $h^1$ is equal to the utility of full health for a proportion *x* of *T* years

  ie

$$v\left(h^1\right).T = v\left(FH\right).xT$$

$$\Downarrow$$

$$x = v\left(h^1\right) / v\left(FH\right)$$

# Note:

- Only attributes of the health state and time enter the experiment
- In both cases the experiment sets *T*
- Individual responses are averaged to determine the QALY weight attached to health state 1

# QALY model: Application

- Say a treatment improves health status for a proportion, $p_1$, of the population undergoing the treatment from
  - $h_1$ (with QALY weight $Q_1$) to
  - $h_2$ (with QALY weight $Q_2$) and
  - lengthens expected survival time from $T_1$ to $T_2$
- The effectiveness per individual in terms of QALYs gained is equal to $p_1 (Q_2 T_2 - Q_1 T_1)$
- $p_1, h_1, h_2, T_1, T_2$ are derived from clinical data
- $Q_2$ and $Q_1$ are derived from the TTO/SG experiment;
  - $T$ in the experiment is unrelated to $T_1$ or $T_2$;
  - $p$ in the experiment unrelated to $p_1$.

# QALYs: Policy Evaluation

- Effectiveness measured by QALY gain combined with cost to give a C/E ratio.
- If the C/E ratio lies below some threshold level, the intervention is accepted.
- The threshold indicates willingness to pay per QALY.
- Setting the threshold effectively monetises effectiveness.

# QALY Restrictions

Under what restrictions on preferences is cost-effectiveness analysis based on QALYs likely to lead to health care resource allocation that reflects individuals' preferences?

# Literature

- Pliskin, Shephard and Weinstein (1980)
- Bleichrodt, Wakker and Johannesson (1997)
- Miyamoto, Wakker, Bleichrodt and Peters (1998)
  - Preferences defined over a constant health state and survival
- Bleichrodt and Quiggin (1997)
  - Preferences defined over non-constant health states over time
  - Expected utility and Rank dependent expected utility
- Bleichrodt and Quiggin (1999)
  - Preferences defined over non-constant health states and consumption over time

# QALY restrictions: B&Q (1999)

- VNM expected utility
- Marginality
- Symmetry
- Standard gamble invariance
- Zero condition

# QALY restrictions: marginality

- Let $y_G$= (\$20, $H_1$); $y_B$= (\$5, $H_2$);     $H_1 > H_2$
- With marginality an individual is indifferent between
  - G1: ($y_B$, $y_B$) with prob=1/2 and ($y_G$ , $y_G$ ) with prob=1/2
  - G2: ($y_B$, $y_G$ ) with prob=1/2 and ($y_G$, $y_B$ ) with prob=1/2
- Both G1 and G2 have the same probability of $y_G$ in both periods and of $y_B$ in both periods.
- Marginality excludes all complementarity across time periods, such as a dislike for variation or a desire to avoid a bad outcome in both periods.

# QALY restrictions: symmetry

- Requires that resequencing does not change utility:

$$U\left(y^1, y^2, \ldots y^T\right) = \sum_{t=1}^{T} U\left(y^t\right)$$

- Inconsistent with positive time preference.

# QALY restrictions: standard gamble invariance

- For all levels of consumption, *c and c'*

$$(c,h) \succ [(c,h'),p;(c,h''),1-p]$$

$$\Rightarrow$$

$$(c',h) \succ [(c',h'),p;(c',h''),1-p]$$

- Utility derived from the health state unaffected by the level of consumption

# QALY restrictions: zero condition

- Less controversial

$$U\left(c,\text{death}\right)=U\left(c',\text{death}\right)=0$$

# Under QALY restrictions

$$U = U\left(\left(h_1^1, c_1^1\right), \ldots, \left(h_T^1, c_T^1\right); p^1, \ldots, \left(h_1^m, c_1^m\right), \ldots, \left(h_T^m, c_T^m\right); p^m\right)$$
$$= \sum_{i=1}^{m} p_i \sum_{t=1}^{T} z(c) v(h_t)$$

- *z(c)* a constant
- i.e. Implies constant consumption over time

UNIVERSITY OF TECHNOLOGY SYDNEY



CHERE CENTRE FOR HEALTH ECONOMICS RESEARCH AND EVALUATION

# Implications of QALY restrictions
# EU: linear in probabilities

- Evaluation function for gambles is linear in probabilities.
- No complementarity across time periods.
- The utility function over sequences of consumption and health state is additively decomposable over time
- One-period utility functions are the same.

# RDEU: probability weighting function

- Changes the marginality condition to generalised utility independence – less strict.

# Discrete choice experiments and health care

- DCEs widely used in transport economics, environmental economics and marketing
- Used in health economics to:
  - Explore trade-offs between attributes of health care interventions
  - Provide monetary valuations of health care interventions
  - Predict uptake of new programs
- Discrete choice experiments can provide data to explore the underlying utility function for health and health care, particularly trade-off between quality of life and survival (QALYs)

# DCE Methods

- Stated preference surveys
- Choose preferred alternative from a series of hypothetical choice sets
- Alternatives described in terms of attributes
- Attributes varied over plausible range of levels of analytical interest
- Experimental design principles used to choose a sample of choice sets to allow for efficient estimation of parameters of interest

# DCE Methods: Binary Choice

$$P_i(j) = \Pr(U_{ij} \geq U_{ik})$$

$$U_{ij} = V_{ij} + \varepsilon_{ij}$$

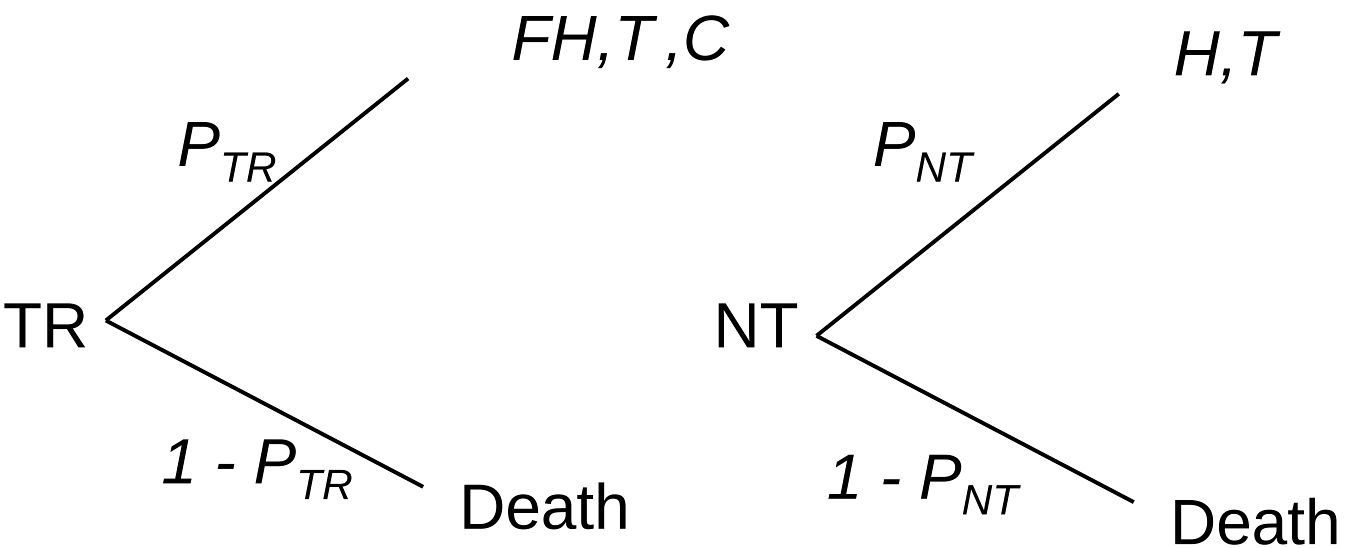
$$P_i(j) = \Pr\left(\left(V_{ij} + \varepsilon_{ij}\right) \geq \left(V_{ik} + \varepsilon_{ik}\right)\right)$$

$$= \frac{e^{\mu V_{ij}}}{e^{\mu V_{ij}} + e^{\mu V_{ik}}}$$

$$= \frac{e^{\mu \beta' \mathbf{x}_{ij}}}{e^{\mu \beta' \mathbf{x}_{ij}} + e^{\mu \beta' \mathbf{x}_{ik}}}$$

# Discrete choice experiment to investigate health care preferences

- Choice between two alternatives: treatment (TR) and non-treatment (NT) for a hypothetical health condition.



# Attribute levels

- Each alternative has three, four-level, alternative-specific attributes.
- Cost of “non treatment” is zero
- Health state from treatment is full health or death

| PTR Probability of survival with treatment | TTR Life expectancy if survive | PRICE Price of treat-ment | PNT Probability of survival without treatment | TNT Life expectancy without treatment | HS Health state without treatment |
|---|---|---|---|---|---|
| 54% | 10 years | $20,000 | 39% | 10 years | HS1 |
| 69% | 20 years | $40,000 | 59% | 20 years | HS2 |
| 84% | 30 years | $60,000 | 79% | 30 years | HS3 |
| 99% | 40 years | $80,000 | 99% | 40 years | HS4 |

# Health states

- Generic health states from EQ-5D multi-attribute utility instrument
- Selected to be ordered, cover the range of health states and to “make sense”

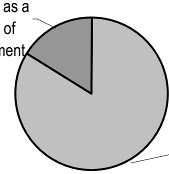
| | HS1 | HS2 | HS3 | HS4 | FH |
|---|---|---|---|---|---|
| **Mobility** | 3 | 2 | 2 | 2 | 1 |
| **Self care** | 3 | 2 | 1 | 1 | 1 |
| **Usual Activities** | 3 | 3 | 2 | 1 | 1 |
| **Pain/Discomfort** | 3 | 2 | 2 | 2 | 1 |
| **Anxiety/Depression** | 3 | 3 | 2 | 1 | 1 |

**You have a health condition that affects your quality of life and your life expectancy. There is a treatment available for your condition and you must decide whether to have treatment or not.**

## If you do have treatment

The out of pocket cost to you is $20,000

The chances of having successful treatment or dying are:




If treatment is successful your life expectancy is 30 years

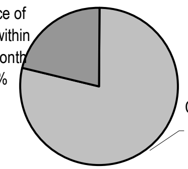

Successful treatment will give you full health which means you have:

No problems in walking about
No problems with self care
No problems peforming usual activities
No pain or discomfort
Not anxious or depressed

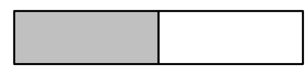
## If you do not have treatment

There is no out of pocket cost to you

The chance of surviving or dying with this condition are:




If you survive your life expectancy is 20 years




Your health is affected by this condition in the following ways:

Some problems in walking about
No problems with self care
Some problems in performing usual activities
Moderate pain or discomfort
Moderately anxious or depressed

**Would you have treatment?**

**Yes** ☐ **No** ☐

# Survey design and data collection

- Six 4-level attributes = 4096 choice sets, 64 for each alternative
- 192 choice sets selected from the full factorial
- Blocked into 12 separate questionnaires, each with 16 choice sets
- Random sample aged 18-60 from Sydney metropolitan area
- door-to-door recruitment; self-completion questionnaires
- 347 respondents in total

# Data

- Treatment was the preferred alternative in 65.1% of observations
- Two choice sets for which 100% of respondents chose treatment
- 9.6% of respondents always chose treatment
- 3.7% of respondents always chose no treatment
- Remaining respondents responsive to changing alternatives

# Methods: Testing QALY model QALY restricted specification

- Expected utility is given by the sum of the product of probability, time and QALY weight.
- In log form and imposing the QALY restrictions gives

$$\ln U_j = \ln P_j + \ln T_j + \varphi_j(H_j) + \kappa(C_j) + \varepsilon_j \quad \text{where j} = \begin{cases} \text{TR} \\ \text{NT} \end{cases}$$

- For rank dependent expected utility $P$ is replaced by a probability weighting function $g(P)$ ; the simplest specification is

$$\ln g(P) = \gamma \ln P$$

# Methods: Testing QALY model Unrestricted specification

- With the zero condition the general form is

$$\ln U_j = \ln g(P_j) + \upsilon_j(H_j, T_j, C_j) + \varepsilon_j \quad \text{where j} = \begin{cases} \text{TR} \\ \text{NT} \end{cases}$$

with

$$\upsilon_j(.) = \alpha_j + \beta_{jT} \ln T_j + \sum_k \beta_{jH_k} H_{jk} + \beta_{jC} C_j + \sum_k \beta_{jH_kT} H_{jk} \ln T_j +$$

$$\sum_K \beta_{jH_kC} H_{jk} C_j + \beta_{jCT} C_j \ln T_j + \beta_{C^2} C_j^2$$

UNIVERSITY OF TECHNOLOGY SYDNEY



CHERE CENTRE FOR HEALTH ECONOMICS RESEARCH AND EVALUATION

# Notes

- Health states under non-treatment are ordered and dummy-effects-coded in the estimation.
- Loss of consumption is proxied by cost of treatment.
- Constant is zero for non-treatment.
- Cost is zero for non-treatment.
- The impact of full health with treatment is in the constant.
- Expansion is up to quadratic terms.

# Logit results

| | Model 1 (unrestricted) | | Model 2 (QALY) | |
|---|---|---|---|---|
| **Variable** | **Coef** | **P>\|z\|** | **Coeff** | **P>\|z\|** |
| Cons | -0.3292 | 0.701 | 0.941 | 0.000 |
| LnPtr | 1.6919 | 0.000 | - | - |
| LnTtr | 0.7157 | 0.000 | - | - |
| Cost | -0.0104 | 0.362 | -0.008 | 0.000 |
| LnTtr_Cost | -0.0018 | 0.500 | - | - |
| Costsq | 0.0001 | 0.310 | - | - |
| LnPnt | 1.4504 | 0.000 | - | - |
| LnTnt | 0.5933 | 0.000 | - | - |
| HS1 | -0.2192 | 0.537 | -0.853 | 0.000 |
| HS2 | 0.4063 | 0.210 | -0.168 | 0.001 |
| HS3 | -0.4169 | 0.185 | -0.386 | 0.000 |
| LnTnt_HS1 | -0.2050 | 0.068 | - | - |
| LnTnt_HS2 | -0.1841 | 0.075 | - | - |
| LnTnt_HS3 | 0.2573 | 0.010 | - | - |
| LnProb | - | - | 1.501 | 0.000 |
| LnTime | - | - | 0.610 | 0.000 |
| Log likelihood | -3226.699 | | -3233.59 | |

UNIVERSITY OF TECHNOLOGY SYDNEY

CHERE CENTRE FOR HEALTH ECONOMICS RESEARCH AND EVALUATION

# Tests of QALY model restrictions

- Equal coefficients on common TR and NT variables
- No interaction effects
- Jointly test zero coefficients on interaction variables
- Coefficients on probability and time variables = 1

# Test of equal coefficients on common TR and NT variables

( 1) $\beta_{Ptr} = \beta_{Pnt}$

chi2(1) = 2.36

Prob > chi2 = 0.1241 CANNOT REJECT

( 2) $\beta_{Ttr} = \beta_{Tnt}$

chi2( 1) = 0.62

Prob > chi2 = 0.4300 CANNOT REJECT

# LR test on interaction effects

$H_0$: No interaction effects

| | |
|---|---|
| Full model | Log L = -3226.6992 |
| Constrained model | Log L = -3232.6693 |

LR test: $\chi^2 = 11.9402$

$$\chi^2_{crit,0.05} = 11.0705$$

REJECT

# Test for zero interactions in constrained model

- Coefficients on interaction terms = 0
  - lnrttr_rcost
  - rcostsq
  - lnrtnt_hsfx1
  - lnrtnt_hsfx2
  - lnrtnt_hsfx3

- chi2( 5) = 10.83
  Prob > chi2 = 0.0549 REJECT (JUST)

# Test: utility function linear in probability and time

( 1) ln(*Prob*) = 1

chi2( 1) = 39.56

Prob > chi2 = 0.0000 REJECT

( 2) ln(*Time*) = 1

chi2( 1) = 46.95

Prob > chi2 = 0.0000 REJECT

# Conclusions

- Empirical analysis of this DCE does not provide support for the QALY restrictions.
- Suggests a more general evaluation function is required for resource allocation in health.

# Why the dominance of QALY model?

- Monetary measures of welfare gain seen as ethically troubling.
- B&Q: QALY model does not avoid ethical judgements.
- Explicit treatment of distribution is preferable.