# Introduction to Effectiveness, Patient Preferences, and Utilities

Josephine Jacobs, PhD

HERC Cost Effectiveness Analysis Course

Feb 14, 2018

# Overview

- Outcomes measurement in CEA

- Concept of QALYs for a CEA

- Estimating QALYs

- Guidelines on selecting measures

# 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

    ✓  Most 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

- Death = 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
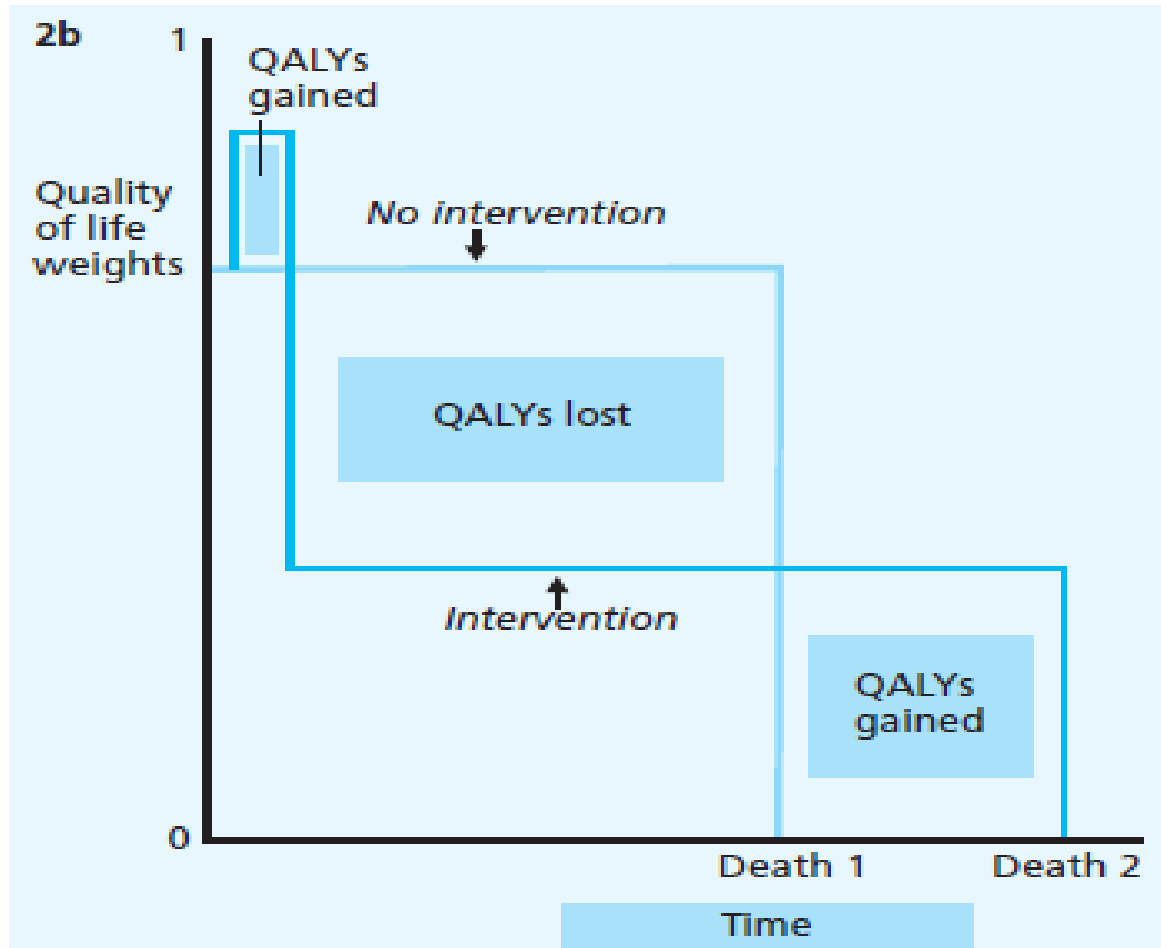  - *(hypothetical all other costs are equal)*

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

# QALY Example #2a
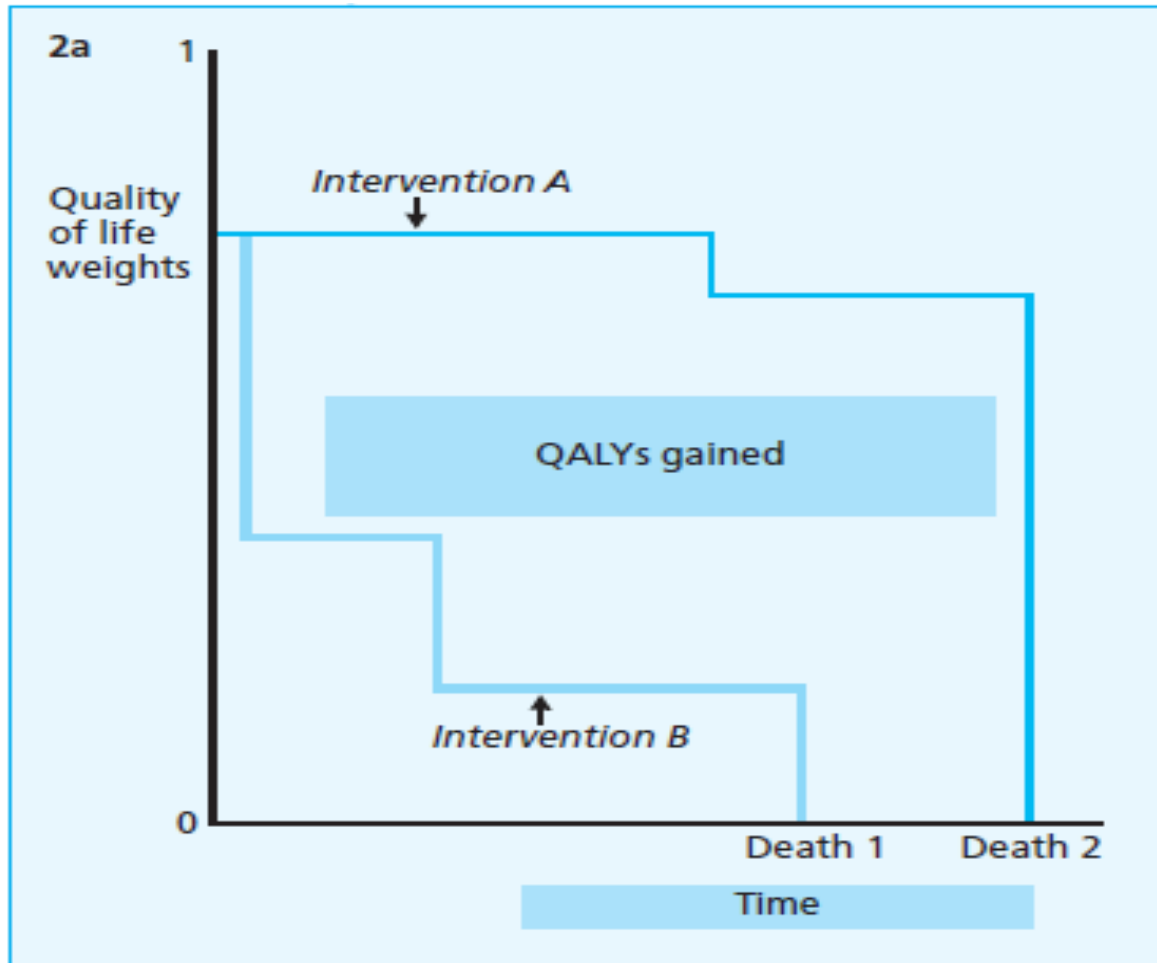


Source: Phillips, 2009.

# QALY Example # 2b



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<br>(.50*1)<br>.50 | .50<br>(.50*1)<br>.50 | .75<br>(.75*1)<br>.75 | .75<br>(.75*1)<br>.75 | .50+.50+.75+.75 = 2.5 |
| **B** | .50<br>(.50*1)<br>.50 | .50<br>(.50*1)<br>.50 | .50<br>(.50*1)<br>.50 | .50<br>(.50*1)<br>.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

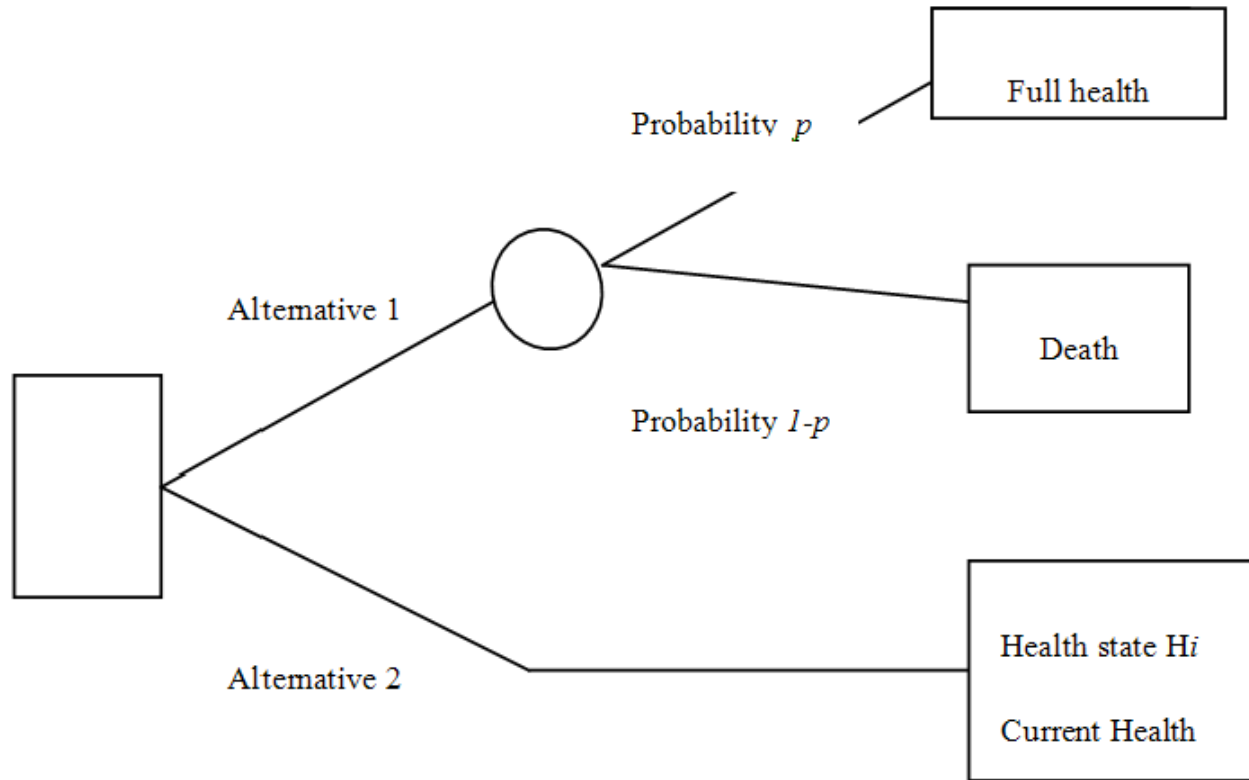  – Indirect

  – 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

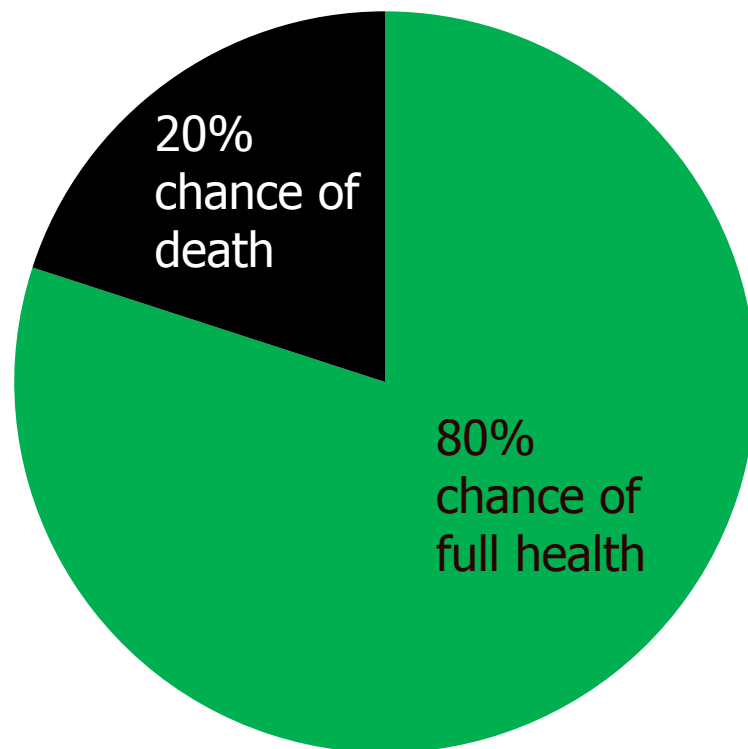- Time trade-off

- 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.



20% chance of death

80% chance of 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
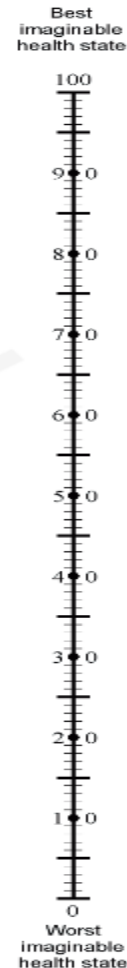
# Direct: Time Trade-off
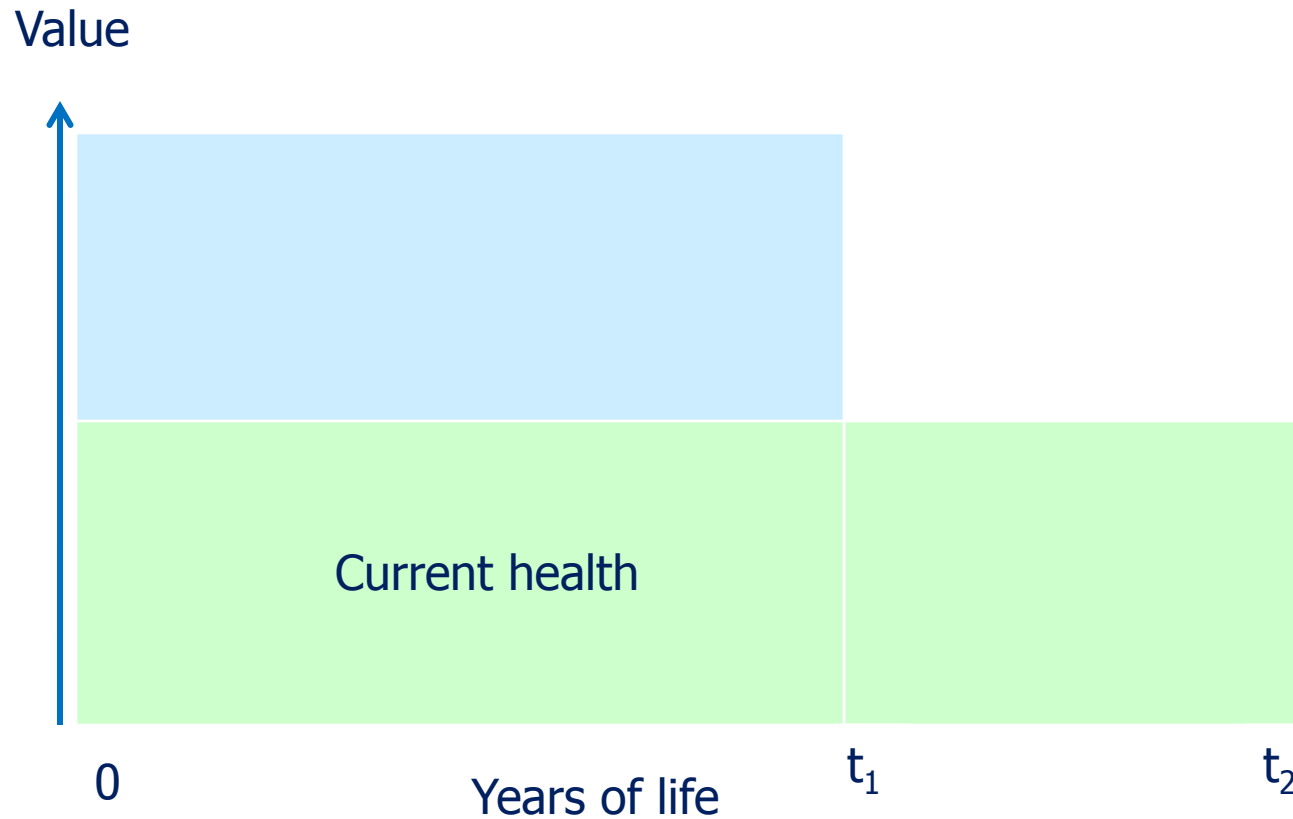
# Considering the health state described

■ 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



Best imaginable health state

100

90

80

70

60

50

40

30

20

10

0

Worst imaginable health state

# 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 problems, some problems, extreme problems
  - Self-care
  - Usual Activities
  - Pain/Discomfort
  - Anxiety/Depression

# Indirect Measures

- Health Utility Index (HUI)

- EuroQol (EQ-5D)

- 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

# EuroQol EQ-5D

- 5 questions in 5 domains of health
  - Mobility, self-care, usual activity, pain/discomfort, or anxiety/depression
  - 245 health states.

- Basis of domain weights:
  - Past studies based on British community sample
  - New US weights recently published

# 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

# 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 potentially sensitive to variations in quality of life for this disease
- Often used in addition to generic measure

# 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)

- 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?

# 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

# Important 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

- National Institute for Health Research, UK

http://www.nets.nihr.ac.uk/

# Important Resources

- Brazier J, Deverill M,  Green C, Harper R, Booth A. A Review of the use of health status measures in economic evaluation. Health Technol. Assess 1999;3(9).

  http://www.nets.nihr.ac.uk/projects/hta/934708

- Brazier et al. Developing and testing methods for deriving preference-based measures of health from condition-specific measures (and other patient-based measures of outcome).
  http://www.nets.nihr.ac.uk/projects/hta/069704

# Important Resources

- Tufts Cost-Effectiveness Analysis Registry
  http://healtheconomics.tuftsmedicalcenter.org/cear4/Home.aspx

- PROMIS
  http://www.healthmeasures.net/explore-measurement-systems/promis

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

# Upcoming HERC Seminars

Estimating Transition Probabilities for a Model

- Risha Gidwani-Marszowski
- 02/28/2018

Medical Decision Making and Decision Analysis

- Jeremy Goldhaber-Fiebert
- 03/07/2018

# QUESTIONS and COMMENTS

# References

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

Phillips, C. (2009). *What is a QALY?* What is…? Series. Hayward Medical Communications.  Available at  www.whatisseries.co.uk.

Sinnott, P.L., Joyce, V.R., & Barnett, P.G. (2007). Preference Measurement in Economic Analysis. Guidebook. Menlo Park CA. VA Palo Alto, Health Economics Resource Center.