Epi213_Lecture2

Cost-Effectiveness Analyses in Medicine and Public Health
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Agenda for the day

1. Logistical issues re homework
2. Quick review: what makes a good question, ICERs, cost-effective v cost-saving
3. Components of a CEA: PICO-HS
4. Key concept: expected value,
5. Decision tree
6. Base-case and sensitivity analyses
7. Key steps in formulating a decision tree
8. Reference case
9. If time permits: rates v probabilities, discounting
10. Plan for next week
11. Sign up for the colloquium? Feb 10, 2017

Any logistical issues re homework?

REVIEW FROM LECTURE 1:

What constitutes a good research question?

***meaningful* equipoise**

$$\text{RESOURCE ALLOCATION} = f\,(\text{efficiency, equity, political imperatives, other considerations})$$

Incremental Cost-Effectiveness Ratio (ICER):

Consider a medical condition X for which therapy A is the current standard of care. A new therapy B has been discovered and proven to be more effective than A; it is also more expensive than A.

$$\text{INCREMENTAL COST-EFFECTIVENESS RATIO (ICER)} = \frac{\text{Cost}_B - \text{Cost}_A}{\text{Outcomes}_B - \text{Outcomes}_A}$$

“Thresholds”:

For the US, used to be \$50,000/QALY, now \$100,000/QALY (arbitrary, consensus-based)
For most Western European countries, a more explicit threshold of 20-30K euros per QALY
For the UK, an implicit threshold of 15K-25K pounds per QALY

WHO recommendation (arbitrary):
ICER < 1 GDP per capita per QALY = highly cost-effective
ICER > 3 GDP per capita per QALY = not cost-effective

Cost-Saving vs. Cost-Effective

Cost-saving implies implementing the new intervention generates the same or better outcomes but at a lower cost. Thus the new option is called the *dominating* option (aka a "no brainer").

Cost-effective implies that the new intervention/strategy being evaluated costs more but the improved outcomes would make the additional investment seem worthwhile or of reasonable value. In a practical sense, one would calculate the incremental cost-effectiveness ratio and compare it with some *willingness-to-pay* threshold (e.g., 100,000 dollars per quality-adjusted life year), and if ICER <= WTP, we would declare it cost-effective.

Components of a good CEA question

Population – age/gender/geography ± inclusion criteria

Intervention

Control (even if "status quo")

Outcomes (be very specific)

Horizon = timeline for analysis – longer better bcoz it captures complete costs + outcomes but more uncertainty 2° to extrapolation of short term data

Perspective

"Health System" NOT "Societal"

Discuss Examples of good CEA questions

Among gay men age 35-45years in San Francisco, is the provision of an online behavioral intervention cost-effective relative to usual care in reducing tobacco use and improving quality-adjusted survival using a 10-year analytic horizon and a health system perspective?

Among persons age 25-60 years in India who have a previous history of coronary heart disease, is treatment with a fixed dose combination pill of evidence-based medications cost-effective relative to usual care, considering a health system perspective and a lifetime analytic horizon?

Key concept:

Expected value

Estimate the expected values in the following scenarios:

Scenario 1
50% chance of survival for 10 years,
25% chance of survival for 5 years, and
25% chance of immediate death.
Expected survival = _6.25__ years

Scenario 2
1% chance the cost is $1,000,000,
33% chance the cost is $100,000,
33% chance the cost is $10,000, and
33% chance the cost is $0.
Expected cost = $__46,300

Creating a Decision Tree

Background: A woman is incidentally discovered to have a 6mm aneurysm (dilatation) of a blood vessel in the brain. These aneurysms are usually asymptomatic but still at risk of spontaneously bursting and bleeding into the brain, resulting in severe disability or death. The alternative is to surgically clip the aneurysm; this surgery carries a risk of peri-op mortality; but survivors can expect a normal life expectancy. **Should our patient have surgery?**

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Research Question?

<< Assignment 2>>

Decision Tree:

<< Assignment 2>>

Input parameters:

Transition Probabilities:
Life Expectancy from diagnosis = 35 years
Lifetime risk of rupture with watchful waiting = 5.0%
A ruptured aneurysm would require emergent surgery: 45% will die, 25% will have severe disability, the remaining will survive
Surgical mortality = 2%
If successfully operated, normal life expectancy (35 years)

Costs:
Elective Surgery: $30,000
Emergent Surgery and recovery: $150,000
Disabled care (lifetime): $1,000,000

Quality of Life:

Full Health = 1
Severe disability = 0.3
Death = 0

Base-Case Analysis

<< Assignment 2>>

Sensitivity Analyses

What if the surgical mortality were 2.5%?

<< Assignment 2>>

Key Steps in a Decision Analysis:

- Meaningful question
- Clearly define PICO-HS, particularly Outcomes
- Search for transition probabilities
- Search for outcomes and costs
- Perform a base-case analysis
- Perform sensitivity analyses
- Communicate your findings

General Approach:

- Ask a meaningful question
- Start simple, then iteratively expand/ealborate
- Allocate approximately equal time to the decision tree, data collection, sensitivity analyses, and communication
- Delve into and enjoy modeling. Have fun with the nuances. Master the challenges.

Seeking Transition Probabilities

Key Concepts:
 # Rates vs probabilities

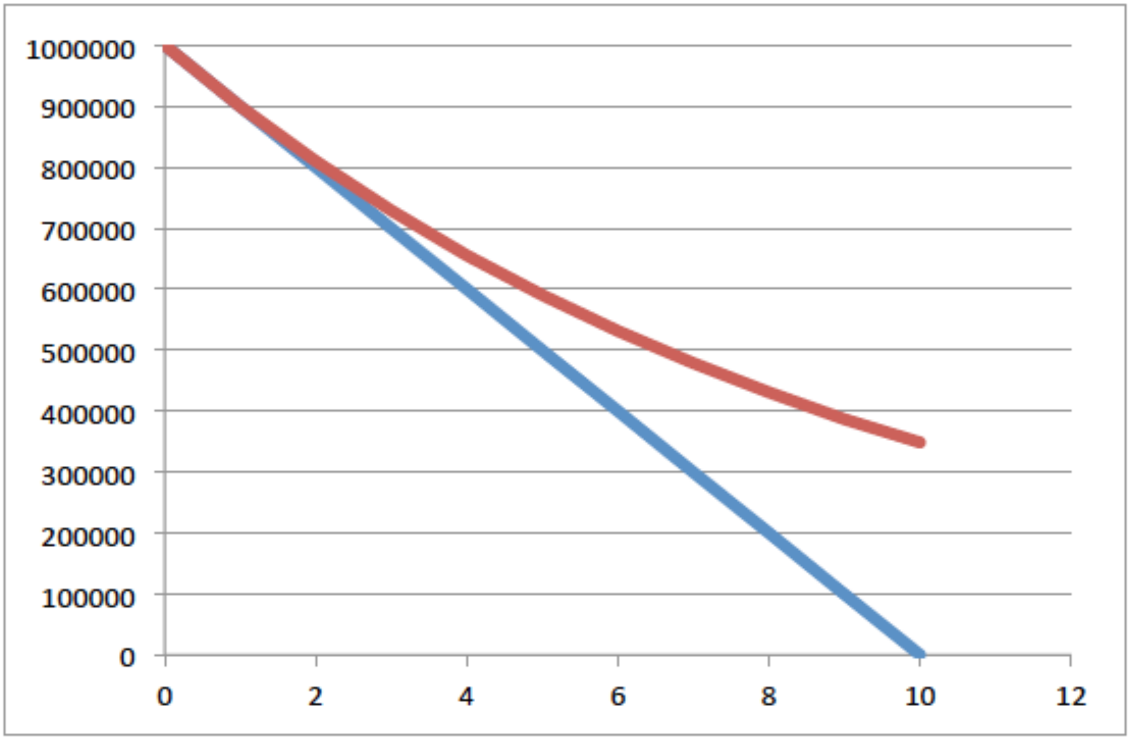
Homework Qs 1-3

1,000,000 people —> mortality 100,000 a year vs. 1,000,000 people —> mortality 10% a year

Survival:

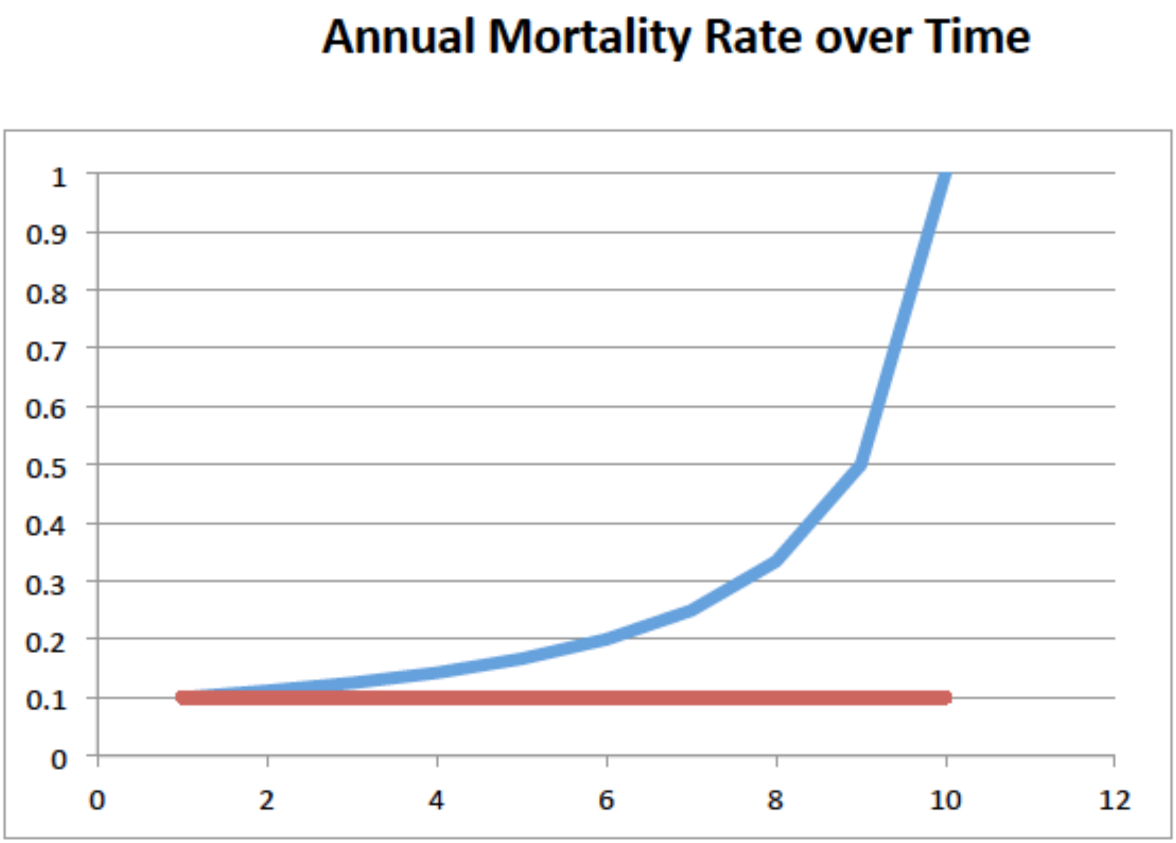
Time (year)	Scenario 1: Persons alive	Scenario 2: Persons Alive
0	1000000	1000000
1	900000	900000
2	800000	810000
3	700000	729000
4	600000	656100
5	500000	590490
6	400000	531441
7	300000	478297
8	200000	430467
9	100000	387420
10	0	348678

Surivival over Time



Annual Mortality Rate:

Time (year)	Scenario 1: Persons alive	Scenario 2: Persons Alive		Scenario 1: Annual Mortality Rate	Scenario 2: Annual Mortality Rate
0	1000000	1000000			
1	900000	900000		0.1	0.1
2	800000	810000		0.11111111	0.1
3	700000	729000		0.125	0.1
4	600000	656100		0.14285714	0.1
5	500000	590490		0.16666667	0.1
6	400000	531441		0.2	0.1
7	300000	478297		0.25	0.1
8	200000	430467		0.33333333	0.1
9	100000	387420		0.5	0.1
10	0	348678		1	0.1



Why do we care?
(mathematical transformation and time trends)

Converting rates to probabilities

Probability to rate

$$\text{Rate} = \frac{-\ln(1-p)}{t}$$

Rate to probability

$$\text{Probability} = 1 - exp^{(-rt)}$$

<<The following material was deferred to a future class >>

Practice questions

The probability of experiencing a breast cancer relapse in a particular cohort is 50% at 1 year. What is the rate of relapse in this cohort?

In an urban population of high-risk individuals, the rate of HIV seroconversion is 5 per 100 per year. What is the probability of seroconversion at 5 years?

In a particular high-risk cohort, the probability of developing diabetes is 60% at 3 years. What is the probability at 1 year?

In the control arm of a randomized study, the rate of death is 300 per 1000 per year. In the intervention arm, the rate is halved. What is the probability of death at 5 years in the intervention arm?

TreeAge Tip:
RatetoProb; ProbtoRate; RatetoRate functions already built in

#Odds Ratio vs Relative Risk

#Precision vs Accuracy (Bias-Variance trade-off)

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Discounting

Conclusion

The usefulness of these analytic techniques should not be overstated. None… is intended to be a magic bullet for removal of judgment, responsibility or risk from decision-making…, though each is capable of improving the quality and consistency of decision making.

At root, they are methods of critical thinking, of approaching choices, and often of placing difficult choices out in the open for discussion.

Drummond MF, SchulpherMJ, Torrance GW, et al.

Methods for the Economic Evaluation of Health Care Programmes

Oxford, 2005

Supplemental notes:

Statistic	Evaluates	Range
Probability/Risk (aka Incidence Proportion)	$\frac{\text{\# of events that occurred in a time period}}{\text{\# of people followed for that time period}}$	0-1
Rate	$\frac{\text{\# of events that occurred in a time period}}{\text{Total time period experienced by all subjects followed}}$	0 to ∞
Relative Risk (aka Risk Ratio)	$\frac{\text{Probability of outcome in exposed}}{\text{Probability of outcome in unexposed}}$	0 to ∞
Odds	$\frac{\text{Probability of outcome}}{1 - \text{Probability of outcome}}$	0 to ∞
Odds Ratio	$\frac{\text{Odds of outcome in exposed}}{\text{Odds of outcome in unexposed}}$	0 to ∞
Risk Difference	Difference in risk (probability) of event amongst exposed and unexposed	-1 to 1
Survival Curve	Point = # of people who are alive at time t | being alive at time t - 1	0 to n
Mean	$\frac{\text{Sum of all observations}}{\text{Total \# of observations}}$	- ∞ to ∞
Mean Difference	Mean of Group 1 – Mean of Group 2	0 to ∞
Standardized Mean Difference	$\frac{\text{Mean of Group 1} - \text{Mean of Group 2}}{\text{Pooled Standard Deviation}}$	- ∞ to ∞

Statistic	Evaluates	Type of Data
Probability/Risk	$\frac{\text{\# of events that occurred in a time period}}{\text{\# of people followed for that time period}}$	Non-Comparative
Rate	$\frac{\text{\# of events}}{\text{Total time period experienced by all subjects followed}}$	Non-Comparative
Odds	$\frac{\text{Probability of outcome}}{1-\text{Probability of outcome}}$	Non-Comparative
Odds Ratio	$\frac{\text{Odds of outcome in exposed}}{\text{Odds of outcome in unexposed}}$	Comparative
Relative Risk (aka Risk Ratio)	$\frac{\text{Probability of outcome in exposed}}{\text{Probability of outcome in unexposed}}$	Comparative
Risk Difference	Difference in risk (probability) of event amongst exposed and unexposed	Comparative
Survival Curve	Point = # of people who are alive at time t | being alive at time t - 1	Non-Comparative
Mean	$\frac{\text{Sum of all observations}}{\text{Total \# of observations}}$	Non-Comparative
Mean Difference	Mean of Group 1 – Mean of Group 2	Comparative
Standardized Mean Difference	$\frac{\text{Mean of Group 1}-\text{Mean of Group 2}}{\text{Pooled Standard Deviation}}$	Comparative

OR is a good measure of RR <u>when the outcome is rare</u>

What's the Relative Risk?—Zhang & Yu

JAMA, November 18, 1998—Vol 280, No. 19