

Decision Analysis: an Overview

Liam Rose, PhD

January 2020

Acknowledgement: Risha Gidwani-Marzowski, DrPH

Today

- Why to use decision analysis
- Overview of different types of decision analysis
 - CEA, CBA, CCA, BIA
 - Introduce concepts used in upcoming HERC lectures that dive deeper into specific areas

Don't forget to submit your questions
throughout!

Why engage in decision analysis?

- Trying to solve a problem
- Have a number of interventions to choose from
- No clear answer on which is best
 - With one clearly superior option, decision analysis may be unneeded or trivial
- Inefficiencies and limited resources
 - Cannot simply try every intervention
 - Example: considering which car to buy, should not keep buying cars to make sure you have the best one!

Why engage in decision analysis?

- Weigh the pros and cons of each intervention to make an informed decision
 - Logical
 - Transparent
 - Quantitative
- Not all “pros” and “cons” are equal:
 - Consequences of pro/con
 - Probability of pro/con
 - Variation in probability

Pros and cons

- Option A:
 - 80% probability of cure
 - 2% probability of serious adverse event
 - Option B:
 - 90% probability of cure
 - 5% probability of serious adverse event
 - Option C:
 - 98% probability of cure
 - 1% probability of treatment-related death
 - 1% probability of minor adverse event
-

Opportunity costs

- Choosing one option means forgoing another
 - Due to funding/resources
 - Due to mutual exclusivity
- Examples:
 - Should a Hepatitis screening be expanded? (limited resources)
 - Operative or nonoperative management for condition X? (mutually exclusive)

Recap, Why to use Decision Analysis

- Allocation of limited resources
- Each intervention has pros and cons
- Each intervention is different:
 - Condition/population
 - Cost
 - Health outcome

Advantages of Decision Analysis

- Evaluates each intervention using the same measure(s)
- Compare results using the same metric:
 - Costs
 - Cost per Life Year Saved
 - Cost per Quality-Adjusted Life Year (QALY)

Decision Analysis can be applied to...

- Drugs
- Procedures
- Health programs
- Screening
- Vaccines
- Reimbursement decisions
- Etc.

Types of decision analysis

Types of decision analysis

- Cost-effectiveness analysis (CEA)
- Cost-benefit analysis (CBA)
- Cost-consequence analysis (CCA)
- Budget impact analysis (BIA)

Poll Question #1

- What type of decision analysis have you conducted?
- Answers: Cost-effectiveness, cost-benefit, cost-consequence, budget impact, none

Decision Analyses are comparative

- CEA, CBA, CCA, and BIA evaluate one option *in relation to another*
- That other option can be:
 - standard of care
 - “do nothing”
 - another active intervention

Cost-Effectiveness Analysis (CEA)

Ratio of Costs to Health effects

- Health effects can be anything
 - Life-years, cancer cases, number of infections, etc.
- Costs
 - What implementation would cost over a time frame

CEA and ICERs

- Cost-Effectiveness Analyses compare the impact of 2 or more interventions
- Result is an Incremental Cost-Effectiveness Ratio (ICER):

$$ICER = \frac{Cost_B - Cost_A}{Health\ Effect_B - Health\ Effect_A}$$

Cost-Utility Analysis

- A particular form of cost-effectiveness analysis
- Health Effect is a Quality-Adjusted Life Year (QALY)

QALY is derived from Utility

CEA versus CUA

Both compare 2 or more interventions

Method	Cost-Effectiveness Analysis	Cost-Utility Analysis
Outcome	$\Delta \text{ Cost} / \Delta \text{ Health Effect}$	$\Delta \text{ Cost} / \Delta \text{ QALY}$

QALYs and Utilities

- $\text{QALY} = \# \text{ of years of life} * \text{Utility of life}$
 - Example:
 - # of years of life lived = 5
 - Utility = 0.8
 - $\text{QALY} = 5 * 0.8 = 4.0$
 - **An (imperfect) method of standardizing the value of life across health states and preferences**
-

Utilities

- Preference for health
 - Not just a measure of health!
 - Combine:
 - Health state a person is in
 - Valuation of health state
 - Conventionally range from 0-1
 - 0 = death
 - 1.0 = perfect health
 - More info in upcoming HERC lecture (March 11)
-

Utility Calculations

Variable	Jane's health (0-1)	Jane's valuation (sum to 1)		Joe's Health (0-1)	Joe's valuation (sum to 1)	
ADL	0.8	0.15	0.12	0.8	0.50	0.40
Exercise	0.2	0.40	0.08	0.2	0.10	0.02
Mental Clarity	0.4	0.40	0.16	0.4	0.25	0.10
Emotional well-being	0.9	0.05	0.045	0.9	0.15	0.135
Total	---	1.0	0.405	---	1.0	0.655

Utility \rightarrow QALY

- Jane's utility is **0.405**
 - Jane lives for **10** years
 - $0.405 * 10 = 4.05$ QALYs
 - Jane lives for **12** years
 - $0.405 * 12 = 4.86$ QALYs
- Joe's utility is **0.655**
 - Joe lives for **10** years
 - $0.655 * 10 = 6.55$ QALYs
 - Joe lives for **5** years
 - $0.655 * 5 = 3.275$ QALYs

Advantages of Utilities/QALYs

- Incorporate morbidity and mortality into a single measure
- Allows for comparison across disparate strategies
 - Newborn screening versus prostate cancer treatment
 - Early childhood education versus community health centers
 - Programs/interventions being considered may otherwise have very different outcome goals

Disadvantages of Utilities/QALYs

- Eliciting preferences is very hard
 - Time varying
 - Context dependent
 - Information asymmetry
 - Future uncertainty
- Assumptions can be made clear, but it does not make them stable or correct

More on preferences and utilities in lecture on March 11th!

ICERs in a CUA, Example

- $$\text{ICER} = \frac{\text{Cost}_B - \text{Cost}_A}{\text{QALY}_B - \text{QALY}_A}$$

	Program A	Program B
Intervention	Mobile text messaging for medication adherence	Diabetes care coordinator
Cost	\$40,000	\$150,000
QALYs	25	35

$$\text{ICER} = \frac{\$150,000 - \$40,000}{35 - 25} = \frac{\$110,000}{10} = \$11,000 \quad \textit{Cost-Effective}$$

Cost saving

- Cost-effective \neq cost-saving!!

Cost-Saving	Cost-Effective
Cost less, provides greater health	Costs more, provides proportionally more health
	Costs less, provides proportionally less health

Cost-Effective

■ Cost-Effective:

- Program B costs more than Program A, but Program B provides proportionally more health benefit than Program A

■ Proportional?

- ICER is $<$ Willingness to Pay Threshold
- This could be what society is willing to pay, the government, the insurance company, etc.

Willingness to Pay (WTP)

- U.S. –\$50,000/QALY often used
 - Willing to pay *up to* \$50,000 for one additional QALY
- Arbitrary, heavily criticized
 - Not an empirically-derived threshold

Thresholds for WTP

- Panel on Cost-Effectiveness in Health and Medicine does not endorse any WTP threshold
- Recommend to compare your results to a range of thresholds
- NICE (U.K.) does not have an explicit threshold for reimbursement
 - Recommended results are presented using WTP of £20,000 and £30,000

Cost-Benefit Analysis

Cost-Benefit Analysis

- Costs and Effects are expressed entirely in dollar terms
 - Convert health effect → cost

$$\text{Net social benefit} = \text{Incremental Benefit (cost)} - \text{Incremental costs}$$

- If Net social benefit is positive, then program is worthwhile

Assigning a dollar value to life

■ Willingness to Pay (WTP)

- Examine revealed WTP or elicit WTP
- Framing effects, loss aversion, age-related effects, varying levels of disposable income, risky behavior

■ Human Capital Approach

- Use projected future earnings to value a life
- Assumes an individual's value is entirely measured by formal employment.
 - Children? Retired people?
 - Pay differences by sex/gender

Cost-Benefit Analysis in Healthcare/Medicine

- Very rarely used:
 - Discomfort of assigning a dollar value to life
 - Problems with evaluating quality of life and converting to dollar amounts

Cost Consequence Analysis

Cost-Consequence Analysis

- Compare the costs and consequences (health outcomes) of multiple interventions
- Each cost and consequence is listed separately

Table I. Example of a cost-consequence tabulation

Cost components	Drug A		Drug B	
	units	costs	units	costs
Direct medical care use/costs				
Drug A/B				
Other drugs				
Physician visits				
Hospital stays				
Home care				
Other medical care (e.g. dialysis)				
Direct nonmedical care use/costs				
Transportation				
Crutches and other equipment				
Paid caregiver time				
Indirect resource use/costs				
Time missed from work for patient				
Time missed from work for unpaid caregiver				
Time missed from other activities for patient				
Time missed from other activities for unpaid caregiver				
Total direct and indirect costs				
Symptom impact				
Patient distress days				
Patient disability days				
Quality-of-life impact				
Quality-adjusted life-years decrement				
Quality-of-life profile measure scores				

Masukopf et al.
Cost-Consequence Analysis in Decision Making.
Pharmacoeconomics. 1998. 13 (3): 277-288.

Benefits and Drawbacks of CCA

■ Advantages

- Draws attention to *specific aspects* of cost or health outcomes that are most impacted

■ Disadvantages

- Does not indicate relative importance of various items
- Users may reach different conclusions about which intervention to pursue

Budget-Impact Analysis

Budget Impact Analysis

- Estimate the financial consequences of adopting a new intervention.
- Usually performed in addition to a cost-effectiveness analysis
 - CEA: does the intervention provide good value?
 - BIA: can we afford it?

BIA, example

Drug A has an ICER of \$28,000 per QALY compared with Drug B. It is cost-effective.

Drug B costs \$70,000.

Therefore, Drug A costs \$98,000. There are 10,000 people eligible for Drug A, resulting in a total cost of \$980 million dollars.

BIA tells us

- The true “unit” cost of the intervention
- The number of people affected by the intervention
- An understanding of the total budget required to fund the intervention

CEA versus BIA

	CEA	BIA
Purpose	Does this intervention provide high value?	Can we afford this intervention?
Outcome	Cost and health outcomes	Cost
Size of Population	Not explicitly considered	Explicitly Considered

Lecture on BIA on April 1st!

Poll question #2

- What type of decision analysis are you most interested in conducting?
- Answers: Cost-effectiveness, cost-benefit, cost-consequence, budget impact,

Approaches to Decision Analysis

Methods for decision analysis

- Modeling
- Measurement alongside a clinical trial

Types and Methods for Decision Analysis

	<i>Measurement alongside a clinical trial</i>	<i>Modeling</i>
Cost-Effectiveness Analysis	x	x
Cost-Benefit Analysis	x	x
Budget Impact Analysis		x

Measurement alongside a trial

- “Piggyback” onto an existing RCT
- Collect extra information from patients enrolled in the trial
 - Utilization (use this to assign costs)
 - Utilities
 - (Efficacy and AEs are already being collected)

More on using CEA with an RCT in lecture on March 25th!

Modeling

- No real-world experiment exists
 - Build a mathematical framework to understand the relationship between inputs and outputs
 - Build model structure in software, populate it with inputs (from literature). Run model to derive outputs
 - You decide on the boundaries of the analysis
 - Time frame, population, interventions of interest
-

Modeling versus Measurement

	Measurement	Modeling
Treatments considered	<ul style="list-style-type: none">• Only the ones in the RCT (which may include placebo)	<ul style="list-style-type: none">• Any of interest – But data also come from RCTs
Advantage	<ul style="list-style-type: none">• Design case-report forms• Individual-patient data (subgroup analysis)• Utilities may be more accurate (treatment and health condition specific)	<ul style="list-style-type: none">• Don't need to wait for a trial to be funded to do your analysis
Disadvantage	<ul style="list-style-type: none">• Short time frame – will still have to project beyond the trial• Will not provide all of your inputs• Utilities come from patient perspective, rather than community	<ul style="list-style-type: none">• Inputs need to come from similar studies on your population of interest

Cost-effectiveness Analysis for Resource Allocation

How is CEA used for decision making?

- Ex-US: Used by NICE (U.K.), PBAC (Australia), CADTH (Canada) for regulatory/market access purposes
- US: Medicare has historically not used cost-effectiveness to drive coverage decisions, ACA prohibits this

“(e) The Patient-Centered Outcomes Research Institute established under section 1181(b)(1) shall not develop or employ a dollars-per-quality adjusted life year (or similar measure that discounts the value of a life because of an individual’s disability) as a threshold to establish what type of health care is cost effective or recommended. The Secretary shall not utilize such an adjusted life year (or such a similar measure) as a threshold to determine coverage, reimbursement, or incentive programs under title XVIII.”

(d) IN GENERAL — Part D of title VI of the Social Security

U.S. Cost-Effectiveness Analysis

- Pharmaceutical companies – international markets
- Academia
- Veterans Health Administration

- NOT used by FDA or CMS

Summary

- Major types of decision analysis:
 - Budget Impact Analysis
 - Cost-Benefit Analysis
 - Cost-Consequence Analysis
 - Cost-Effectiveness Analysis
 - Cost-Utility Analysis - QALYs, a measure of morbidity and mortality
 - Operationalize your decision analysis:
 - Measurement alongside a clinical trial, or
 - Modeling
 - Cost-effective \neq cost-saving!
-

Resources:

Decision Analysis and CEA

- Neuman PJ, Saunders GD, Russell LB, Siegel JE, Ganiats TG, eds. Cost-Effectiveness in Health and Medicine. Second Edition. New York: Oxford University Press; 2017.
- Gold MR, Siegel JE, Russell LB, Weinstein MC, eds. Cost-Effectiveness in Health and Medicine. New York: Oxford University Press; 1996.
- Hunink M, Glasziou P, Siegel J, et al. Decision Making in Health and Medicine: Integrating Evidence and Values. Cambridge, UK: Cambridge Press; 2004.
- Muennig P. Designing and Conducting Cost-Effectiveness Analyses in Medicine and Health Care. San Francisco, CA: Jossey-Bass; 2002.

Questions?

liam.rose@va.gov