Introduction to Medical Decision Making and Decision Analysis

Based on the lecture of Gillian D. Sanders Ph.D.
Clinical v. Statistical Approaches

"While I understood statistics, I failed to understand people."

Clinical

- Based on human judgment
- Subjective evaluation/case study
- Qualitative and narrative
- Sensitive to human bias

Statistical

- Based on probability
- Objective data from large sample sizes
- Quantitative
- Sensitive to measurement bias
Why do people adhere to clinical approach?

• Unaware…
  - of the biases that can affect their decisions
  - that the statistical approach is available
  - of how to implement the statistical approach

• Aware but resistant
  - False belief in the validity of their judgments
  - Desire for perfect prediction
  - Fear of losing “human element, their uniqueness, their jobs
Why is the “statistical approach” superior?

- **Validity**
  weighting different types of information

- **Units of Measurement**
  combining different types of information

- **Reliability**
  using the same decision rule given same situation

- **Redundancy**
  taking into account redundant information

- **Regression Effects**
  getting closer to the mean the more times you test it
Decision Analysis

- Decision analysis is a quantitative, probabilistic method for modeling problems under situations of uncertainty.
Making a Decision

• We make a decision when we irreversibly allocate resources.

• We typically use the following steps:
  » gather information
  » assess consequences of the alternatives
  » take an action

• Goal of decision analysis is to clarify the dynamics and trade-offs involved in selecting one strategy from a set of alternatives

• Usually, in everyday decision-making, we do not take the time to thoroughly analyze the decision
Decision Analysts

- Deliberately seek out new, creative alternatives
- Identify the possible outcomes
- Identify relevant uncertain factors
- Encode probabilities for the uncertain factors
- Specify the value placed on each outcome
- Formally analyze the decision.
Decisions Vary in Degree Of:

- **Complexity** -- large number of factors, multiple attributes, more than one decision-maker

- **Time factor** -- static (no change over time) vs. dynamic (time-dependent)

- **Uncertainty** -- *deterministic* vs. *probabilistic*. Deterministic means there is no uncertainty and the problem can be solved with a set of precise equations
Decision Analysis is Most Helpful

• For important, unique, complex, nonurgent, and high-stakes decisions that involve uncertainty

• “Decision Analysis = Decision Therapy.”
  » A great deal of work is done to decompose the decision problem, work out the relation between factors, specify probabilities for uncertain events, and identify what is at stake and how it might be affected by the decision

  » Constructing the tree, even before “solving” it mathematically, can provide important insights.
Cost-Effectiveness Analyses

• Cost-effectiveness analysis (CEA) is a methodology for evaluating the tradeoffs between health benefits and costs

• CEA is aid to decision making, not a complete resource allocation procedure
Cost-Effectiveness Ratio

Compares a specific (new) intervention to a stated alternative (old) intervention

\[
\frac{\text{Cost}_{\text{new}} - \text{Cost}_{\text{old}}}{\text{Benefit}_{\text{new}} - \text{Benefit}_{\text{old}}} \]

- Incremental resources required by the intervention
- Incremental health effects gained by using the intervention
Decision Model

- Schematic representation of all the clinical important outcomes of a decision.
- Used to combine knowledge about decision problem from many sources
- Computes average outcomes (e.g., QALYs, costs, etc.) from decisions.
Elements of Decision Analysis

- Structure the problem
  - Identify decision alternatives
  - List possible clinical outcomes
  - Represent sequence of events
- Assign probabilities to all chance events
- Assign utility, or value, to all outcomes
- Evaluate the expected utility of each strategy
- Perform sensitivity analyses
Structuring the Problem

• Decision model (usually decision tree) is chosen to represent the clinical problem

• Model needs to be simple enough to be understood, but complex enough to capture the essentials of the problem

• Need to make a series of assumptions for modeling
Decision Node:
A point in a decision tree at which several choices are possible. The decision maker controls which is chosen.

Only 2 choices shown here. But can have more, as long as they are mutually exclusive.
Chance Node:
A point in a decision tree at which *chance* determines which outcome will occur.

- Disease present
- Disease absent

Only 2 outcomes shown here. But can have more, as long as they are *mutually exclusive* and *collectively exhaustive*.
Some Definitions

• Mutually exclusive
  » The intersection of the events is empty
  » One (and only one) of the events must occur

• Collectively exhaustive
  » Events taken together make up the entire outcome space
  » At least one of the events must occur
Terminal Node:
Final outcome state associated with each possible pathway

Some measure of value or worth needs to be applied to the terminal nodes (e.g., LYs, QALYs, costs)
Outline

• Decision analysis
  » Components of decision analysis
  » Building a tree: Example
  » Sensitivity analyses

• Markov models
  » Background
  » Constructing the model
  » Example
  » Monte Carlo simulations
Example

- Symptomatic patient:
  - operate (risky)
  - medical management
- If disease present at surgery, must decide whether try for cure or palliate
- Want to evaluate surgery vs. medical management
Each path through the tree defines a unique potential result.

1. Decide to operate
2. Find disease at surgery
3. Try for surgical cure
4. Patient survives surgery
5. Surgery unsuccessful
Insert probabilities at each chance node. Sources include data from literature/studies, modeling, expert judgment...

- Surgery: 10% disease present, 90% disease absent
  - Disease present: 10% survive, 90% operative death
  - Disease absent: 99% cure, 1% operative death

- Drug: 10% disease present, 90% disease absent
  - Disease present: 10% cure, 90% no cure
  - Disease absent: 98% palliate, 2% operative death

- Try cure: 90% survive, 10% no cure
  - Cure: 90% survive, 10% no cure
Compute average results, working right to left...

Average LYs here:

\[
= 10\% \times 20 + 90\% \times 2 \\
= .1 \times 20 + .9 \times 2 \\
= 2.0 + 1.8 \\
= 3.8 \text{ LY}
\]
Replace these chance nodes with the average...

Average LYs here:
\[
= 10\% \times 20 + 90\% \times 2 \\
= .1 \times 20 + .9 \times 2 \\
= 2.0 + 1.8 \\
= \textbf{3.8 LY}
\]
Replace these chance nodes with the average…

Average LYs here:

\[
= 10\% \times 20 + 90\% \times 2
\]

\[
= 2.0 + 1.8
\]

\[
= 3.8 \text{ LY}
\]
Replace next chance node with the average...

Average LY here:

\[ \text{Average LY} = 0.1 \times 3.8 + 0.9 \times 20 \]

\[ = 0.38 + 18 \]

\[ = 18.38 \text{ LY} \]
Replace next chance node with the average...

Average LY here:

\[
= 10\% \times 3.8 + 90\% \times 20 \\
= 0.1 \times 3.8 + 0.9 \times 20 \\
= 0.38 + 18 \\
= 18.38 \text{ LY}
\]
Continue this process...

- **surgery**
  - disease present
    - 10% cure
    - 90% survive
      - 99% survive
        - 20 LY average
      - 1% operative death
        - 0 LY average
  - disease absent
    - 90% survive
      - 99% survive
        - 20 LY average
    - 1% operative death
      - 0 LY average

- **drug**
  - 10% cure
  - 90% survive
    - 90% survive
      - 20 LY average
    - 10% operative death
      - 0 LY average
  - 10% no cure
    - 2 LY

Average LY here:

\[
= 98\% \times 3.8 + 2\% \times 0
= .98 \times 3.8 + .02 \times 0
= 3.72 + 0
= 3.72 \text{ LY}
\]
Continue this process...

surgery

- disease present
  - 10%
  - 90%

- disease absent
  - 90%
  - 1%

- drug
  - 18.38 LY

- survive
  - 90%
  - 99%
  - 1%

- operative death
  - 10%
  - 0%

- palliate
  - 20 LY

- cure
  - 90%
  - 10%

- no cure
  - 2 LY

Average LY here:

= 98% x 3.8 + 2% x 0
= .98 x 3.8 + .02 x 0
= 3.72 + 0
= 3.72 LY
Continue this process...

- Disease present
  - 10% surgery
  - 90% disease absent
    - 90% surgery
    - 1% surgery
- Operative death
  - Survive
    - 90% cure
    - 10% no cure
  - Palliate
    - 3.72 LY
- Average LY here:
  \[
  = 90\% \times 20 + 10\% \times 2
  = .90 \times 20 + .1 \times 2
  = 18 + .2
  = \textbf{18.2 LY}
  \]
Continue this process...

- **disease present**
  - surgery: 10%
  - drug: 90%

- **disease absent**
  - surgery: 90%
  - drug: 1%

- **survive**
  - cure: 90%
  - palliate: 99%

- **operative death**
  - cure: 10%
  - palliate: 1%

Average LY here:
\[
\text{Average LY} = 90\% \times 20 + 10\% \times 2
\]
\[
= .90 \times 20 + .1 \times 2
\]
\[
= 18 + .2
\]
\[
= 18.2 \text{ LY}
\]
Continue this process...

- Disease present
  - Surgery: 10%
  - Drug: 90%
    - Disease absent
      - Surgery
        - Survive: 20 LY
      - Drug
        - Operative death: 0 LY
    - Palliate
      - 3.72 LY

Average LY here:

\[
\text{Average LY} = 0.9 \times 18.2 + 0.1 \times 0 = 16.38 + 0 = \text{16.38 LY}
\]
Continue this process...

Average LY here:

\[
= 90\% \times 18.2 + 10\% \times 0 \\
= .90 \times 18.2 + .1 \times 0 \\
= 16.38 + 0 \\
= \boxed{16.38 \text{ LY}}
\]
At a **Decision Node** you choose which path you wish to take -- no averaging!

Here, we would choose to try for cure -- obviously!
- Disease present:
  - Surgery: 10% chance of cure, 90% chance of disease absence.
  - Disease present:
    - Surgery: 10% chance of cure, 90% chance of disease absence.
    - Disease absent:
      - Surgery: 10% chance of cure, 90% chance of disease absence.

- Disease absent:
  - Surgery: 10% chance of cure, 90% chance of disease absence.
  - Drug: 18.38 life years (LY).

- Operative death:
  - Surgery: 1% chance of death, 99% chance of survival.
  - Drug: 0 LY.

- Cure:
  - Surgery: 10% chance of cure, 90% chance of disease absence.
  - Drug: 16.38 LY.
Continue working from right to left, averaging out at Chance Nodes, and choosing best branch at Decision Nodes...

Average LY here:

\[0.99 \times 20 + 0.01 \times 0 = 19.8 \text{ LY}\]
Average LY here:
= 99% x 20 + 1% x 0
= .99 x 20 + .01 x 0
= 19.8 + 0
= **19.8 LY**
Average LY here:

\[
= 10\% \times 16.38 + 90\% \times 19.8 \\
= 0.1 \times 16.38 + 0.9 \times 19.8 \\
= 1.638 + 17.82 \\
= 19.46 \text{ LY}
\]
Average LY here:

= \(10\% \times 16.38 \ + \ 90\% \times 19.8\)

= \(0.1 \times 16.38 + 0.9 \times 19.8\)

= 1.638 + 17.82

= \(19.46\) LY
The outcome for each decision is more apparent now:

- **Surgery (intending cure)** produces an average of **19.46 LY**
- **Medical management** yields an average of **18.38 LY**

The **incremental** benefit of Surgery versus Medical Management is:

\[ 19.46 - 18.38 = \boxed{1.08 \text{ LY}} \]
Repeat this Decision Analysis Using Other Outcome Measures

- Instead of just using average life years, can use QALYs at each endpoint.

- If you use both costs and QALYs at each endpoint:
  - Then can calculate the incremental cost effectiveness of surgery versus medical management
Sensitivity Analysis

- Systematically asking “what if” questions to see how the decision result changes.
- Determines how “robust” the decision is.
- Threshold analysis: one parameter varied
- Multi-way analysis: multiple parameters systematically varied
Sensitivity Analysis: Probability of Operative Death

Life Expectancy, Years

Probability of Operative Death

Surgery
Drug

Threshold
Base Case
Two-Way Sensitivity Analysis: 
$p_{\text{Disease}}$ vs. $p_{\text{Operative Death}}$
Summary: Medical Decision Analysis

• Clearly defines alternatives, events, and outcomes
• Formal method to combine evidence
• Can prioritize information acquisition
• Can help healthcare providers to make medical decisions under uncertainty