Cost-Utility Analysis (CUA), Part I

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Cost-Effectiveness Analysis HSMP 6609 2020

Outline

- Utility theory in economics
- Utility under uncertainty
- Measuring preferences
- Scales, standard gamble, time trade-off
- Examples
- The EQ-5D
- \blacksquare The EQ-5D in the US
- Calculating QALYs

Department of Very Big Ideas in Brief

- Suppose that an intervention increases life expectancy by 10 years. The quality of the extra years is not that great (say, a person is confined to bed)
- We want to adjust the extra years to account for the poor quality of life
- We could come up with a number between 0 and 1 that reflects how people feel about life in that condition. The lower the quality the lower the number
- For me, that number is about 0.15. I'd probably be beyond grumpy/bored if confined to bed. And don't like to depend on others for care
- So my **quality-adjusted life year** (QALY) would be $10 \times 0.15 = 1.5$ years. So the intervention increased quality-adjusted life years by 1.5 years not 10
- That's all. Seriously, that's it. But there is a lot of theory, controversy, and methods to calculate the 0 to 1 number

Micro 101: Consumer theory

- People (aka "consumers") make choices about what to buy, time spent studying, partying, working, and so on
- Economists start thinking about this problem by assuming that people have preferences over activities and goods. These preferences are described by a utility function
- The utility function is the "pleasure, satisfaction, or need fulfillment that people get from their economic activity" (Nicholson, 2000)
- Wikipidea says "In economics, utility is a measure of preferences over some set of goods and services" (too narrow, it's also preferences for pretty much everything)
- It turns out that we need to make very few assumptions about preferences for this framework to work remarkably well

Micro 101: Consumer theory

- Consumers also face a budget constraint. All of us (even the very wealthy) cannot purchase all we want or do all we want to do
- We don't have enough income and the day happens to have 24 hours (plus we sleep about 30% of that time)
- So what should a rational consumer do? She should maximize her utility subject to the budget constraint
- In my experience, the first time students encounter this framework they either fall in love with it and torture their friends talking like an economist or they think that economists are insane
- Both have a good point... Just remember that it's a simplification of reality to analyze a problem. Not a description of reality. And the baby version is not all of economics. Don't take it too seriously
- And no, Freakonomics, the books and the podcast, is not a representative sample of economics

Micro 101: Consumer theory

- Assume that we only have two goods, X and Y and that there is a function that translates the quantity of both goods into utility: U(X, Y)
- We can write the budget constraint as $p_X X + p_y Y = I$
- p_x and p_y are the prices of goods X and Y and I is income
- If you solve the maximization problem, you get some familiar things with different names: in equilibrium marginal utility with respect to a good is equal to its price or up to the point where the marginal rate of substitution of one good over the other is equal to the ratio of their prices
- In other words, marginal cost (price) = marginal benefit (utility)
- This is how you can derive a demand curve. A demand curve tells us how much a consumer wants to buy of a good at different prices

Micro 101: The other side: Producer Theory

- For today's class and for the (little) theory behind cost effectiveness we don't need to cover producer theory, but that's the other side
- Producers are assumed to maximize profits (revenues costs) subject to a budget constraint. They need labor and capital to produce goods and services
- Solving the maximization problem, you find that producers will produce quantities up to marginal cost (cost of production) = marginal benefit (revenue)
- So the cost of producing another unit is equal to money they get selling it
- From this we can derive **supply curves**
- From the interaction of supply and demand curves we get prices and quantities sold in the market. So we have a market

Properties of utility functions

- Preferences need to follow some basic properties
 - 1 Complete: Goods can be compared
 - **2 Reflexive**: X is at least as preferred as X (trivial but needed)
 - **3 Transitive**: If we prefer X over Y and Y over Z, then we prefer X over Z
 - **4 Continuity**: Preferences can be represented by a utility function that is continuous (technical)
- As my old micro textbook says: "A utility function is a very convenient way to to **describe** preferences, but it should not be given any psychological interpretation. The only relevant feature of a utility function is its **ordinal** character" (Varian, 1992)
- A utility function tells us that consumers prefer one thing over the other; we don't care why or if that's happiness, practicality, vanity, or a combination of all that
- All consumers are assumed to have is preferences that rank things, the measurement of utility does not matter

Applications

- It's pretty amazing how much economists have done with the utility framework
- Gary Becker used it to analyze marriage, racism, crime, addiction (got a Nobel for that although not all those theories are that great -my two cents)
- It's part of any field in economics (sometimes not in macro, but that's another story)
- Most economists would tell you that utility cannot be measured
- However, economists do think that we can deduct preferences from actual choices consumers make under different circumstances (revealed preferences)
- But there is another type of utility function

Utility under uncertainty (von Neumann-Morgenstern utility)

- vNM utility framework incorporates uncertainty
- Consumers now choosing goods ("payoffs") that are not certain
- For example, a consumer faces a possible loss of L with some probability P_L . He needs to decide if he buys insurance that will pay I if the loss happens. If it not happens, he will lose the insurance premium C_p with probability $(1 P_L)$
- vNM showed that a rational consumer should maximize expected utility
- **Expected utility** is the payoff from certain events multiplied by the probability that those events happen
- For this problem to have a solution, the properties of utility functions need to be modified. In particular, the utility function now has a cardinal interpretation (so this is the utility that in theory can be measured)

von Neumann-Morgenstern utility

- That's where the concepts of risk seeking, risk averse, and risk neutral come from
- Example: Somebody tells you that you can have \$100 for sure now or \$200 if a coin toss is heads and \$0 if tails. What do you prefer?
- A **risk-neutral** person is indifferent between the options because the expected value of the gamble is also \$100 (200 × 0.5 + 0 × 0.5)
- A risk-averse person would prefer the \$100 for certain
- A risk-seeking person would prefer the gamble
- Of course, people are not always risk seeking or neutral, depends on the gamble, the payoff and many other factors
- (I'm risk-averse when it comes to gambling but I keep running in spite of several broken bones in the process)

Department of Students Pondering Big Thoughts

- What does this have to do with economic evaluations?
- We will use the concept of utility under uncertainty to measure preferences over (uncertain and hypothetical) health states to come up with that number between 0 and 1
- Health state: The health status of a give person or population at one point in time (ill, alive, well, dead, confined to bed, etc)
- We will use a questionnaire to define health states more precisely and some (sort of funny) questions to elicit preferences
- Methods to elicit preferences
 - 1 Scales
 - 2 Standard gamble
 - 3 Time trade-off

The very simplistic way: scales

To help people say how good or bad a health state is, we have drawn a scale (rather like a thermometer) on which the best state you can imagine is marked 100 and the worst state you can imagine is marked 0.

We would like you to indicate on this scale how good or bad your own health is today, in your opinion. Please do this by drawing a line from the box below to whichever point on the scale indicates how good or bad your health state is today.

> Your own health state today

Best imaginable health state 100 90 80 80 -70 -60 + 50 Ŧ 40 ŧ 3 200 Worst imaginable health state The very simplistic way: scales

- Your textbook has a description of several procedures according to whether the states are chronic or temporary
- The problem is that rating scales are more ordinal than cardinal.
 Good for figuring out if one health state is preferred to other but not how much more
- A difference, say, from 0.2 to 0.1 is not the same as 0.8 to 0.7
- In studies comparing scales to other methods, scales don't do well
 - 1 Responders tend to not use the end of the scales
 - 2 Responders tend to space responses
- Scales do not follow logically from utility under uncertainty
- On the other hand, scales are easy to administer and it's possible to map from scales to numbers from other methods

- The standard gamble approach does follow directly from vNM utility theory
- Example: You are confined to bed and are offered two alternatives
 - **1** Alternative 1: You receive a treatment that cures you and you live healthy for 10 years with probability P, or you die immediately with probability (1 P)
 - 2 Alternative 2: You receive no treatment and live for 10 years in current state
- The interviewer will change the probability until you are indifferent between the alternatives
- For example, if P=1, you would (hopefully) choose alternative 1. If P=0, you may choose Alternative 2, unless you can't bear the thought of being confined to bed and prefer to die immediately.
 What value of P makes you indifferent?
- The worse the health state, the lower the P. The P chosen is the preference over the health state



- You probably can see the problem with this approach
- The problem is, of course, that for most of us thinking with probabilities is difficult
- You could train responders and give them aids but not that helpful
- It's really hard to think in term of probability (see Brase, Cosmides, and Tooby, 1998):
 - http://www.cep.ucsb.edu/papers/individnatsamp98.pdf
- Probability wheels: "These wheels have two areas of different colors representing the probabilities for the outcomes of the gamble. The areas of the wheel are iteratively adjusted until the subject is indifferent to the outcome" (Garza, 2008)



Figure 2. Standard gamble decision card.

Time trade-off

- Developed to avoid the difficulties of asking people to think in terms of probabilities
- Example: You are confined to bed (again, sorry) and are offered two alternatives
 - **1** Alternative 1: You live in the current state for t = 10 years
 - **2** Alternative **2**: You get a treatment that will allow you to be healthy but you will live healthy *x*, where *x* is < *t*
- What value of x would leave you indifferent between the alternatives?
- In other words, to be healthy, you need to trade off years of life
- The preference is given by ^x/_t, which is between 0 and 1. For bad health states, ^x/_t will be low
- This is the most common method for **eliciting preferences**

Eliciting preferences

- How do we ask get people to value health states in practice?
- To elicit preferences we need a way to define health states –like being in bed, not walking, etc
- The EQ-5D has become the most popular method
- Developed by the EuroQol Group in Europe
- 5 domains of health: mobility, self-care, usual activities, pain/discomfort, anxiety/depression

EQ-5D

Figure 1: EQ-5D (UK English version)

By placing a tick in one box in each group below, please indicate which statements best describe your own health state today.

Mobility

I have no problems in walking about I have some problems in walking about I am confined to bed	1 2 3
Self-Care I have no problems with self-care I have some problems washing or dressing myself I am unable to wash or dress myself	
Usual Activities (e.g. work, study, housework, family or leisure activities) I have no problems with performing my usual activities I have some problems with performing my usual activities I am unable to perform my usual activities	
Pain/Discomfort I have no pain or discomfort I have moderate pain or discomfort I have extreme pain or discomfort	
Anxiety/Depression I am not anxious or depressed I am moderately anxious or depressed I am extremely anxious or depressed	

Defining states

- A person that is in **perfect health** will mark the first box for in all questions, so her health state is: 11111
- In the example that I have been using today, a person confined to bed but otherwise well, would be: 31111. A bit unrealistic because that person is probably not very happy, so his state would be: 31112
- We are not assigning values, we are just **defining** more precisely the health states
- The EQ-5D defines a total of $3^5 = 243$ states

Eliciting preferences for a population

- In the US, one large study was conducted around 2002 (Shaw et al, 2005)
- Used a **representative** sample of the US adult civilian noninstitutionalized population (N=3,650)
- "109 field interviewers, including 30 bilingual interviewers. Each face-to-face interview consisted of 3 stages. All stages used a single set of 45 health states, with each health state described on a separate card. Only 15 health states/cards were used with each respondent. Interviews were administered in English or Spanish. The interview used a paper-and-pencil format, and respondents were paid \$30 for their participation" (Shaw et al, 2005)
- So just in compensation to participants: $30 \times 3650 = 109,500$

Eliciting preferences for a population

- Not all states were evaluated (too time consuming)
- They used regression models to extrapolate to other health states. As a result, negative values are possible, so scale is from -1 to 1. Scores of less than 0 are interpreted as "worse than death"
- The result of the study are **weights** that transform answers to the EQ-5D into a **preference score** ("tariffs")
- In other words, the magic number between 0 and 1, although adjusted to be -1 to 1

EQ-5D in the US

- You can get the weights to score preference scores from Shaw et al (2005) in the MEPS website: http://archive.ahrq.gov/professionals/ clinicians-providers/resources/rice/EQ5Dscore.html
- For example, a health state of 33333 is valued at -0.1091. In other words, a representative sample of the US thinks that living in that state is a lot worse than death
- What about the example I have been using? Health state 31111 (confined to bed but otherwise healthy): 0.442
- So a representative sample, on average, gave up 4.4 years to avoid being confined to bed
- What about 31112 (confined to bed and somewhat depressed or anxious)?: 0.426

EQ-5D scores (see spreadsheet)

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мо	SC	UA	PD	AD	m1	s1	u1	p1	a1	m2	s2	u2	p2	a2	d1	i2	i22	i3	i32	pre	full health	EQ-5D index(US_D1)
3	3	3	3	3	0	0	0	0 0	0	1	1	1	1	1	4	0	0	4	16	-0	-0.109	-0.1090707
3	3	2	3	3	0	0	1 1	1 0	0	1	1	0	1	1	4	0	0	3	9	-0	-0.100	-0.0996728
3	3	1	3	3	0	0	() (0	1	1	0	1	1	3	0	0	3	9	-0	-0.100	-0.0995382
-	-	-	-	-	-	-								-		-	-	-	-	-		

How is this used in practice?

- The typical case:
 - 1 Ask participants to complete the EQ-5D
 - 2 Score the EQ-5D using the Shaw et al (2005) weights for a study in the US
 - 3 Adjust years of life gained using the preference index to get QALYs
- Several problems in practice: in some interventions most people will be healthy (for example, screening for diabetes or celiac in children)
- So why bother with QALYs at all?: comparability and the need to go from intermediate to final outcomes
- In the US, the EQ-5D is often not used; instead, other generic or disease specific scales (like the SF-12 or FACT-G) are common (there are methods to predict one from the other)

More on the EQ-5D

- There are two versions now: EQ-5D-3L and EQ-5D-5L
- The EQ-5D-5L has 5 answers to each of the 5 domains, so 3125 health states
- Not widely used yet because somebody needs to elicit preferences for those 3125 health states...
- Lots studies validating the EQ-5D. Weights available for many countries
- See User Guide: http: //www.euroqol.org/fileadmin/user_upload/Documenten/PDF/ Folders_Flyers/EQ-5D-3L_UserGuide_2015.pdf

The big picture (wake up!!)

- We conduct an intervention and calculate the years of life gained for the participants
- We use a survey instrument to define the health states of the participants
- But then we use the preferences of a representative sample of the US to put a value on the quality of those health states
- Put it differently, we are using a societal perspective to value (hypothetical!) health states of *others*
- The Panel on CE recommended using the valuation of a representative sample
- Some studies value the preferences of the participants. Results are different. People adapt

Loose ends: double counting

- Question: when you were answering the question about preferences over the confined-to-bed question, did you consider your inability to work in your valuation? Was it lower because of it? I was not. I was thinking about running and skiing
- This is the double counting problem. Some authors argue that people should be instructed to not take into account productivity loses when valuating health states because productivity loses, from a societal perspective, are also part of the costs (the numerator in ICER)
- The new CEA recommendation is that people should be told not to take into account productivity loses

Calculating QALYs

Remember that in CUA ICER becomes:

$$ICER = rac{C_1 - C2}{QALY_1 - QALY_2} = rac{\Delta C}{\Delta QALY}$$

- In other words, ICER is the cost per unit of QALY
- All we covered before is still valid here; we just calculated QALYs
- We now have the 0 to 1 number (or -1 to 1) and need to adjust years of life gained by their quality

Calculating QALYs

- Using the weights is pretty easy; exactly the same as in the first slide (I told you that was all)
- A person lives 5 years in EQ-5D health state 31111. Weight for this state is 0.442. So QALY is 5 × 0.442 = 2.21
- With aggregated data, you need to somehow come up with an average weight reflecting the different times spent in different health states
- It's easier to see how to do this once we cover Markov models

Next class

- More on other instruments used to measure health states and preferences
- A common problem in the US is that interventions do not use instruments that can be easily converted to preferences
- We will (briefly) talk about prediction methods to go from health status instruments to preferences
- We will talk about the idea of thresholds to decide if an intervention is cost-effective. We had to wait because thresholds are calculated in terms of \$\frac{\$}{QALYs}\$
- Then the last class before the break will be about cost-benefit, cost of illness, and budget impact