### Introduction to Economic Evaluations

Marcelo Coca Perraillon

University of Colorado Anschutz Medical Campus

Cost-Effectiveness Analysis HSMP 6609 2020

## Outline

- Logistics
- An overview of this class
- What is an economic evaluation?
- Examples of economic evaluations
- Types of economic evaluations

Cost analysis Cost-effectiveness analysis Cost-utility analysis Cost-benefit analysis

- Overview of key concepts and elements of economic evaluations
- Methods: ICER, decision trees/Markov models

### Logistics

- The textbook is NOT required this year. I'll make copies of important chapters
- However, I strongly recommend you get the book. An older edition would be fine
- The awesome thing about the textbook is that it covers pretty much every important topic and nuance in EE. The problem is that it covers every single topic and nuance
- The textbook can be a royal pain in the [insert anatomical part] but the alternatives are not great
- The class notes are your guide to the key parts in each chapter but you should read the assigned chapters if something is not clear
- Don't wait until the night before a homework is due. I'll be sleeping

### Logistics

- Groups: Groups are not mandatory but try to form one. It will help you
- We will have homework most weeks. First homework due next week. Choose articles wisely
- Office hours: Please email me or Ben. Stop by. I'll always make time

## What is an economic evaluation?

Textbook: "The comparative analysis of alternative courses of action in terms of both their costs and consequences"

- 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, years of life gained combined with a measure of quality (e.g. QALY), or even \$. Confusion alert: benefits, consequences, outcomes, effectiveness. They all mean more or less the same –until they don't...

#### Purpose

- The ultimate goal of an economic evaluation is to inform decision makers about the efficient allocation of resources
- Resources are scarce; we need a (good) way to decide how to allocate them
- Economic evaluations provide a way to systematically think about cost and consequences of alternatives
- We will talk more about how economic evaluations are used (or not used) in the US and in other countries later in the class
- Even if not used for actual allocation of resources, they are an attempt to assess if an intervention or technology provides VALUE

- Is medical spending worth it? Does it have good value?
  - I'll talk about the notion of value many (many!) times during the class
  - Value in economic evaluations has a very precise meaning: it's a comparison of incremental costs versus incremental benefits (consequences, outcomes)
  - Not so different from the way we think about value in our daily life: Do I want to pay \$3 extra for craft beer (compared to, say, Coors or one of the other watery ones)? Is the extra/additional benefit (taste, satisfaction, snobbery) I get greater than \$3?
  - If you were paying attention during your health economics class, this is the usual utility framework: people will consume until marginal utility equals marginal costs (technically,  $MRS_{xy} = \frac{P_x}{P_y}$ )
  - Note that this is a narrow way of making choices. And value doesn't mean that we can afford something!
  - You'll read Cutler (2018) for your first homework... Now, examples

#### The Return on Investment of a Medicaid Tobacco Cessation Program in Massachusetts

#### Patrick Richard<sup>1<sup>#</sup></sup>, Kristina West<sup>1</sup>, Leighton Ku<sup>2</sup>\*

1 Department of Health Policy, School of Public Health and Health Services, The George Washington University, Washington, District of Columbia, United States of America, 2 Center for Health Policy Research, School of Public Health and Health Services, The George Washington University, Washington, District of Columbia, United States of America

#### Abstract

Background and Objective: A high proportion of low-income people insured by the Medicaid program smoke. Earlier research concerning a comprehensive tobacco cessation program implemented by the state of Massachusetts indicated that it was successful in reducing smoking prevalence and those who received tobacco cessation benefits had lower rates of in-patient admissions for cardiovascular conditions, including acute myocardial infarction, coronary atherosclerosis and nonspecific chest pain. This study estimates the costs of the tobacco cessation benefit and the short-term Medicaid savings attributable to the aversion of inpatient hospitalization for cardiovascular conditions.

Methods: A cost-benefit analysis approach was used to estimate the program's return on investment. Administrative data were used to compute annual cost per participant. Data from the 2002–2008 Medical Expenditure Panel Survey and from the Behavioral Risk Factor Surveillance Surveys were used to estimate the costs of hospital inpatient admissions by Medicaid smokers. These were combined with earlier estimates of the rate of reduction in cardiovascular hospital admissions attributable to the tobacco cessation program to calculate the return on investment.

## Richard et al. (2012)

- Did a smoking cessation program for Medicaid beneficiaries in MA save money?
- Note that they say it's a cost-benefit analysis. You will learn in this class that they in fact did a cost analysis
- Calculated the cost of the intervention (\$183 per participant). Calculated that because some people stopped smoking they had lower short term medical costs: \$571 in savings → For every \$1 of program costs Medicaid saved \$3.12 (<sup>571</sup>/<sub>183</sub>)
- The study has received attention but has some problems: measure of program impact a bit suspicious, short-term analysis
- Long-term cost studies of smoking cessation programs are somewhat sad but highlights the difference between cost studies and cost-effectiveness/benefit. Saving money is not a public health goal

#### British Medical Journal (BMJ)

#### Training care givers of stroke patients: economic evaluation

Anita Patel, Martin Knapp, Andrew Evans, Inigo Perez, Lalit Kalra

#### Abstract

Background Training care givers reduces their burden and improves psychosocial outcomes in care givers and patients at one year. However, the cost effectiveness of this approach has not been investigated. 

tion in basic skills of moving and handling, facilitation of activities of daily living, and simple nursing tasks; care givers received training over three to five sessions, lasting for 30-45 minutes each, and a follow up session at home.

Assessment of care givers' quality of life

## Patel et al. (2004)

- Evaluate the cost effectiveness of (informal) caregiver training by examining health and social care costs, informal care costs, and quality adjusted life years in **caregivers** (only 1 year follow up)
- A bit unusual in the sense that the study was concerned about the quality of life of the caregiver, not the patient
- About 300 caregivers were randomized into two groups: training and control group
- They compared costs and benefits
- Conclusion: Caregiver training during rehabilitation of patients reduced costs of care while improving overall quality of life in caregivers



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#### ARTICLE

### Cost-effectiveness of Prostate Cancer Screening: A Simulation Study Based on ERSPC Data

E. A. M. Heijnsdijk, T. M. de Carvalho, A. Auvinen, M. Zappa, V. Nelen, M. Kwiatkowski, A. Villers, A. Páez, S. M. Moss, T. L. J. Tammela, F. Recker, L. Denis, S.V. Carlsson, E. M. Wever, C. H. Bangma, F. H. Schröder, M. J. Roobol, J. Hugosson, H. J. de Koning

## Heijnsdijk et al. (2015)

- Objective: Assess the cost effectiveness of prostate cancer screening (PSA)
- Used data from European Randomized Study of Screening for Prostrate Cancer (ERSPC)
- Simulation of clinical story of 10,000 men who are screened starting at age 55
- Predicted the numbers of 1) prostate cancers diagnosed, 2)prostate cancer deaths averted, 3) life-years and 4) quality-adjusted life-years (QALY) gained, and cost-effectiveness of 68 screening strategies for prostate cancer
- Conclusion: Prostate cancer screening can be cost-effective when it is limited to two or three screens between ages 55 to 59 years.
  Screening above age 63 years is less cost-effective because of loss of QALYs due to overdiagnosis."

## My examples

Colorado Family Planning Initiative

Provided free long-acting contraceptives (LARCs) (IUDs, implants) at Title X clinics

Supported by an anonymous private foundation

Research question: what are the cost savings from the perspective of the state of Colorado?

Blood screening for celiac disease and diabetes

Large scale screening in children ages 2-17. UC-BDC plans to screen in Denver (and offer follow-up)

Opportunity to measure (some) costs and benefits

The intervention will last a couple of years but the effects have to be taken into account over a lifetime

As in the prostrate screening example, we need to use simulations

DH tobacco cessation program

System-wide changes related to smoking cessation programs Did the intervention work? Why? Did it save money?

Background

## Key elements of economic evaluations

- **1** Analytical perspective or point of view of the analysis: Who is incurring the costs and consequences?
  - Provider, health department, societal, insurance company (private or Medicare/Medicaid)
  - "Consequences" or outcomes, typically patients
- **2 Time horizon** (analysis time, follow-up time): short term, long term? Whole lifespan?
- **3 Discounting**: Time preference. A benefit/cost now is not the same as the same benefit/cost 30 years later (opportunity cost, not really inflation)
- 4 Sensitivity analysis: Evaluates the effects of uncertainty in data
- 5 Methods: Simple aggregation of costs and benefits, decision trees, Markov models

### Prerequisites of EEs

- This may sound obvious, but you want to make sure the alternative you want to compare works and does no harm
- A story about economic evaluation proposals or why most EEs never happen
- If you think about an economic evaluation idea, consider if there is any evidence of effectiveness
- Example: Does contact with nature improve health?

### Challenges

- The main challenge of economic evaluation is lack of DATA
- Data synthesis (e.g. meta-analysis) is many times a key component of EEs
- Rarely a single sources of data; EEs combine multiple sources of data
- (I'll provide examples of data sources for the US)
- As a consequence, statistical inference is essentially non-existent in economic evaluations
- Sensitivity analyses is the way uncertainty is incorporated in economic evaluations

## Types of economic evaluations

- How benefits/outcomes/consequences are measured (or not) distinguishes different types of economic evaluations
  - 1 Cost-analysis (CA)
  - 2 Cost-effectiveness analysis (CEA)
  - 3 Cost-utility analysis (CUA)
  - 4 Cost-benefit analysis (CBA)

## Cost analysis

- Objective: compare the cost of two or more alternatives. Consequences are not examined (partial evaluation)
- $\blacksquare$  But... if benefits are the same  $\rightarrow$  it's like comparing costs and benefits
- Cost savings studies are a common type of cost studies. The underlying assumption is that the health consequences are the same or better with the new alternative but the new alternative has the potential to reduce costs as well
- This seems to be the modern public health holy grail because of shrinking budgets. I find it problematic: improving health costs money
- Another type: cost of illness study (COI): COI measures the economic burden of a disease and estimates the maximum amount that could potentially be saved if a disease were to be eradicated (usually biased)
- We will spend about three weeks on costs and cost analysis because it's common to all types of economic evaluations

#### More on costs

What costs should be considered depends on the **perspective** of the analysis and the time horizon

**Health sector**: drugs, equipment, hospital stays of the program and possibly follow-up

Other sectors: Social services, follow-up personnel, home health

**Patient and/or family**: Travel time, caregiver's lost income, cost of informal care...

**Productivity changes**: Interventions may affect ability to work now but could increase the ability to work in the future

 The general guideline is to include ALL costs (societal perspective); in practice, it's very difficult

## Cost-effectiveness Analysis (CEA)

- Objective: compare the cost and benefits/consequences of two or more alternatives
- Benefits are measured in "natural units"
- Effectiveness measure should be relevant/important
- Example of benefits: cases averted, births averted, cases detected, years of life gained, blood pressure, days free of pain...
- Can compare several alternatives with same benefit measure

Cost-effectiveness Analysis (CEA)

- Results presented as (incremental) costs per unit of outcome
- 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

- We will spend some time discussing and calculating ICERs
- Limitations: a) There is no reference "value" associated with the measure of benefit. b) Can only compare alternatives in which the "natural unit" of benefit is the same.

## Cost-utility Analysis (CUA)

- Same objective: compare the cost and consequences of two or more alternatives
- Benefits are measured in quality-adjusted life years (QALYs)
- QALYs combine both gains in extra years of life (quantity) with the quality of those gains
- Can compare more alternatives because most health interventions have the goal of extending life (and hopefully with good quality)
- Life years gained is a fairly relevant outcome

Cost-utility Analysis (CUA)

- We will talk a lot about how economists measure "quality" (preferences)
- As before, ICER provides a summary of results but now  $E_i = QALY_i$

$$ICER = \frac{C_1 - C_2}{QALY_1 - QALY_2}$$

- Advantage: there are reference thresholds to show value
- Limitation: not all measures of benefits are easily measured in QALYs
- Example: How do you measure life years gained in interventions that promote breastfeeding? Few choices other than simulations stacking one assumption on top of another...

## Using ICER

- We have ICER comparing alternative B to usual care, A. Now what?
- Easy cases:
  - 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 cases:
  - **1** B is more expensive and more effective (how do we know if the extra benefit is worth the extra cost?) [this is the typical scenario we will study this semester]
  - 2 B is both less expensive and less effective (how do we know if the cost savings are worth the reduced effectiveness?) [not a common situation]

#### The cost-effectiveness plane



Back to the harder cases

- A) Is the extra benefit worth the extra costs or B) Are the costs savings worth the reduced benefits?
- There is an implicit notion of value. Think about how you make decisions in your life.
  - Is it worth it to get an MPH?
  - Last year's ski boots (cheaper) than this year's model (more expensive)?
- Key is what we are giving up and how we value it
- The notion of **opportunity cost**: the loss of potential gain from other alternatives when one alternative is chosen
- The notion of a threshold value. The (in)famous \$50,000 per QALY.
- We will come back (several times) to these issues during the semester

## Cost-benefit Analysis (CBA)

- Once again, same objective: compare the cost and consequences of two or more alternatives
- But now benefits are measured in monetary units
- CBA is the type of economic evaluation that is more consistent with economic theory
- CBA can tell us if an intervention is worth doing
- The problem of using CBA in health is that it is very difficult to transform measures of benefits into monetary values

## Cost-benefit Analysis (CBA)

- What is the dollar value of a case averted? What is the value of an extra year of life? What is the value of not being able to walk?
- This doesn't mean that economists don't try... We will study ways to assign monetary values to (some) benefits
- CBA is extensively used in certain project evaluations (infrastructure) but not so often in health
- If benefits > costs, then it is worth it to do the project
- Summary measure: *benefit*/cost ratio or (*benefit* cost)

## A note on terminology (jargon)

- "Economic evaluation" is the more general term. It may refer to one of the four types of studies we discussed (CA, CEA, CUA, and CB)
- "Cost-effectiveness analysis" is also used as a general term but it refers to CEA and CUA. Blame your textbook (up to third edition) for insisting on separating CEA and CUA
- There is always confusion with names this class is called Cost Benefit and Effectiveness in Health...
- More important than definitions is understanding the principles and making the right decisions
- Remember, EE is about making decision on how to allocate resources

### Methods

- We have talked about how to calculate ICER adding up costs and calculating benefits (e.g. cases detected, QALYs)
- But there are other ways of conducting economic evaluations: decision trees/Markov models
- Think of them as alternative ways of calculating ICER, but ways that allow us to incorporate uncertainty more directly and extend the time horizon
- It's going to be clear by mid-semester, don't worry

#### Methods

### Decision analysis

- Definition: A systematic approach to decision making under uncertainty (Raiffa 1968)
- It originated as a way of figuring out how we should make decisions when there is uncertainty (that is somehow quantifiable)
- The (initially) confusing part is that we will use decision analysis methods to calculate the ICER
- Your textbook does not cover much on decision analysis; we will complement it with other readings
- We will use Excel; TreeAge is the most popular software but it's not the best tool for learning (black box). Excel is actually more flexible (but requires more programming)

#### Decision Tree



#### Decision Tree



#### Markov Models

- Useful when events are recurrent (like migraines)
- Essentially, a recursive decision tree with cycles/periods
- Mutually exclusive "states" representing possible consequences
- Simulate a cohort going from one state to the other in each cycle with certain probability
- Each state may have a cost and/or benefit associated with it, which transforms Markov models into an economic evaluation

Methods

## Example: Burlone et al. (2013)

# Evaluating the effects of extending contraceptive coverage under the ACA $$\rm in\ OR$$



Importance

## Why this class should be useful

- Although CEA is not often considered as a basis for making decisions in the US, the field is rapidly advancing
- Several studies have influenced practice (e.g. screening for cancer)
- In the last decade, several organizations have adopted economic evaluation methods as part of their framework for "value" of new medications and procedures (see Neumann and Sanders, 2017)
- The cost of new, some of them impressive, treatments are getting crazy expensive. We can't just ignore the issue of costs. See Johnson (2018) article on the Washington Post (under additional readings) about gene therapy for blindness (\$850K!!)
- There are going to be more jobs for people who know the economic evaluations (\$); costing methods are applicable in many situations
- Finally, there is of course the joy of learning...

Importance

## Monthly cost of new cancer medications

If trend continues, in 2020 the monthly cost would be \$19,220 per month or \$230,417 per year (!!)



