Framing an Economic Evaluation and Decision

Parts 5 and 6

Framing an Economic Evaluation and Decision Analysis

Sean D. Sullivan, PhD
David L. Veenstra, PharmD, PhD

Outline

- Framing the study
- Research question
- Data sources
- Errors in decision making
- How to perform a decision analysis
- Software demonstration

Objectives of analysis

- Inform policy makers about the value of a health care program
  - what are the incremental costs?
  - what are the incremental benefits - both clinical and humanistic?
  - what are to opportunity costs? e.g., what are the alternatives?
  - is the program worth the additional cost?

Objectives of analysis

- Needs of policy makers
  - clearly defined question
  - relevant intervention, population
  - costs only
  - benefits only
  - short-term
  - transparent assumptions
  - implications of assumptions
- May conflict with your goals

Objectives of analysis

- Specific decision or general policy
  - decision at your institution or health care system?
  - state or federal level?
  - general guidelines?

Audience

- Specific audience
  - guidelines (e.g., PBAC, CCOHTA, NICE, Regence/MCO, PHS)
- General audience
  - influence opinion (e.g., national guidelines)
- Secondary audience
  - patients, press, individual providers

Types of analyses

- Economic evaluation: which one and why?
  - cost-minimization
  - cost-consequences
  - cost-effectiveness
  - cost-utility
  - cost-benefit

Perspective

- Societal perspective is “recommended”
  - not a “governmental” perspective
- Health care institution
- Third-party payer
- Patient and family
- Multiple perspectives

Define the intervention

- Frequency
- Patient population
  - age, sex, disease severity, comorbidities
- Applicability for policy makers
- Delivery of program
  - e.g., NP's vs. Physicians
  - inpatient vs. outpatient

Target population

- Population should reflect audience
- Sub-group analyses
  - significant differences
  - difficulty obtaining clinical data (loss of precision)
- Sub-groups should be feasible

Example: Hypercholesteremia

- 1985 National Cholesterol Education Program (NCEP) created
- 1988 NCEP recommended cholesterol be checked
  - every 5 yrs. for all adults
  - high risk (>240 mg/dl or 200-239 + risk factor) more often
  - Patients with high LDL levels should get diet/drug
  - 1/3 of adult population eligible for diet/drug

Example: Hypercholesteremia

	Cost per year of life saved		
Cholesterol >300	Low-risk patient	All patients	High-risk patient
Women 35-44	$1,500,000		$195,000
Men 55-64	$58,000		$15,000
Heart disease, cholesterol >250			
Women 35-44		$4,500	
Men 55-64		$1,600	

Goldman et al, JAMA 1991;265:1145

Example: Hypercholesteremia

- In response to studies like these, NCEP 1994 guidelines were more modest
- 1988 NCEP guidelines:
  - $20-27 billion would be spent for 20 mg/day
  - $47-67 billion to provide 80 mg/day
  - Thus, more selective strategies can free significant resources for investment in other areas of healthcare

Comparison program

- One of the most important aspects of an economic evaluation
- Include “do-nothing” option?
- Common practice or existing guidelines?
- May be a variety of comparators
  - select each one
  - use as mixture

Comparator cont'd

- Use next less-intensive program as comparator
  - e.g., annual BrCA screening vs. biannual screening
- Include intensities that are feasible
  - also can inform future studies

Example: Cervical Cancer Screening

- 1980 ACS recommended women aged 20-65 <annually if 2 negative tests
- 1988 discretion of physician after 3 negative tests
- 1980 recommendations based on study by Eddy

Example: Cervical Cancer Screening

	Cost per year of life saved			
Strategy	4 years	3 years	2 years	1 year
Compared to no screening	$10,101	---	---	---
Compared with screening at the next longer interval	---	$184,500	$262,800	$1,100,000

Eddy Ann Int Med 1990;113:214.

Example: Cervical Cancer Screening

- Increasing frequency of screening is an expensive way to save lives
- Starting or ending age did not have a big impact
- Req. for 3 neg. smears added only hours of life expectancy

Example: Cervical Cancer Screening

- Screening women who have not been screened regularly significantly better
- Thus, may decide to invest resources in providing screening to more women rather than more screening to those already being screened

Boundaries

- Individuals included
- How far do "spillover" effects go?
  - E.g., smoking cessation
- Examples
  - childhood illness affects parents
  - infectious diseases can affect many
  - debilitating diseases affect families
- Magnitude of effect should dictate inclusion/exclusion

Boundaries

- Health outcomes
  - QoL domains: physical, mental, emotional
- Non-health
  - costs of providing care for disabled
  - income effects

Time Horizon

- Need to capture the major health and economic outcomes, both intended and unintended
- Lifetime analyses common
- Discounting

Identify and bound the policy decision

- What is the decision being faced?
- What are the potential alternative actions?
- What are the events that follow the decision?

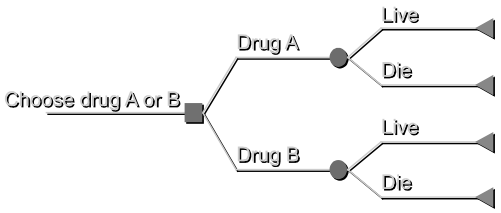
Conceptual model

- Outlines an “event pathway”
  - Disease pathway
  - Effect of intervention on disease
  - Other effects of intervention
  - Cost events may necessitate modification of model

Structure the problem

- Use a decision tree
- A decision tree depicts graphically the components of the decision problems and relates actions to consequences

Decision tree



Research Question

- Having addressed the preceding, you should be able to clearly state your research question
- What is your overall goal?
- What is your hypothesis?
- What are your specific aims?

Example: Neural tube defects

- Background
  - NTD's effect 4,000 pregnancies per year
  - Spina bifida and anecephaly most common
  - US PHS recommends 0.4 mg folic acid qd, but less than 1.0 mg qd
  - At >1.0 mg qd, possible that B12 deficiency would go undiagnosed
  - Neurological manifestations of B12 deficiency are most commonly parathesias, but CNS impairments and death have been reported

Gold book, p. 313

Example: Neural tube defects

- Objectives
  - PHS recommendation includes 3 possible strategies
    1) improved dietary habits
    2) fortification of US food supply
    3) dietary supplements
  - FDA considering a rule requiring manufacturers to fortify cereal

Example: Neural tube defects

- Audience
  - Primary
    - Public health service
    - FDA
  - Secondary
    - Clinicians
    - Population at risk
    - Patients with NTD's

Example: Neural tube defects

- Perspective
  - Societal

Example: Neural tube defects

- Type of analysis
  - Cost utility analysis
  - Why QALY's?
    - captures both premature morbidity and mortality, e.g., anencephaly, spina bifida, parathesias

Example: Neural tube defects

- Time horizon
  - Lifetime costs and effects of 1 year of the program
  - All costs and benefits which occur *as a result of* actions taken during the first year are included, regardless of when they occur
- Time preference
  - 3% annual discount rate

Example: Neural tube defects

- Target population
  - All women in US capable of becoming pregnant
  - Rates of folate deficiency vary widely, but few data available to estimate potential differential effects

Example: Neural tube defects

- Intervention
  - Food fortification
    - 3 fortification levels: 0.07, 0.14, and 0.35 mg/100g
    - passive, not targeted
  - Supplementation
    - based on public-health behavior-change programs
    - active, targeted

Example: Neural tube defects

- Scope of the study
  - Many parties are affected
  - Intergenerational, broad-based
  - 2 groups chosen
    - NTD-affected births of at least 20 weeks gestation
    - people with vit. B12-related neurological complications resulting from masking of symptoms
  - Psychological and physical impacts on families not considered
  - Dietary improvement strategy not considered

Remember - building a conceptual model

- is a dynamic process
- is driven (unfortunately) as much by data availability/feasibility as natural disease progression
- Investigate different models