MEDICAL DECISION MAKING

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CHAPTER SIX

EXPECTED VALUE DECISION MAKING

With this chapter, our emphasis shifts from *diagnosis* to *making choices.* A clinician makes many choices every working day: a test is ordered, a treatment is chosen, or a patient is hospitalized. The experienced clinician makes many decisions easily because there is little at stake or because the problem is familiar. The experienced clinician has standard operating procedures for many such situations. These heuristics, or *clinical policies,* are among the distinguishing features of the master clinician.

Unfamiliar problems are important sources of intellectual stimulation for clinicians. These situations often require careful thought before a choice is made. When perplexed, the physician seeks help from a colleague or goes to the library. These responses are important but are not always sufficient, because the solutions obtained from the medical literature or a colleague's personal experience may not apply to an individual patient. Required resources may be unavailable. Clinicians need a general approach to decision making when the situation is unique, the outcome is uncertain, and the stakes are high or when trying to formulate a clinical policy that will be applied many times in the future.

There are several quantitative methods that use the ideas developed in the preceding chapters to solve difficult clinical decision problems. These methods include:

- *The decision tree:* A method for representing and comparing the expected outcomes of each of the management alternatives
- *Threshold probability:* A method for deciding if new information can change a management decision

These methods enable the decision maker to understand the decision problem and choose the management alternative that is most likely to help the patient.

The purpose of this chapter is to show how decision analysis is applied to an easy clinical problem. In learning about decision analysis, the reader will understand the importance of measuring clinical outcome, which will be described in the following two chapters. Decision trees and decision thresholds are the subjects of subsequent chapters. At that point, the reader will have learned a general approach to making difficult clinical decisions.

This chapter is divided into two parts:

A. Basic concepts of expected value decision making
B. Decision analysis: An introductory example

A. BASIC CONCEPTS OF EXPECTED VALUE DECISION MAKING

A.1 Comparing Uncertain Prospects

Most biological events occur randomly. Consequently, the outcome of an individual's illness is unpredictable. Even something as stereotyped as a common cold may last 2 days, 1 week, or even 2 weeks. Choosing among treatments with unpredictable effects is a challenging problem. Consider two hypothetical treatments for a fatal illness. Survival after undergoing either treatment is unpredictable, as illustrated by a frequency distribution of length of life (see Figure 6–1). These distributions may also be summarized in tabular form, as in Table 6.1.

Giving treatment A is a gamble. There is no way to know which year will be the patient's last. The time of death is also uncertain with treatment B. Although survival until the fourth year is more likely with treatment B, the patient might die in the first year with treatment B and survive to the fifth year with treatment A. Thus, the choice between these treatments is a choice between gambles.

How should one choose between these two gambles? One approach is to characterize each gamble by one number and use that number as the basis for choice. The ideal criterion for choosing a gamble should be a number that reflects preferences for the outcomes of the gamble. *Utility* is the name given to a measure of preference that has a desirable property for decision making: the gamble with the highest utility *should* be preferred. Utility will be described in Chapter 7.

FIGURE 6–1
Time of death after two hypothetical treatments for a fatal disease.

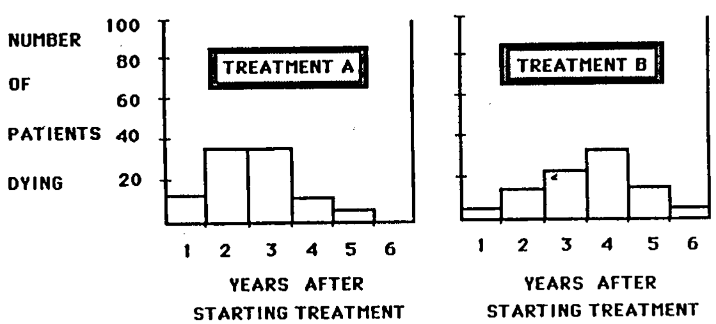


TABLE 6.1
Mortality Probabilities of Two Treatments

	Probability of Death	
Years after Treatment	*Treatment A*	*Treatment B*
1	.15	.05
2	.35	.15
3	.35	.25
4	.10	.35
5	.05	.15
6	0	.05

For this introductory chapter, we will use the average duration of life to summarize gambles involving length of life. The mean duration of survival is obtained by weighting the length of a period of survival by the probability that death will occur at the end of that period and summing these over all possible lengths of life. (Death during an interval is assumed to occur at the midpoint of the interval.)

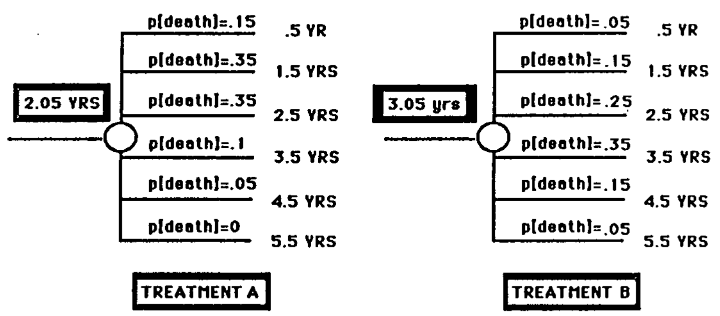
$$\begin{aligned}\text{Mean survival for treatment A} &= (.15 \times .5\text{ yrs}) + (.35 \times 1.5\text{ yrs}) + (.35 \times 2.5\text{ yrs}) \\ &\quad + (.1 \times 3.5\text{ yrs}) + (.05 \times 4.5\text{ yrs}) \\ &= 2.05\text{ years}\end{aligned}$$

$$\begin{aligned}\text{Mean survival for teatment B} &= (.05 \times .5\text{ yrs}) + (.15 \times 1.5\text{ yrs}) + (.25 \times 2.5\text{ yrs}) \\ &\quad + (.35 \times 3.5\text{ yrs}) + (.15 \times 4.5\text{ yrs}) \\ &\quad + (.05 \times 5.5\text{ yrs}) \\ &= 3.05\text{ years}\end{aligned}$$

Thus, treatment A is a gamble characterized by an average length of life equal to 2.05 years. Treatment B is a gamble characterized by an average survival of 3.05 years. If length of life is the criterion for choosing, treatment B is a better gamble.

The uncertainty of the length of life after the two treatments may also be represented as shown in Figure 6–2. The circle is a *chance node*, which is a symbol that is used in a decision tree. A chance node is a representation of an event that is under the control of chance. Each line emanating from the chance node corresponds to one of the events that might occur. The expected value at these chance nodes is calculated by the process just described: Each chance node outcome is multiplied by the probability that it will occur, and

FIGURE 6–2
A chance node representation of survival after two treatments.



the products are summed over all outcomes. The place of chance nodes in a decision tree will be discussed in the latter half of this chapter.

A.2 Expected Value Decision Making

The terminology for characterizing gambles is expected value. *Expected value is the result that is expected on the average.* Death could occur earlier with treatment B than with treatment A, although the opposite is more likely. We do not know when any one patient will die, but we do know what to expect on the average: The expected length of life with treatment A is 2.05 years and 3.05 years with treatment B.

We will use *expected value* as our terminology for characterizing choices with uncertain outcomes. In this example, the outcome measure was length of life. The outcome measure could have been dollars spent for care, number of days confined to bed, or a measure of how the patient felt about the quality of his life. These gambles would then have been characterized by their expected cost, expected bed days, or expected utility, respectively.

Consider the following strategy when there are treatment choices with uncertain outcomes: Calculate the expected value of each decision alternative, and then pick the alternative with the highest expected value. This approach is called *expected value decision making*. In this chapter, the value that we will use to characterize treatment decisions is *length of life*. Maximizing expected length of life is a reasonable criterion for choice but is not necessarily the best criterion. Nevertheless, we shall use length of life as a means of helping the reader to achieve the goals of this chapter: *learning how to create a decision tree and compute expected value.*

B. DECISION ANALYSIS: AN INTRODUCTORY EXAMPLE

The expected value decision maker must become proficient in representing decision problems as decision trees. To introduce the steps involved in creating and analyzing a decision tree, we will use a hypothetical problem: Should surgery be performed on a patient with a potentially fatal illness? This decision occurs often in clinical practice. Often, there is little reason to hesitate before operating. However, the decision may be quite difficult when the disease may resolve without surgery, when the operation does not guarantee a cure, and when the operation itself is sometimes fatal.

There are four steps in decision analysis:

1. Create a decision tree: This step is the most difficult because it includes formulating the decision problem, assigning probabilities, and measuring outcomes.
2. Calculate the expected value of each decision alternative.
3. Choose the decision alternative with the highest expected value.
4. Use sensitivity analysis to test the conclusions of the analysis.

B.1 Create the Decision Tree

A decision tree is a method for representing in schematic form all the clinically important outcomes of a decision. Creating a decision tree is an opportunity to clarify one's understanding of the decision problem. The decision tree is a means of communication that may resolve conflict between several decision makers.

The following sequence of steps is used to create a decision tree:

1. Define the decision problem.
2. Identify the decision alternatives.
3. List the possible clinical outcomes of each of the decision alternatives.
4. Represent the sequence of events leading to the clinical outcomes by a series of chance nodes and decision nodes.
5. Choose a time horizon for the problem.
6. Determine the probability of each chance outcome.
7. Assign a value to each clinical outcome.

B.1.1 Define the Decision Problem

The first step in solving any problem is to define the problem clearly. To do so may require writing out a statement of the problem and reviewing each word to be certain that the problem definition is free from ambiguity.

B.1.2 Identify the Decision Alternatives

The second step in creating a decision tree is to understand the decision maker's range of options. The term *decision alternative* denotes one of the choices. The clinician should list each decision alternative. The decision itself is represented by a *decision node.*

> **DEFINITION Decision node:** A point in the decision tree at which several choices are possible.

The symbol for a decision node is a *box.* Each decision alternative is represented by a line attached to the box (see Figure 6–3). In this case, there are two decision alternatives: surgery and medical management. Therefore, there are two lines attached to the box that represents the decision node. The decision node that represents the basic decision problem is usually placed at the left side of the decision tree.

B.1.3 List the Possible Clinical Outcomes of Each of the Decision Alternatives

Having listed the decision alternatives, the next step is to list the final outcomes of each alternative. In this case, the outcomes are given in Table 6.2.

B.1.4 Represent the Sequence of Events Leading to the Outcomes by a Series of Chance Nodes and Decision Nodes

The next step in creating a decision tree is to write down the tree. Regardless of which decision alternative is chosen, the patient's final outcome is determined by a series of chance events. An event whose outcomes are under the control of chance is denoted by a *chance node.*

> **DEFINITION Chance node:** A point in a decision tree at which chance determines which outcome will occur.

FIGURE 6–3
A decision node with two decision alternatives.

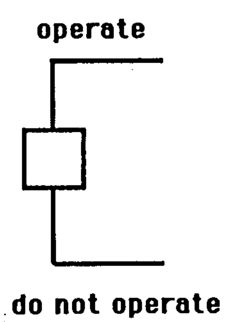


TABLE 6.2
Delineating Clinical Outcomes

Decision Alternative	*Outcome*
Do not operate	Die of disease Survive
Operate	Die of operation Survive operation but die of disease Survive operation and remain alive

The symbol for a chance node is a circle (see Figure 6–4). Each outcome of a chance event is denoted by a line attached to the circle. Each line is labeled with the name of the outcome. Events under the control of chance include the outcome of treatment, the results of a test, or the diagnosis. The decision tree is usually written from left to right, starting with the initial decision node on the extreme left and the final outcomes on the extreme right. The sequence of chance nodes from left to right in the decision tree usually follows the temporal sequence of events. For the decision to "operate," the first event is the findings at the operation. The surgeon may find the disease to be *present* or *absent.*

- *Disease absent:* The patient may die during surgery. If the patient survives the operation, he is cured;
- *Disease present:* If disease is discovered at the time of operation, the surgeon then has a choice: carry out a risky curative operation or do a less risky but noncurative operation. This choice is represented by a decision node on the "disease present" branch of the first chance node (Figure 6–5).

FIGURE 6–4
A chance node at which two outcomes are possible.

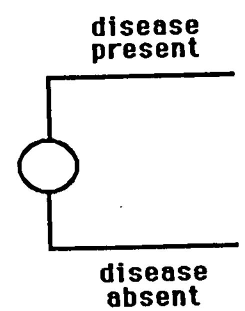
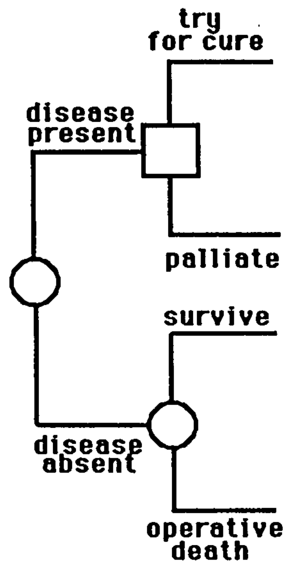


FIGURE 6–5
Outcome of the decision to operate on a patient with a hypothetical disease.



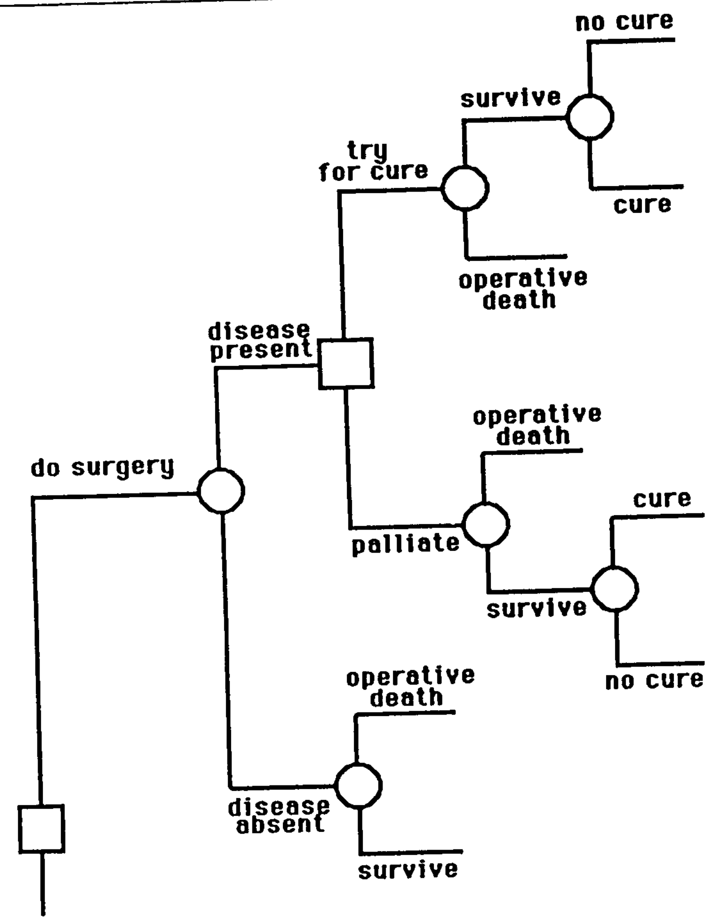
The remaining events are represented as follows: Either surgical procedure could be fatal; if the patient survives the operation, the patient may be cured. These events are represented by a series of chance nodes (Figure 6–6). The completed tree is shown in Figure 6–7.

Although this decision tree shows just two immediate outcomes at each chance node, *the number of immediate outcomes at a chance node is unlimited.* For example, if there were three outcomes of a treatment (cure, palliation, and immediate death from drug toxicity), the chance node would have three lines emanating from it. One rule must be remembered: *The events at a chance node must be mutually exclusive and exhaustive.* Thus, all events must be listed, and there must be no overlap in the definition of these events.

B.1.5 Represent the Time Horizon for the Problem

An essential step in creating a decision tree is to decide how far in the future to describe the outcomes of the decision. Ideally, the tree should represent all events until the patient dies, but such a tree is often unnecessarily complex. The decision maker has several choices:

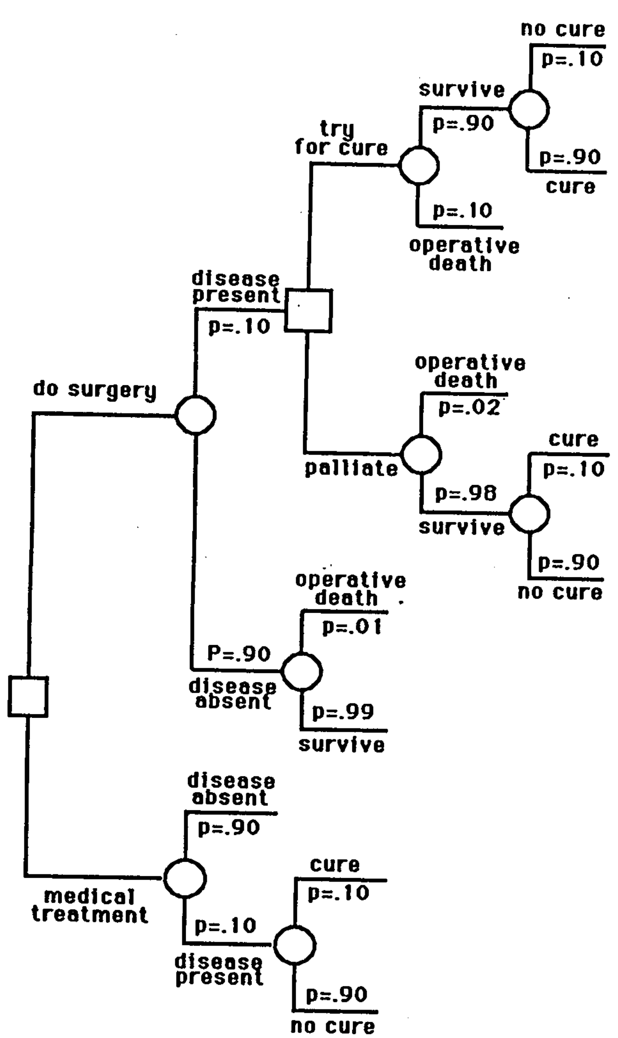
FIGURE 6–6
Outcomes of surgery for a hypothetical disease.



1. Choose a time horizon that is sufficiently short that the tree can include all possible events without becoming overly complicated.

   EXAMPLE: In an acute illness, such as sore throat or suspected appendicitis, the illness has usually resolved within a few weeks.

FIGURE 6–7
The complete decision tree for comparing medical and surgical treatment of a hypothetical disease.



2. Use a long time horizon but remove branches if possible to avoid needless complexity ("pruning the tree").

   EXAMPLE: The patient's lifetime is often the appropriate time horizon, particularly for chronic diseases or acute illnesses with chronic sequellae.

3. Use a decision model that is suited to representing the events in the course of a chronic disease. We will briefly review one such method (the Markov model) in Chapter 7.

In this hypothetical decision problem, we will choose the patient's lifetime as the time horizon.

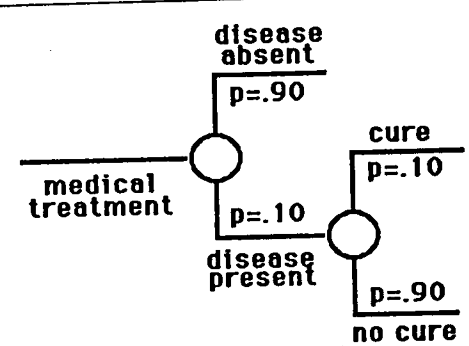
B.1.6 Determine the Probability of Each Chance Outcome

The next step in creating a decision tree is to assign a probability to each event that is controlled by chance. To estimate the probability of the outcomes faced by the patient, we may use published prior experience with similar patients, or we may make a guess based on an intuitive processing of personal experience.

If we do not operate, the only uncertainty is whether the patient has the disease. This information is often found in the medical literature. In this hypothetical example, we shall say that the probability of disease is .10. If the patient has disease, there is a 10% chance of spontaneous cure. The decision tree should be labeled with these two probabilities (Figure 6–8).

If we operate, there is a 10% chance that disease will be discovered. If disease is present, the surgeon may choose a radical procedure that has a 10% operative mortality and a 90% chance of cure. If the surgeon chooses a palliative procedure, the operative mortality is only 2%, and there is a 10%

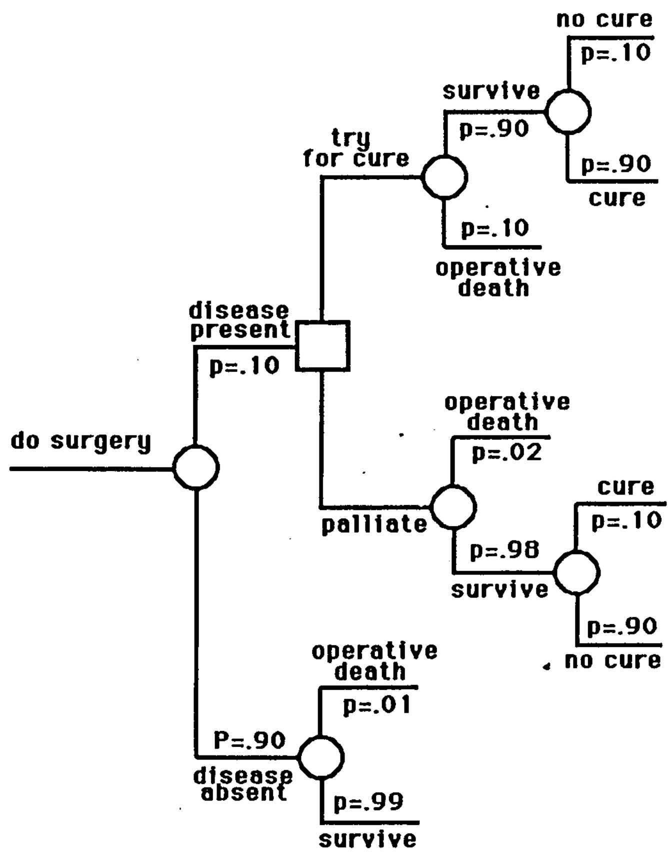
FIGURE 6–8
Probabilities of the outcomes of medical treatment.



chance of spontaneous cure. These probabilities are represented on the tree depicted in Figure 6-9.

In assigning the probabilities to the immediate outcomes at a chance node, the following rule must apply: *The sum of the probabilities at a chance node must be 1.* Thus, checking to be sure that the probabilities at each chance node add up to 1 is an essential part of this step.

FIGURE 6-9
Probabilities of the outcomes of surgical treatment.



Often, the clinician is quite uncertain about the probability of an event but knows the lowest and highest probability that could apply. In addition to a best estimate of the probability, the clinician should list the range of probabilities.

B.1.7 Assign a Value to Each Final Outcome

Assigning a value to each outcome is often the most difficult step in creating a decision tree. This topic will be discussed in the next two chapters. For this example, we shall use average length of life as the measure of the outcome states (see Table 6.3).

B.2 Calculate the Expected Value of Each Decision Alternative

The next step in the analysis is to calculate the expected value of each decision alternative. The analysis done with the best estimates of the probabilities and outcome values is called the *base case analysis.* The expected value of each decision alternative is calculated by a process known as *averaging out and folding back.* This process is easily understood by following an example. The process starts at the "tips of the branches of the tree" and works back to the "root" of the tree.

Start by calculating the expected length of life with medical treatment. Begin at the tips of the tree following the chance node that describes what can happen to a patient who has the disease. The expected length of life with disease is calculated by multiplying the length of life associated with each outcome of the disease by the probability of the outcome (Figure 6-10).

$$\begin{aligned}\text{Expected length of life} &= p[\text{medical cure}] \times \text{normal length of life} \\ &\quad + p[\text{no medical cure}] \times \text{shortened length of life} \\ &= 0.1 \times 20 \text{ years} + 0.9 \times 2 \text{ years} \\ &= 2.0 + 1.8 = 3.8 \text{ years}\end{aligned}$$

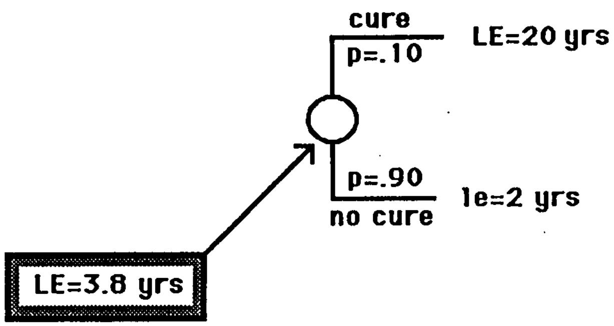
Now examine the chance node that represents the occurrence of disease in medically treated patients (Figure 6-11). The outcomes at this node are "disease absent" and "disease present." The outcome of the "disease absent"

TABLE 6.3
Allotting Values to Outcomes of Illness

Outcome	*Value of Outcome**
Operative death	0 year
Death from progression of the disease	2 years
Cure	20 years†

*Average length of life.
†Normal life expectancy.

FIGURE 6–10
Calculating the expected length of life for medically treated diseased patients.



branch is a life of normal length. The outcome of the "disease present" branch is either cure or no cure. The expected length of life for people who are diseased was just calculated to be 3.8 years.

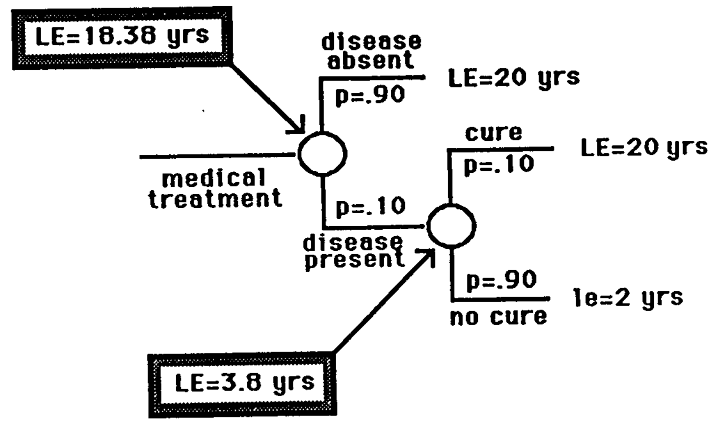
$$\begin{aligned}\text{Expected length of life} &= p[\text{disease}] \times \text{expected length of life with disease} \\ &\quad + p[\text{no disease}] \times \text{normal length of life} \\ &= 0.1 \times 3.8 \text{ years} + 0.9 \times 20 \text{ years} \\ &= .38 + 18.0 = 18.38 \text{ years}\end{aligned}$$

A similar process is followed for the "surgery" branch of the decision tree. This branch of the tree has a decision node within it: When the surgeon discovers that the patient has disease, there is a choice between a radical procedure with a high cure rate but a high operative mortality and a palliative procedure that has a low cure rate and low operative mortality. At each decision node, the surgeon chooses the procedure that offers the highest expected length of life. The process of choosing the branch with the highest expected value and ignoring the other branches is called *folding back* (Figure 6–12).

B.3 Choose the Decision Alternative with the Highest Expected Value

After calculating the expected length of life with each decision alternative, the tree may be represented in simplified form by Figure 6–13. The expected value decision maker will choose the decision alternative with the highest expected value. In this case, the expected length of life with surgery (19.46 years) is higher than with medical treatment (18.38 years). Surgery is the option with the highest expected length of life.

FIGURE 6–11
Calculating the expected length of life with medical treatment.



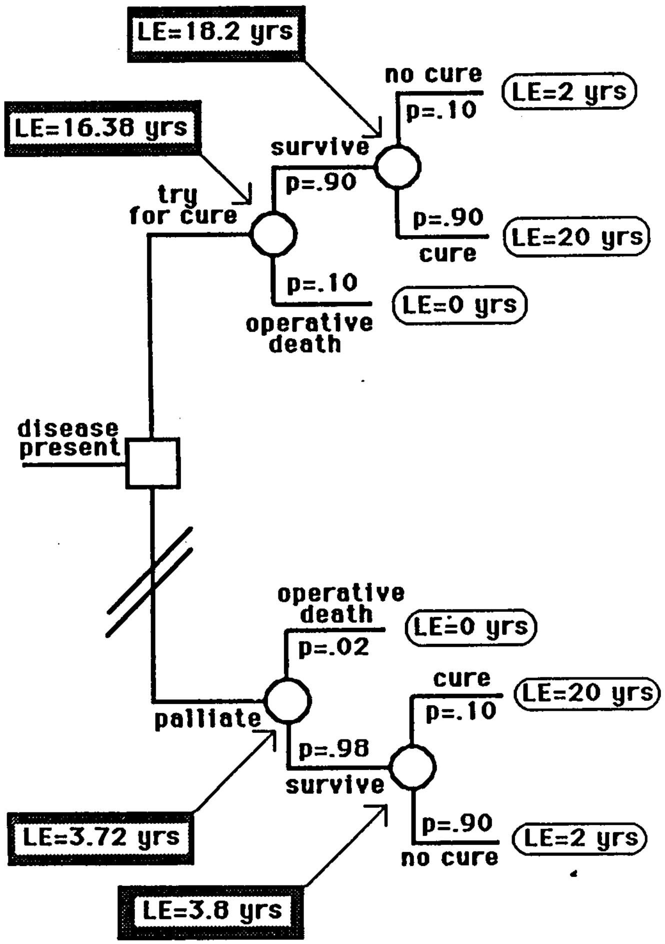
The clinician can identify the most probable outcomes by calculating *path probabilities*. There is one sequence of branches at chance nodes that leads from the root of the tree to the tip of each branch. One obtains the path probability by taking the product of all the probabilities along the path from the root of the tree to the tip of one branch. The expected value of an outcome is the product of the path probability and the value of the outcome. The expected value of the path indicates the contribution of an outcome to the expected value of a decision alternative. The clinician can use the expected value of a path to analyze the importance of the outcome for decision making. These concepts are illustrated in Figure 6–14.

The alert reader may note that the surgical option may lead to radical surgery, which has a 10% operative mortality. Some patients might favor a treatment that offers a larger chance of survival for at least 1 year. Thus, preferences for length of life might be a better outcome measure than length of life itself. The decision alternative that maximizes a measure of the patient's preference for illness outcomes may be the best choice for the patient.

B.4 Perform Sensitivity Analysis

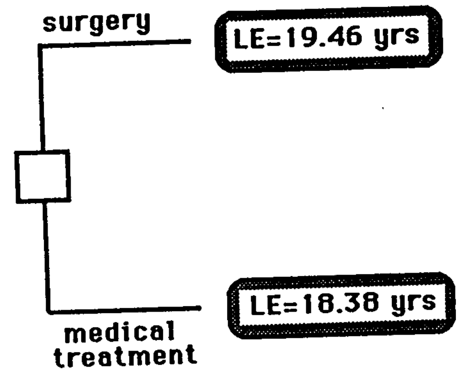
The last step in analyzing a decision tree is *sensitivity analysis*. Sensitivity analysis is a method for testing the validity of the conclusion of a decision analysis. Although the probability at a chance node may be the best estimate

FIGURE 6–12
Calculating the expected length of life in diseased patients treated with surgery.



that is available, there is often a wide range of "reasonable" probabilities that could be used with nearly equal confidence. Sensitivity analysis asks this question: "Do my conclusions change when the probability and outcome estimates are assigned values that lie within a reasonable range?"

FIGURE 6–13
Expected value of the decision alternatives.



DEFINITION **Sensitivity analysis:** A test of the stability of the conclusions of an analysis over a range of probability estimates, value judgments, and structural assumptions.

For this example, we have done several sensitivity analyses to see how the expected values of the decision alternatives are affected by assigning a range of reasonable values to:

- The probability of death during radical surgery—which takes account of the patients whose medical condition makes them a relatively poor operative risk.
- The probability of disease—which indicates how the preferred treatment may change in patients who have relatively few of the typical clinical findings of the disease.

The process in each sensitivity analysis is the same. The expected value of each treatment is calculated several times. Each time, the probability or outcome is assigned a different value within the "reasonable" range. If one treatment has the highest expected value throughout the range of a probability or outcome estimate, the decision is not sensitive to that factor.

B.4.1 Effect of the Operative Mortality from Radical Surgery

The sensitivity analysis for the probability of death during radical surgery indicates that this factor is not an important determinant of the expected outcome (see Table 6.4). Life expectancy with surgery is higher than with medical treatment even for a patient who is a very poor operative risk.

FIGURE 6–14
A path probability.

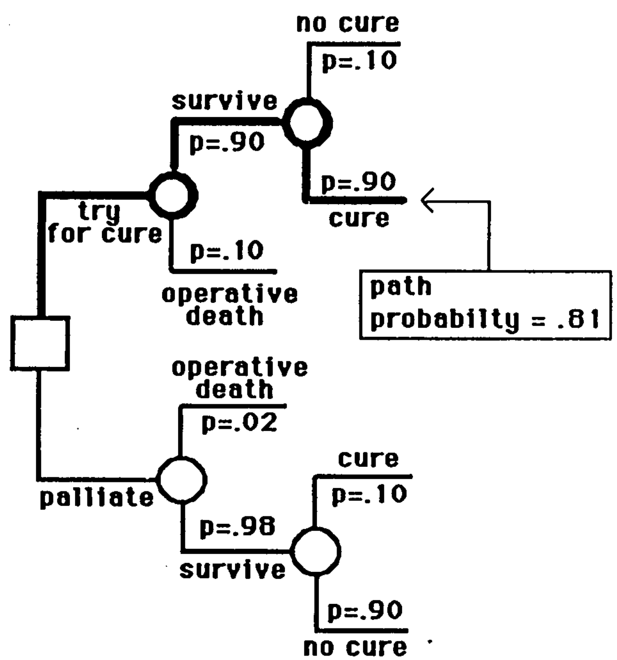


TABLE 6.4
Sensitivity Analysis of the Effect of Operative Mortality

	Life Expectancy	
p (Operative Death)	*Surgery (years)*	*Medical (years)*
0	19.64	18.38
.05	19.55	18.38
.10	19.46	18.38
.15	19.37	18.38
.20	19.28	18.38
.25	19.19	18.38

TABLE 6.5
Sensitivity Analysis of the Effect of the Probability of Disease

	Life Expectancy	
p[Disease]	*Surgery (years)*	*Medical (years)*
0	19.80	20.00
.10	19.46	18.38
.20	19.11	16.76
.30	18.77	15.14
.40	18.43	13.52
.50	18.09	11.90

B.4.2 Effect of the Probability that the Patient has the Disease

Recall that this operation is performed when there is still uncertainty about the true state of the patient. When the probability of disease increases, the advantage of surgery increases (Table 6.5). As the disease becomes more likely, life expectancy for both treatments decreases. However, a medically treated patient's life expectancy decreases much faster than that of a surgery patient.

This analysis shows that surgery is preferred even when the probability of disease is less than .10. In fact, surgery is preferred when the probability of disease is .016 or greater. The probability of disease at which the expected length of life is the same for medical and surgical treatment is called the *treatment threshold probability*. The treatment threshold probability is discussed in Chapter 9.

These sensitivity analyses provide considerable reassurance about the validity of the conclusions of this decision analysis. The base case analysis favored surgery. We tested this conclusion by making several assumptions that were prejudicial against surgery.

SUMMARY

This chapter introduced several important ideas that will dominate our detailed consideration of decision making:

- Representing a decision alternative by its expected value
- Expected value decision making
- The decision tree
- Averaging out and folding back
- Sensitivity analysis

Even a hypothetical example shows how a decision problem that seems difficult at first can be understood once it has been represented by a decision tree. When one decision alternative consistently has the highest expected value after sensitivity analysis has been performed for all variables, the clinician may proceed with confidence. *Even when the decision alternative with the highest expected value has been chosen, an individual patient may suffer a bad outcome. However, each patient will have the best chance at a good outcome if the physician is an expected value decision maker.*

PROBLEM

1. Your patient may have acute Tarkism. If not treated, acute Tarkism is fatal in 20% of patients. An operation always cures acute Tarkism if the patient survives the operation. However, the operation is fatal in 10% of patients.

   There is a test (the AT-test) that is always positive in patients who have acute Tarkism. However, the test is sometimes positive in patients who have symptoms of Tarkism but do not have Tarkism. Therefore, not everyone with a positive test has Tarkism.

   Suppose you have done the AT-test and it is positive. To decide whether to operate, you look up the performance characteristics of the AT-test. At what probability of acute Tarkism in patients with a positive AT-test should you be indifferent between operating and not operating?

NEW WORDS IN THIS CHAPTER

Chance node: A point in a decision tree at which chance determines which outcome will occur.
Decision node: A point in the decision tree at which several choices are possible.
Sensitivity analysis: A test of the stability of the conclusions of an analysis over a range of probability estimates, value judgments, and structural assumptions.

CHAPTER SEVEN

MEASURING THE OUTCOME OF CARE

In Chapter 6, we learned a method for solving a decision problem. The method is called *expected value decision making,* and it has the following basic components:

- Identify the decision alternatives
- Create a decision tree
- Assign probabilities to the chance nodes
- Assign a numerical measure to each illness outcome
- Calculate the expected value of each decision alternative

In preceding chapters, we have learned how to perform all but one of these tasks. In this chapter, we complete our preparation for expected value decision making by learning several ways to assign a numerical measure to an illness outcome. In many respects, this task is the most difficult of all.

DEFINITION Illness outcome: A state of health that results from an illness.

This chapter is divided into five parts. Each part corresponds to a method for characterizing the outcome of an illness. Combining several of these attributes into one summary measure is the subject of the next chapter.

A. Measuring the patient's preferences: utility assessment

   Utility is a quantitative measure of the strength of the patient's preference for an outcome. Expected utility is the preferred criterion for choosing among decision alternatives with uncertain outcomes. The decision alternative with the highest expected utility *should* be preferred, as will be discussed in this chapter.

   Assessing the patient's utility for a health outcome requires the patient's cooperation and understanding. Some patients are unable to participate in utility assessment and require other outcome measures, which are also discussed in this chapter.

B. Estimating the length of life

   Most people are concerned about how long they will live. Therefore, the length of life is an important measure of the outcome of an illness. However, the length of life is an incomplete measure of outcome without taking into account the *quality of life.*

C. Measuring how well the patient is able to function

   Functional status is a measure of how well the patient carries out the activities of living. Functional status is an indirect measure of the