

## **Primer on Medical Decision Analysis:**

### **Part 1 -Getting Started**

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This paper is Part 1 of a five-part series covering practical issues in the performance of decision analysis. The intended audience is individuals who are learning how to perform decision analyses, not just read them. The series assumes familiarity with the basic concepts of decision analysis. It imparts many of the recommendations the authors have learned in teaching a one-semester course in decision analysis to graduate students. Part 1 introduces the topic and covers questions such as choosing an appropriate question, determining the tradeoff between accuracy and simplicity, and deciding on a time frame. *Key words:* decision analysis; expected value; utility; sensitivity analysis; decision trees; probability. (*Med Decis Making* 1997;17:123- 125)

Over the past ten years, our group at the University of Toronto has taught a one-semester course on decision analysis. Unlike educational initiatives that solely focus on readings about and discussions of decision analysis, our course has required that each student perform a decision analysis. This requires building a decision tree on paper, modeling that tree on a computer, obtaining probabilities and utilities by direct measurement or literature review, evaluating the tree, performing sensitivity analyses, presenting the study to peers, and writing up the analysis. Several of the students have gone on to publish their work.<sup>1-8</sup> To pass the course, each student is required to develop a model that "works."

In this series<sup>9-13</sup> and the accompanying note on presentation,<sup>14</sup> we attempt to impart what we, as teachers, have learned about the practical issues of performing decision analysis. Much of what we have

learned has come from teaching our students how to develop a working model.

The intended audience for this series is individuals who are learning how to perform decision analyses. The series assumes that the reader is already familiar with the concepts of decision analysis and has read and comprehended several decision analyses before trying his or her hand at one. Before embarking on this series, the reader should read a two-part users' guide aimed at consumers of decision analyses<sup>15,16</sup> to achieve a grounding in basic concepts such as decision nodes, probability nodes, expected value, tree representation, sensitivity, and threshold analyses, and is also referred to two textbooks.<sup>17,18</sup> The goal of the present series is to give practical suggestions for performing decision analysis.

### **When Is Decision Analysis Appropriate?**

One of the first steps in a decision analysis is to decide whether the technique is appropriate for the given question. Two issues merit special attention. First, there should be some uncertainty about the appropriate clinical strategy for patients with a given health state. There are some circumstances in clinical medicine when published primary clinical evidence already clearly identifies the optimal clinical strategy. For example, patients who have carotid artery stenosis of more than 70% after suffering transient ischemic attacks clearly benefit from carotid endarterectomy, as demonstrated by the NASCET

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Trial.<sup>19</sup> One need not perform a decision analysis of this question. However, most clinical decisions are not as clearly supported by direct evidence.

Sometimes randomized trials have been performed but the optimal decision remains uncertain because several outcomes are involved. The randomized trials of anticoagulation in atrial fibrillation, for example, show a reduction in the risk of stroke but an increased risk of bleeding and, in some trials, a considerable dropout because patients do not wish to be on coumadin for indefinite periods of time. A decision analysis<sup>20</sup> can incorporate these three outcomes as well as possible different levels of risk depending on patient characteristics (e.g., age, other existing cardiac disease). Such a model can help the decision maker understand the risk-benefit tradeoff more explicitly.

The second main issue in assessing the potential value of a decision analysis is to ensure that there is a meaningful tradeoff in the problem. Decision analyses always require comparison of at least two clinical strategies. One strategy ought to contain advantages and countervailing disadvantages. If one strategy clearly dominates the other because it results in lower rates of adverse disease outcomes, less risk of treatment side effects and better utilities, then a decision analysis is not necessary. An example of this situation might be a noninvasive diagnostic technique (such as echocardiography) compared with an equally accurate but more invasive diagnostic technique (such as angiography) for assessing myocardial wall motion or thickness. Of course, decision-analytic models can also be used to estimate the cost-effectiveness of interventions involving dual outcomes such as costs and utilities. In this circumstance, one strategy may clearly be better than the other in the clinical sense but result in higher expected costs. In this series, however, we focus on models that have only one clinical outcome measure.

### Accuracy versus Simplicity

Decision-analytic models must be sufficiently complex to incorporate the important events and values, yet sufficiently simple to be understandable. A decision tree is not a complete representation of “the real world” but rather a simplified and highly stylized model of the most important components. To determine the appropriate level of complexity, the analyst must consider whether the model captures the key issues necessary to fully describe the risk-benefit tradeoff. If a key element of the risk-benefit tradeoff is missing, the model will not achieve its goal of helping the reader understand the tradeoff.

A second major consideration concerns availability of data. In some circumstances, analysts will wish to develop more complex models to better fit the nature of the problem. However, data for such a complex tree, such as a Markov model (described in Part 5 of this series)<sup>13</sup> that uses transition probabilities from one state to another over successive short time periods, are often unavailable. In these circumstances, it may be wise to develop a simpler model. Like others,<sup>21</sup> we have often found that the insights derived from a simple model are similar to those derived from a complex formulation. We encourage beginners to develop the simplest models.

How many strategic alternatives should be included? Some clinical questions require consideration of multiple combinations of several interventions. For example, in suspected pulmonary embolism, a simple model would consider empirically anticoagulating the patient, or performing tests such as a ventilation-perfusion lung scan, venous doppler ultrasonography, impedance plethysmography, and angiography. The various combinations would result in dozens of possible strategies. The best approach for modeling such a circumstance is to limit the number of options to those that are clearly different from each other and cover the spectrum of the problem. Thus, one might consider only three options in the pulmonary embolism example: doing nothing further, anticoagulating without further tests, and performing a ventilation-perfusion lung scan and going on to angiography only if its result is neither clearly positive (high-probability scan) nor clearly negative (normal scan). One can always add other options later on.

### Time Frame

In comparing outcomes of alternative strategies, the analyst must determine an appropriate time frame. When considering alternative strategies for preventing or treating chronic illnesses, the time frame should be long. When considering therapeutic strategies to avoid short-term complications (e.g., perioperative management issues), the time frame can be brief. Thus, the time frame will depend upon the nature of the clinical problem.

In choosing an appropriate time frame, one confronts the same tradeoff as noted in the above section on accuracy versus simplicity, namely desire for completeness versus availability of data. Most clinical studies including randomized trials have relatively limited time frames. Even for trials involving chronic diseases such as coronary artery disease, the period of observation is usually between one and three years and rarely as long as five years. Occasionally one gets reports of long-term follow-up, but

the observation periods in these reports rarely exceed ten years.<sup>22</sup> Some cohort studies, such as the Framingham Study, have much longer periods of observation. One, therefore, must balance the desire for complete long-term follow-up with the availability of valid and precise data. Most analysts will have to extend the period of observation of a randomized trial for their analysis and therefore will have to extrapolate the data. The performance of extensive sensitivity analyses around these extrapolations is important.

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## Glossary

**Markov Model:** A decision-analytic model that involves a Markov process, a modeling technique derived from matrix algebra, which describes the transitions a cohort of patients make among a number of health states during a series of short intervals or cycles.