Introduction to Effectiveness, Patient Preferences, and Utilities

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HERC Cost Effectiveness Analysis Course

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HERC
Health Economics Resource Center

VA
U.S. Department of Veterans Affairs
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Overview

- Outcomes measurement in CEA
- Concept of QALYs for a CEA
- Estimating QALYs
- Guidelines on selecting measures
- Issues surrounding QALYs
- References for more details

The ICER

CEA compares the outcomes and costs of two (or more) interventions

$$\frac{(Cost_{treatment} - Cost_{control})}{(Outcomes_{treatment} - Outcomes_{control})}$$

CEA/CUA review

- Compare outcomes and costs across interventions
  - Outcome defined by the health benefit achieved with the intervention.
  - Outcome(s) quantified in a single scale

Which outcome to use?

1) Mortality/life years gained

- ✓ Primary objective is to extend life (e.g. cancer therapies)
- ✓ Generic outcome across life-saving interventions
- \- Does not capture QoL or patient preferences

Which outcome to use?

2) Morbidity/disease specific outcomes

- ✓ Choosing among therapies for same condition
- ✓ More practical in clinical trials
- - Limits comparisons between other types of interventions

Which outcome to use?

3) Quality adjusted life year (QALY)

- ✓ Combines both quantity and quality of life in one generic measure
- ✓ Takes into account patient preferences
- ✓ Many guidelines recommend using QALYs

What is a QALY?

- Measure of a person's length of life weighted by a valuation of their HRQoL

Length of life

x

Quality of life valuations (health utilities)

How to Interpret QALYs

- 1 year in full health = 1 QALY
- 1 year in health state 0.5 = 0.5 QALYs
- Dead = 0 QALYs
- Negative values possible

QALY Example #1

- Prophylactic antibiotic Rx vs. standard of care

	3 mo.	3 mo.	3 mo.	3 mo.	Total QALYs
New Txt.	.50	.60	.80	.80	?
UC	.50	.35	.50	.80	?

QALY Example #1

- Prophylactic antibiotic Rx vs. standard of care

	3 mo.	3 mo.	3 mo.	3 mo.	Total QALYs
New Txt.	.50 (.50 x .25) .125	.60 (.60 x .25) .15	.80 (.80 x .25) .20	.80 (.80 x .25) .20	(.125+.15+.20+.20) =.675
UC	.50 (.50 x .25) .125	.35 (.35 x .25) .0875	.50 (.50 x .25) .125	.80 (.80 x .25) .20	(.125+.0875+.125+.20) =.5375

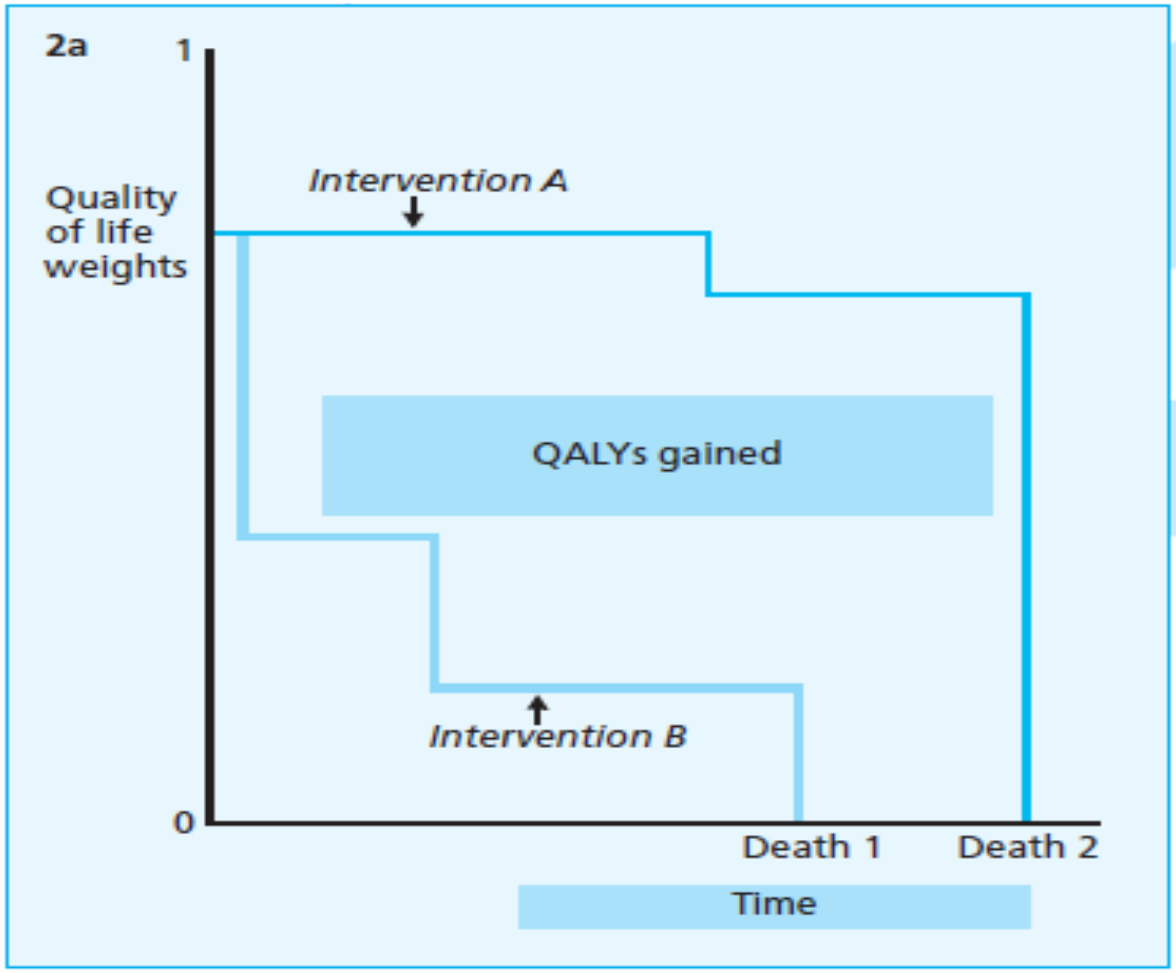
Calculating cost/QALY

- ICER – New Rx vs. standard care

  (hypothetical all other costs are equal)

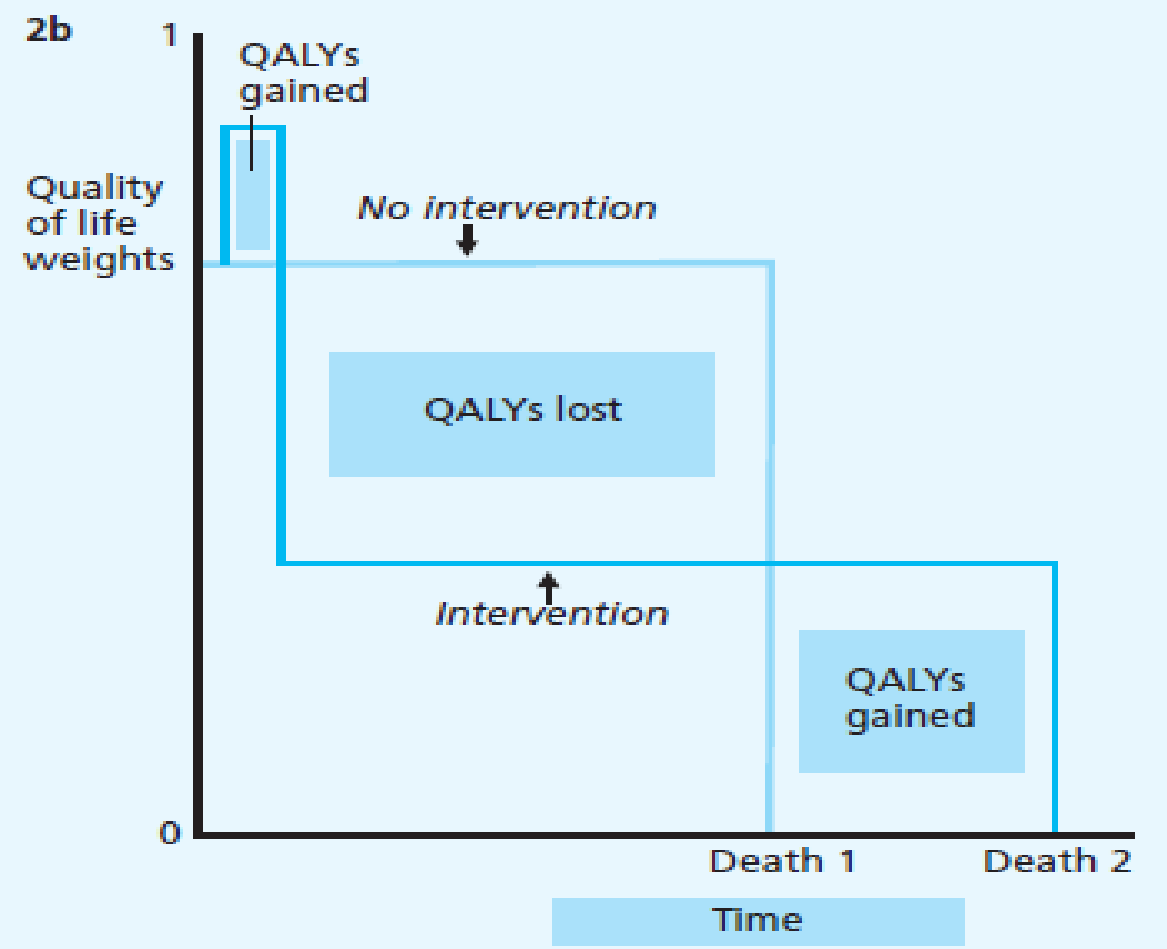
$$\frac{(\$10{,}000 - 0)}{(.675 - .5375)} = \frac{\$10{,}000}{.1375} = \$72{,}727 / QALY$$

QALY Example #1



Source: Phillips, 2009

QALY Example # 2



Source: Phillips, 2009

QALY Example #3

	1 year	1 year	1 year	1 year	Total QALYs
A	.50	.50	.75	.75	?
B	.50	.50	.50	.50	?

Poll

- What are the additional QALYs generated by Treatment A?

a) 1 QALY
b) 2 QALYs
c) 0.5 QALYs
d) 0.25 QALYs

QALY Example #3

	1 year	1 year	1 year	1 year	Total QALYs
A	.50 (.50*1) .50	.50 (.50*1) .50	.75 (.75*1) .75	.75 (.75*1) .75	.50+.50+.75+.75 = 2.5
B	.50 (.50*1) .50	.50 (.50*1) .50	.50 (.50*1) .50	.50 (.50*1) .50	.50+.50+.50+.50= 2.0

Deriving Preferences or Utilities

- Basic methodology:
  - Individuals provide a personal reflection on the relative value (preference weight) of different health states experienced or described.

Deriving preferences or utilities

- Three methods to derive preferences:
  - Direct
  - Indirect
  - Off-the-shelf

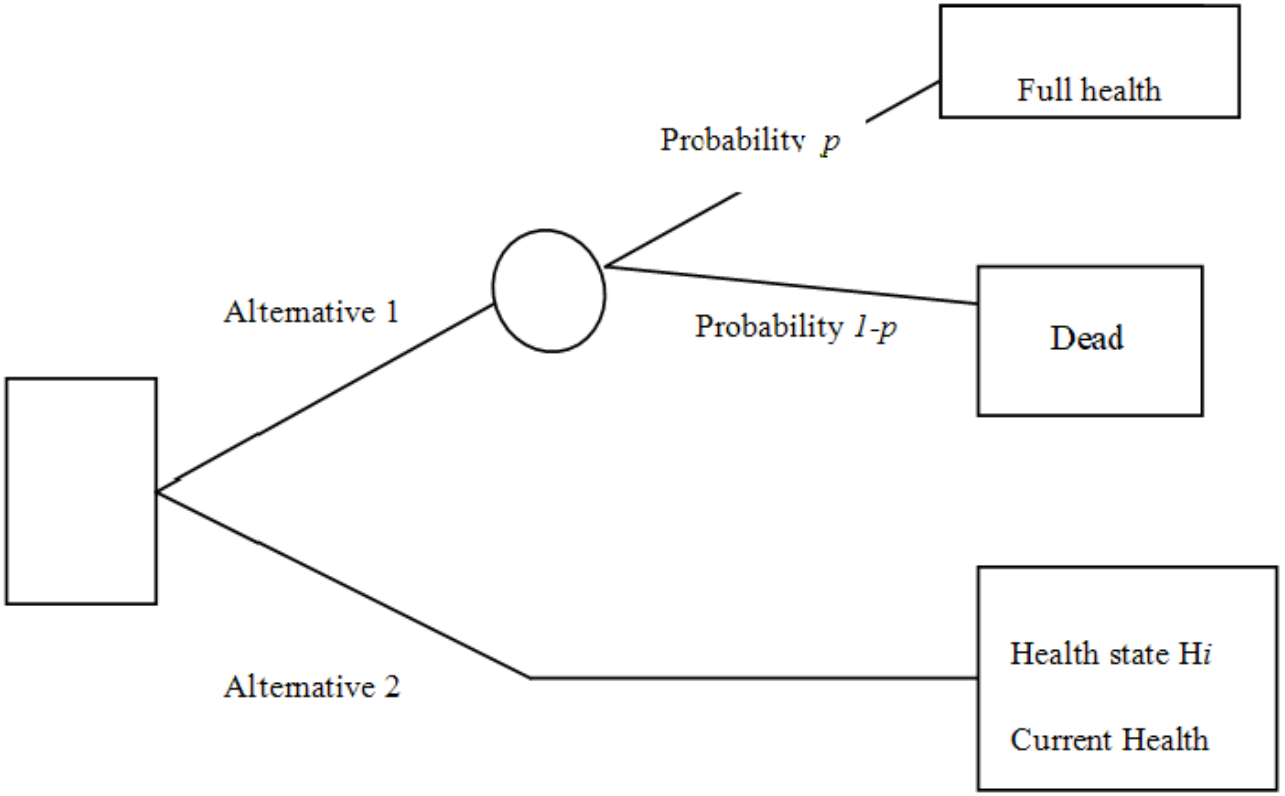
Direct Methods

- Individuals asked to choose (declare *preferences*) between their current health state and alternative health status scenarios

Direct: Valuation Method

- Standard Gamble
- Time trade-off
- Rating scale (visual analogue scale)

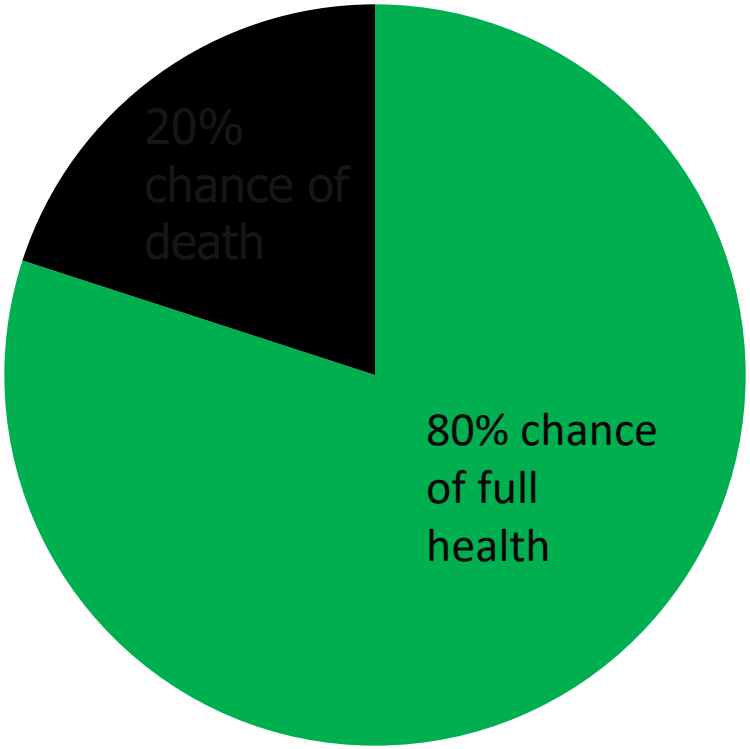
Direct: Standard Gamble



Source: Sinnott et al., 2007

Direct: Standard Gamble

- Rest of life in current health state; or
- "take a pill (with risks) to be restored to perfect health"
- Scale represents risk of death respondent is willing to bear in order to be restored to full health.



Standard Gamble Scenario

- You are able to see, hear and speak normally
- You require the help of another person and a cane to walk or get around.
- You are occasionally angry, irritable, anxious and depressed.
- You are able to learn and remember normally.
- You are able to eat, bathe, dress and use the toilet normally.
- You are free of pain and discomfort.

Standard Gamble Scenario

- Treatment A: allows you to live 10 years in this health state

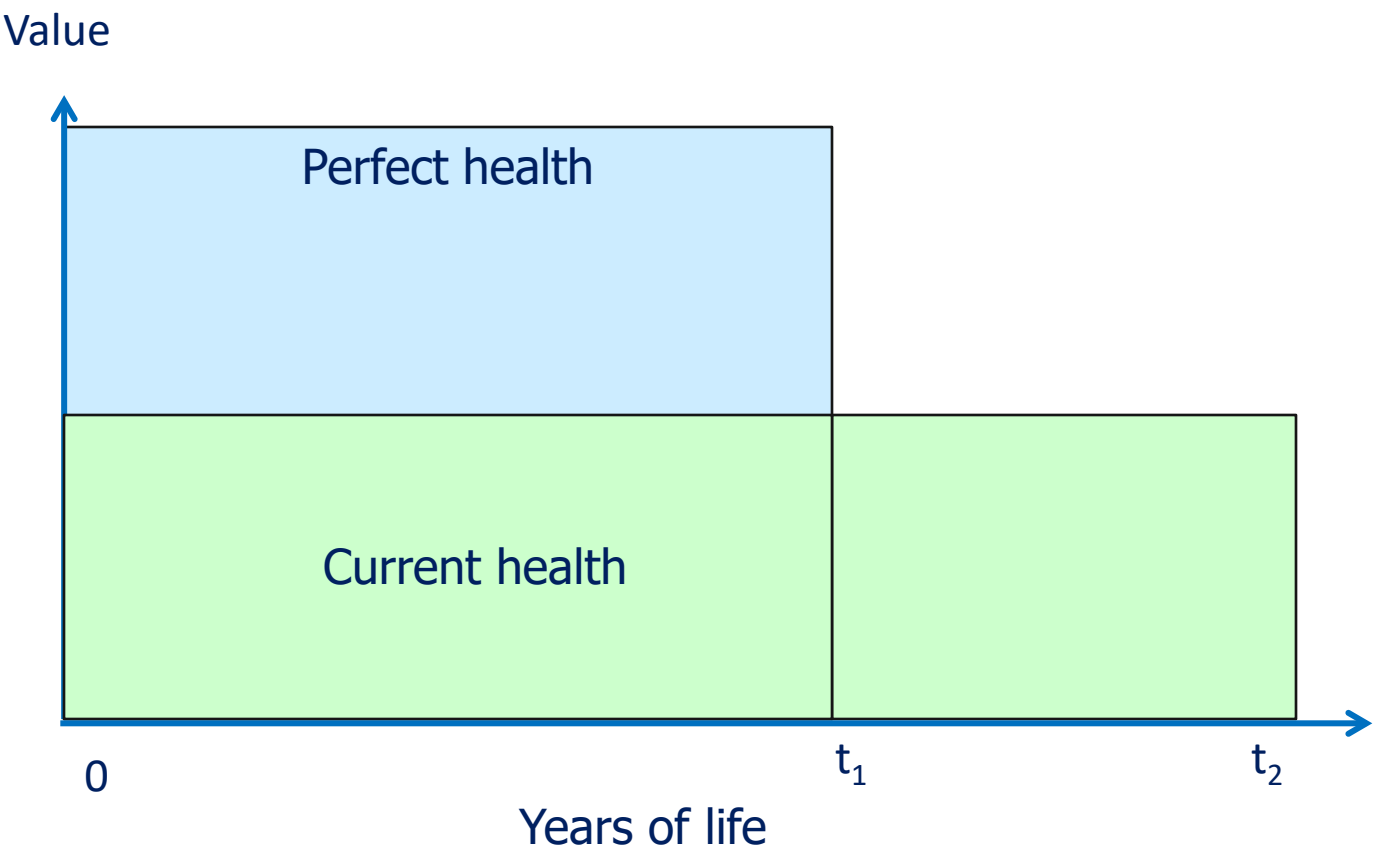
- Treatment B: Gives a p% chance of returning to full health and (100-p%) chance of death
  - Successful=10 years of full health
  - Unsuccessful = immediate death

Standard Gamble Scenario

- Your doctor tells you that the chance the second treatment will succeed is not known

- Please indicate the minimum chance of success (i.e. p%) that you would require to accept the second treatment

Direct: Time Trade-off

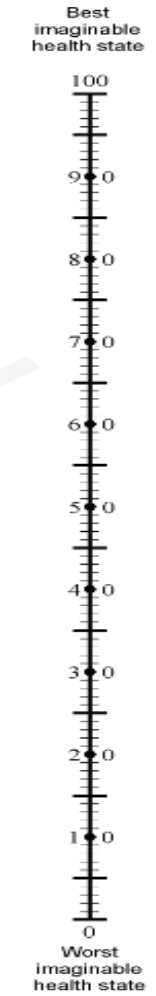


Considering the health state described

- How many years of life in your current state would you be willing to give up to live out your life in perfect health?
  - 5 years
  - 10 year
  - No years

Direct: Rating Scale (VAS)

- Place health state on line
- Anchors:
  - Best possible health state
  - Worst possible health state
- Generates values, not utilities



Poll

- With which valuation method would a respondent's utility be affected by their willingness to take on risk?

a) Standard gamble
b) Time trade-off
c) Visual analogue scale

Direct Methods

- SG measures preferences under conditions of uncertainty
- TTO choices are made under conditions of certainty
- VAS involves neither choice nor uncertainty

Direct Methods

- May be necessary if effects of intervention are complex:
  - Multiple domains
  - Effects not captured in indirect or disease-specific instruments

Direct: Whose preferences?

- Patient
  - Experience disease and treatment
  - Recruitment challenges
  - Higher valuations of health states
- General public/“community preference”
  - Society’s resources

Indirect Methods

- Study subjects complete surveys
- Multiple domains of health
- Composite describes the health status
- Composite state is linked to community results (or “weights”)

How are you today? (EQ-5D)

- Which statements best describe you today?
  - Mobility:
    - No (1), slight (2), moderate (3), severe (4), or extreme problems (5)
  - Self-care
  - Usual Activities
  - Pain/Discomfort
  - Anxiety/Depression
- Health profile ranging from 11111 to 55555

Indirect Measures

- EuroQol (EQ-5D)
- Health Utility Index (HUI)
- 15D
- Quality of Well-Being Scale (QWB)
- SF-6D

Indirect Measures

- Vary with respect to:
  - Dimensions or attributes included;
  - Population used to establish the weights;
  - Health states defined by the survey; and
  - Method of valuation

Indirect measures

- ✓ Standard surveys that are widely used
- ✓ Describe generic health states
- — May lack sensitivity in specific contexts
  (Payakachat, Ali & Tilford, 2015)

EuroQol EQ-5D

- 5 questions in 5 domains of health
  - Mobility, self-care, usual activity, pain/discomfort, or anxiety/depression
  - EQ-5D-5L has 5 levels (“no,” “slight,” “moderate,” “severe,” and “extreme”/”unable to”
  - 3,125 health states (5^5)
- Basis of domain weights:
  - Past studies based on British community sample
  - US weights now available (Pickard et al., 2019)

Health Utility Index (HUI)

- 41 questions
- 8 domains of health and 972,000 health states
  - vision, hearing, speech, ambulation, dexterity, emotion, cognition, and pain
- Basis of domain weights:
  - Canadian community sample rated hypothetical health states
  - Utility theory

SF-6D*

- Converts SF-36 or SF-12 scores to utilities
- 6 health domains
  - physical functioning, role limitations, social functioning, pain, mental health, and vitality
  - Defines 18,000 health states
- Basis of domain weights
  - British community sample originally
  - US community sample (Craig et al., 2013)

15D

- 15 health domains:
  - Mobility, vision, hearing, breathing, sleeping, eating, speech, excretion, usual activities, mental function, discomfort and symptoms, depression, distress, vitality, sexual activity
  - 5 levels each
- Basis of domain weights:
  - Finnish community sample (Sintonen, 1995)
- For more details:
  - http://www.15d-instrument.net/15d/

Indirect: Disease-specific surveys

- Key methods issues:
  - Difficult to describe health state to community respondent
  - Difficult to establish values when there are a large number of possible health states
- Expensive, but sensitive to variations in quality of life for specific diseases
- Often used in addition to generic measure
- Can sometimes be mapped to generic measures

Off-the-shelf values

- Use preference weight determined in another study for health state of interest
  - Not all health states have been characterized
- Useful in decision modeling

Which method to use?

- Trade-off between sensitivity and burden

- Start with a literature search re:
  - The condition of interest
  - In the population of interest
  - For the outcomes of interest

Ease of Use

- Off-the-shelf utility values
- Indirect Measures (HUI, EQ-5D, QWB, SF-6D, 15D)
- Disease-specific survey during trial and transform later to *preferences*
- Direct measures (SG, TTO)

Issues surrounding QALYs

- Lack of sensitivity
- Inadequate weight attached to emotional/mental health problems
- Lack of consideration for non-health outcomes
- A QALY is a QALY is a QALY?

Published Example

Jodar-Sanchez et al. (2015). Cost-Utility Analysis of a Medication Review with Follow-Up Service for Older Adults with Polypharmacy in Community Pharmacies in Spain: The conSIGUE Program. *Pharmacoeconomics* 33(6), 599-610

- Collect EQ-5D data at baseline and follow up
- Generate EQ-5D index scores
- Calculate QALY gains for intervention and control groups

Useful Resources

- Tufts Center for Evaluation of Value and Risk in Health

  https://www.tuftsmedicalcenter.org/Research-Clinical-Trials/Institutes-Centers-Labs/Center-for-Evaluation-of-Value-and-Risk-in-Health.aspx

- Tufts Cost Effectiveness Analysis Registry

  http://healtheconomics.tuftsmedicalcenter.org/cear4/Home.aspx

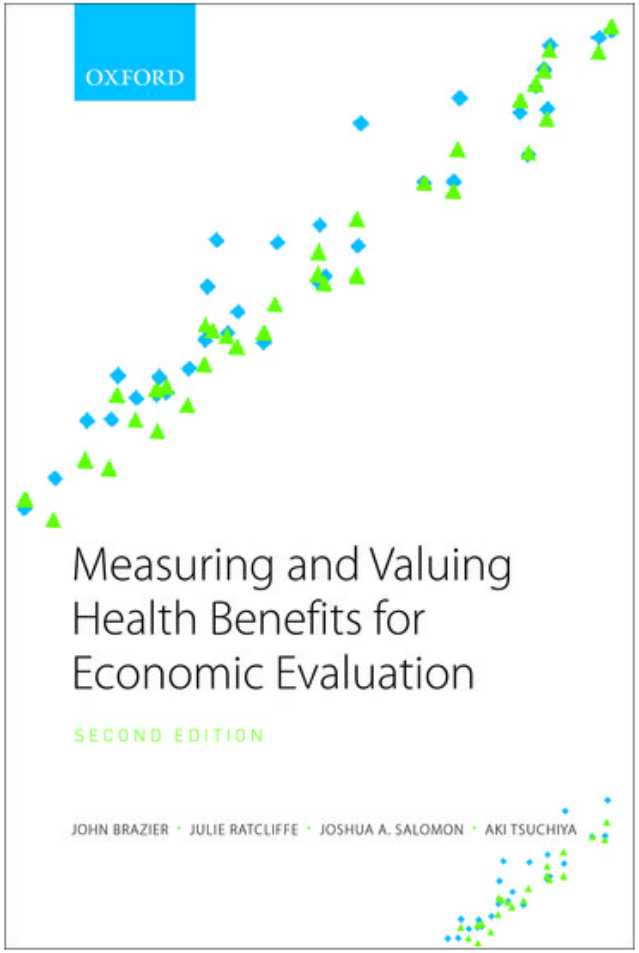
- ISPOR

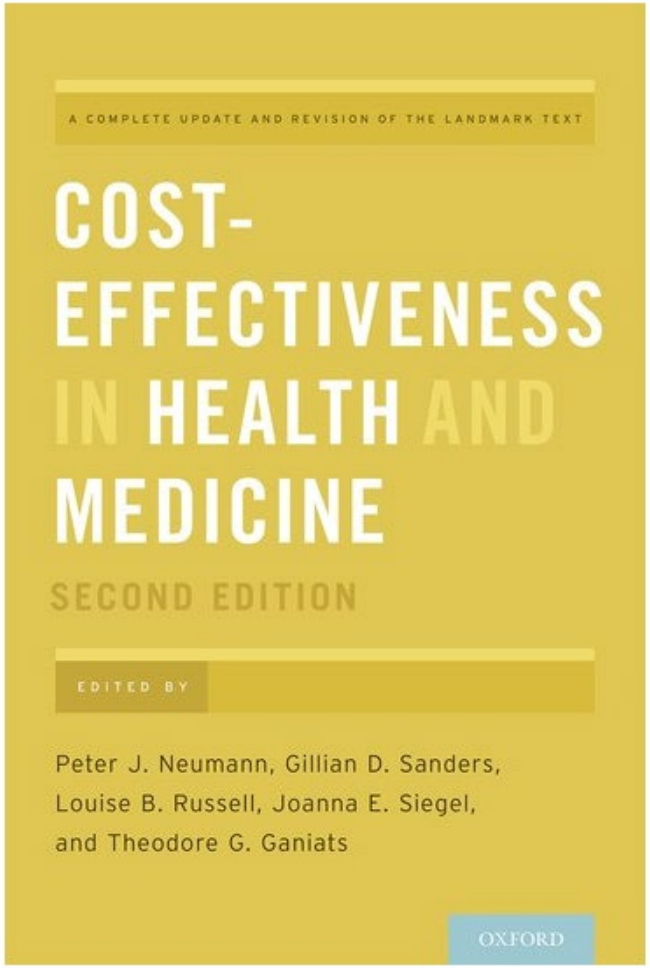
  https://www.ispor.org/heor-resources/good-practices-for-outcomes-research

- National Institute for Health and Care Excellence, UK

  https://www.nice.org.uk/

Useful Resources





Useful Resources

- Institute for Clinical and Economic Review (ICER)
  https://icer.org/our-approach/methods-process/cost-effectiveness-the-qaly-and-the-evlyg/

- Preference Measurement in Economic Analysis. Guidebook. VA Health Economics Resource Center.
  http://www.herc.research.va.gov/publications/guidebooks.asp

Condition-Specific Measure Resources

- Person-Centered Assessment Resource
  http://www.healthmeasures.net/resource-center/measurement-science/intro-to-person-centered-assessment

- Brazier J, Deverill M, Green C. (1999). A Review of the use of health status measures in economic evaluation. *J Health Serv Res Policy*, 3(9):174-184. https://www.ncbi.nlm.nih.gov/pubmed/10538884

- Brazier J et al. (2012). Developing and testing methods for deriving preference-based measures of health from condition-specific measures (and other patient-based measures of outcome). *Health Technol Assess*, 16(32):1-11. https://www.ncbi.nlm.nih.gov/pubmed/22832015

- Brazier Jet al. (2014). A systematic review, psychometric analysis and qualitative assessment of generic preference-based measures of health in mental health populations and the estimation of mapping functions from widely used specific measures. Southampton (UK): NIHR Journals Library; (Health Technology Assessment, No. 18.34.) Chapter 4, Mapping mental health condition-specific measures to generic preference-based measures. https://www.ncbi.nlm.nih.gov/books/NBK262023/

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Upcoming HERC Seminars

CEA Alongside a Clinical Trial
- Todd Wagner
- 04/06/2020

Budget Impact Analysis
- Todd Wagner
- 04/20/2020

Understanding Cost Variables in DoD DaVINCI Databases
- Libby Dismuke-Grier
- HERC Health Economics Seminar Series
- 04/13/2020

Questions or Comments?

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