Economic evaluations of healthcare interventions: Defining value, affordability, perspective, and patient-reported outcomes

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Outline

- Economic evaluations: definition and goals
- Key elements: perspective, time horizon, relevance of costs
- Key metric: Incremental cost effectiveness ratio (ICER)
- Defining "value"
- Value vs affordability vs sustainability
- Patient outcomes: measuring quality
- Examples

Economic evaluations

- Definition: "The comparative analysis of alternative courses of action in terms of both their costs and consequences" (Drummond et al, 2015)
- **Alternative courses of action**: Health interventions like screening programs, new ways of delivering care, new medications, or new imaging technology. Also, intensity (dosage, screening frequency)
- **Comparative analysis**: Always a *comparison* – current way of doings things versus new ways; old drugs versus new drugs; new imaging technology versus old imaging technology. A valid alternative could be "doing nothing." A very common one: "usual care"
- **Costs**: Measured in monetary units ($)
- **Consequences**: *Outcomes* measured in natural units (cases detected/averted, years of life gained, disability days), years of life gained combined with a measure of quality (e.g. QALY), or even $

Types of economic evaluations

- Different combinations of the elements described in the previous slide produce different types of economic evaluations – in part, just a taxonomy
- **Cost savings (cost averted)**: Consider only costs of alternatives, not outcomes or consequences. Does doing A saves resources compared to doing B?
- **Cost-effectiveness analysis (CEA)**: Consider costs and outcomes, but outcomes measured in "natural" units: cases detected, years of life gained
- **Cost-utility analysis (CUA)**: Same as CEA but outcome is life years adjusted for quality. The catch is that "quality" is measured as preferences over health states, which is how economists define "utility"
- Most people now use CEA to refer to either CEA and CUA
- **Cost-benefit analysis (CBA)**: Both cost and consequences/outcomes measured in $. Not done in practice for healthcare interventions since it requires translating outcomes into monetary units. What is the monetary value of 1 cases averted? A life? Ethical concerns

Purpose

- This is the most important consideration to understand the rest of today's presentation: **Why do we do economic evaluations?** (In particular, CEA/CUA)
- The goal of an economic evaluation is to **inform decision makers** about the efficient allocation of scarce resources
- We don't have an infinite budget. We need to allocate **scarce resources** in the most efficient way
- To do so, we need to compare costs and consequences
- Other goals result in different types of **economic or financial** studies – we will see today Budget Impact Analysis as an example

Key elements

- If the goal is to inform decision makers about the most efficient allocation of resources, the **analytical perspective** is key
- **Perspective**: from which analytical perspective are we conducing the study? Payer (Medicare/Medicaid, insurance company, government), provider (hospital), society (considers all costs regardless of who pays for it)
- Note that economic evaluations are not conduced from the analytical perspective of the patient, although the societal perspective does include patient costs
- Consequences/outcomes are ALWAYS measured from the point of the view of the patient: consequences are health outcomes
- **Time horizon**: For how long do we measure costs and consequences? 1 year? 5 years? Lifetime?
- **Relevance of cost for the decision**: Always a comparison so only costs that are difference between alternatives are important

CEA outcome measure: ICER

- Results presented as (incremental) costs per unit of outcome, the *Incremental Cost Effectiveness Ratio* (ICER)

$$ICER = \frac{C_1 - C_2}{E_1 - E_2}$$

  C_i and E_i are the costs and effectiveness measure of alternative i
- The incremental part is important. We don't compare averages (i.e. $\frac{C_1}{E_1}$ vs $\frac{C_2}{E_2}$) because it doesn't match the decision
- The decision is whether we should do 1 or 2. This is the very foundation of economic theory and maximization subject to constraints: everything is at the "margin"
- Note that ICER is expressed as $\frac{\$}{outcome\ \ unit}$

Examples of studies and objectives

- Did a smoking cessation program for Medicaid beneficiaries save money? (Cost saving, 5-year, Medicaid perspective)
- **Digression**: Although popular, cost saving studies are partial evaluations. Saving money is not a goal of health care. It's just the numerator of ICER. But if we can argue that outcomes are same or better, then we can show value too
- Is a new medication that cures hepatitis C cost effective (comparing cost and and consequences using QALYs)? Alternatively, is the new medication worth the extra cost? (CEA, lifetime, societal perspective)
- Is a new surgical procedure for back pain worth it? Do the extra costs compared to the additional benefits provide good value?
- What is the most cost effective way of screening for prostate cancer (how often)? (CEA, lifetime, societal perspective)

Using ICER to make decisions

- Suppose that we have an ICER comparing any of the interventions above
- Let's call the new technology B, which we compare to to usual care, A. Now what?
- Easy decisions:
  1. B is more expensive and less effective (prefer A) [most likely, no economic evaluation done]
  2. B is less expensive and more effective (prefer B)
- Not-so-easy decisions:
  1. B is both less expensive and less effective [not a common situation; we probably wouldn't consider B since it's less effective]
  2. B is more expensive and also more effective $\leftarrow$ **this is the typical scenario we will review today**

Value in CEA

- If B is more expensive and also more effective, how do we establish value? Is B worth it? Let's say that we found that ICER is \$400K per QALY or \$20K per case detected (screening program). *What do we do with these numbers?*
- Not much by themselves. **Value is a relative concept**. We would need to compare to other alternatives
- If we use QALYs, there are commonly accepted thresholds (rule of thumb). In the US, anything below $\$200K$ or $\$150K$ per QALY would be considered cost effective (i.e. good value)
- The threshold has a long history and controversy. It's both the opportunity cost and the **willingness to pay for an extra year of life** (adjusted for quality)
- If we used other units of outcomes (e.g. cases detected), we can only compare the \$20K per case detected to alternatives that used the same unit of outcome
- **This is a very narrow definition of value**: a comparison of incremental costs and incremental benefits/outcomes. That is. No consideration of equity, future advances, age of patients, etc

Comparability, comparability, comparability

- If the notion of value is relative and it's essentially a comparison, then we should be using the same measure of outcome to calculate ICERs so we can make comparisons
- Life years is a top contender. We all care about not dying prematurely, and is a convenient way of **comparing many different technologies** (e.g. a cancer drug to a cardiac procedure to a smoking cessation program)
- But modern medicine is very good at extending life years even if those extra years are spent in very poor quality
- Therefore, it make sense that we want to adjust life years for the "quality" of those years
- This is why QALYs remain the most used and recommended measure of outcome in economic evaluations
- Is QALY the best and only measure? **Of course not**. Patients care about other outcomes, but we need the same unit to make comparisons

Efficient allocation of resources

- How do we connect CEA and ICERs with the efficient allocation of resources?
- Suppose that we have a finite healthcare budget. We could maximize life years gained (adjusted for quality) if we do the following:
  - Sort interventions by ICER (from lowest to highest)
  - Spend on the program/technology with lowest ICER first
  - If there is money left, spend on the program/technology with the second lowest ICER
  - Repeated until the budget has been allocated
- That would be the most efficient way of allocating scarce resources that would **maximize life years** (adjusted for quality)

Example: Sovaldi

- The case of Sovaldi is worth considering because it highlights the difference between value, affordability, and sustainability
- Sovaldi (sofosbuvir) entered the market in 2013. It's a very effective medication, essentially a cure for hepatitis C
- When it launched, the 12-week course of treatment would cost US $84,000 per patient
- The CEA question would be: is the extra cost of Sovaldi (compared to standard treatment) worth the extra effectiveness?
- Some studies found that Sovaldi is cost effective (compare to threshold) for certain groups with more advanced disease. Other studies found that it was not cost effective compared to best alternative treatments for hepatitis C (Zhang et al, 2015)
- **None of these studies showed that a payer or health system could afford to cover Sovaldi**. Many states were sued because some states (through Medicaid) didn't cover Sovaldi

Budget Impact Analysis

- CEA tell us if an intervention/technology has relative value, but not if a health system or payer can **afford** to cover it
- It's not a statement about **sustainability** either from the point of view of a provider (e.g. hospital), even if the study was from the point of view of a provider
- Budget Impact Analysis is a type of financial analysis with the goal of ascertaining the impact of an intervention/technology on finances
- Usually from the perspective of a payer (not society), short-term (3 to 5 years)
- Key ingredient: how many people will use the intervention? What is the uptake?
- A Medicaid BIA study would show affordability issues regarding Sovaldi (at the $84K cost)

Patient perspective

- The only analytical perspective that takes into account the patient perspective in the ICER numerator is the societal perspective
- Transportation costs, family time, time not working, etc. These can be significant in many cases and should be taking into account (e.g. laparoscopic surgery)
- Consequences/outcomes are always about the **patient**
- Life years gained is straightforward. Say that intervention A, on average, results in 10 years of life compared to alternative B
- To adjust those 10 years for quality, we need to find the average quality of those extra years, a number between 0 and 1
- If not great, say, 0.3, then only $10 \times 0.3 = 3$ QALYs, not 10 life years. If very good, say, 0.95, then 9.5 QALYs

Quality in CEA

- This is a vast area of research, but how quality is defined in economic evaluations is somewhat peculiar
- Quality is conceptualized as "**preferences**" over **health states**
- A representative sample of the population are asked to state their preferences over health states
- It follows economic theory: utility under uncertainty

Defining health states

- The EQ-5D is the most common instrument used to define health states

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	❑	1
I have some problems in walking about	❑	2
I am confined to bed	❑	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	❑

Quality in CEA

- In the US, a large study (Shaw et al, 2005) asked a representative sample to value the health states defined by the EQ-5D
- These are expensive, large studies. Before that study CEA in the US would use UK preferences
- Example: quality for state 31111 (confined to bed but in good health otherwise) is 0.44
- Moderate pain but not other problem (11131) is 0.93 – with severe pain, 0.57
- A study would ask participants to complete the ED-5D, but it would use the preferences of a representative sample who valued **hypothetical health states** (so-called community preferences)
- The reason is that, in theory, society should decide on the allocation of resources

Is QALY a good outcome measure?

- Yes and no. Obviously life years are important; we all care about it
- We could consider other patient-centered outcomes, but we need to compare to other studies using same outcome
- Consider the case of surgery for back pain. We could still measure the outcome in terms of QALYs: life years adjusted for quality
- We wouldn't expect that life years will be affected by the new surgery, so the ICER would be driven by quality
- If on average back surgery reduces pain from severe to moderate, that's a gain of 0.36
- The EQ-5D is crude instrument

Summary

- The purpose of economic evaluations is the efficient allocation of scarce resources given budget constraints
- Value in economic evaluations is a comparison of incremental costs and incremental benefits
- Comparability makes QALYs the most used outcome, but it doesn't mean it's the most appropriate or patient-centered outcome
- Important to understand CUA's definition of value, and the difference between affordability or sustainability

References

Drummond, M.F., Sculpher, M.J., Claxton, K., Stoddart, G.L. and Torrance, G.W., 2015. Methods for the economic evaluation of health care programmes. Oxford university press.

Shaw, J.W., Johnson, J.A. and Coons, S.J., 2005. US valuation of the EQ-5D health states: development and testing of the D1 valuation model. Medical care, pp.203-220.

Zhang, S., Bastian, N.D. and Griffin, P.M., 2015. Cost-effectiveness of sofosbuvir-based treatments for chronic hepatitis C in the US. BMC gastroenterology, 15(1), p.98.