The Need for Evolution in Healthcare Decision Modeling

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STATEMENT OF PROBLEM. Many healthcare decisions are difficult because they are complex and have important consequences such as the impact on survival or quality-of-life of individuals and on allocation of limited resources. The present state-of-the-art in healthcare decision modeling is often inadequate to properly assess these decisions.

METHODS. Based on a literature search and the experience of the authors, typical methodologies used in healthcare decision analysis modeling are explored and compared with methods used in other practices. An example of hormonal therapy decisions is used.

RESULTS. Useful methods that have been developed in other fields are presented. These include methods targeted toward appropriate assessment and representation of the complexity of decisions, assessment of uncertainty, use of nonexpected value decision analysis, and use of multi-attribute decision criteria.

CONCLUSION. The state-of-the-art in healthcare decision modeling can be improved through learning from other practices.

Key words: Decision analysis; uncertainty analysis; utility analysis; hormonal therapy; women’s health. (Med Care 2003;41:1024–1033)

Statement of Problem

Decisions are difficult when they involve a high degree of complexity, large uncertainty, tradeoffs across multiple objectives, and/or perspectives of different stakeholders. Many healthcare decisions are difficult because they are complex and have important consequences such as impact on quality-of-life of individuals and on allocation of limited resources.

An example is “What hormonal therapies should women take?” As indicated in Figure 1, this decision has several time-dependent aspects (eg, age of woman, duration of therapy). Each type of therapy, both singly and in combination, has benefits, risks (of side effects), and costs. Great uncertainty is associated with many variables, and large variation exists within (eg, over time) and between individual women. Stakeholders with potentially different perspectives and preferences include the women themselves, women’s health organizations, physicians, pharmaceutical firms, insurance companies, and healthcare systems. Key objectives of this decision could include maximizing health benefit, minimizing health risks, maximizing patient choice, maximizing physician involvement, and so on, while satisfying resource and other constraints and being fair and equitable. The alternatives could include single therapies at particular times for particular types of women, numerous combinations of therapies, or doing nothing at all. The question is particularly important to postmenopausal women, who might have past exposures and multiple concurrent exposures that could alter their risk/benefit profiles for many diseases. The question is also important in

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publicly funded healthcare systems, in which population-based therapeutic decisions are routinely made, and with regard to clinical practice guidelines and formulary funding decisions.

Questions concerning this decision appear in the media everyday. How should a woman, a physician, or a healthcare system go about systematically evaluating this decision? The term “evidence-based” decision making is often promoted, but evidence is of limited use without a framework for systematically and rigorously incorporating it into decision making. The most rigorous existing means of facilitating such decisions, in terms of evaluating evidence and tradeoffs across risks, benefits, and costs associated with healthcare strategies, are provided by multiattribute utility theory (MAUT) and decision analytic techniques. Decision models are often useful to assess complex and difficult decisions under a state of uncertainty.

However, we fear that the current state-of-the-art in healthcare decision analysis is inadequate to facilitate many health decisions. Disappointingly little work on model quality and reliability has been published in the healthcare literature compared with other fields. This state-of-the-art appears to be largely associated with economic evaluations, which address only a subset of healthcare issues. Based on our experience and searches of the healthcare literature (using PubMed), few analyses have yet applied a wide range of appro-

![Diagram](image)

**Fig. 1.** Representative hormonal therapies, relevant temporal variables, and potential disease/condition outcomes (both positive and negative) associated with therapies.
ppropriate and available modeling techniques that are routinely used in other fields such as engineering, environmental science, and operations management. The reasons for this are unclear, but we argue that the healthcare decision analysis practice can learn from these other practices.

The analytic concepts presented in this article have been previously published in the nonhealthcare literature and in some cases suggested but not widely implemented in the healthcare literature. Areas of potential evolution include appropriate representation of the complexity of decisions; assessment of uncertainty and variability; use of nonexpected value decision analysis; and use of multi-attribute decision criteria.

Each of these areas is briefly discussed in relation to the hormonal therapy decision example when applicable. The intent of the present discussion is not to conduct an analysis, but rather to provide researchers and analysts with research suggestions and pragmatic guidance. In any case, people make decisions; decision modeling only informs and facilitates decisions.

Areas of Potential Evolution

Appropriate Representation of Decision Complexity

Healthcare decision analysis sometimes suffers from lack of appropriate consideration of decision complexity. The hormonal therapy issue is potentially an extremely complex decision, like many other healthcare decisions. A literature search using the search terms “decision analysis” plus the individual therapies indicated in Figure 1 revealed dozens of published papers on narrowly defined questions, none of which, in our opinion, are useful for informing the pragmatic question “what hormonal therapies should women take?” We think a holistic yet individualized approach to this question is possible.

Excessive simplification of a complex decision is not informative. For example, practicing healthcare professionals have noted that a major limitation of many published decision analyses is their focus on narrowly defined populations.7 Physicians often realize that biologic complexity/interactions and population heterogeneity make individualized decisions as well as useful generalizations difficult.

The complaint “we don’t have enough data to model this decision” has sometimes been proposed as a reason to simplify models.8–10 Yet, as Stinnett and Paltiel11 state, “the use of informed but imperfect estimates is preferable to the alternatives of either making uninformed decisions or relying on assumptions that lack face validity.” Properly structured models can clarify which areas would benefit most from further research. Matching the complexity of decision analysis models to the true scope of the decision at hand, based on the inputs that can be controlled and the outcomes of interest that they affect, can avoid creating needlessly simple analyses for complex problems or vice versa.

Phillips12 uses the term “requisite models” to refer to a model that contains everything essential for solving the issue at hand. Questions that we might ask include: 1) Who are the (legitimate) stakeholders? 2) Who bears the consequences of the decision? 3) Who is responsible for making/implementing/enforcing the decision? 4) What do the stakeholders care about? 5) What are their preferences for different outcomes? 6) What tradeoffs are they willing to make among different consequence dimensions (eg, cost vs. safety)? 7) What are the competing decision options to be evaluated? 8) What information do the stakeholders need to make well-informed decisions? What questions are they asking? 9) What information is immediately available about the probable consequences of different decisions? What data gaps and uncertainties exist and what means exist to reduce uncertainties? 10) What analytic tools and experts are available? 11) What are the resource and time constraints on making the decision?

An iterative approach is often useful, ie, conducting a simple analysis to help focus the question and identify initial areas of uncertainty, then conducting a more complex analysis as required.13,14 A pragmatic way to elicit the important aspects of a decision from stakeholders and experts and to structure complex decisions is to use influence diagrams to represent “mental models” of risk and decision processes.15 These are graphic representations of the dependency relations among variables accompanied by processes useful for communication, computation, or both. Influence diagrams are more efficient for this purpose than decision trees because they can be both hierarchical (representing the decision in varying levels of complexity) and “modular” (allowing the choice of different techniques or components depending on the complexity of the decision). Although use of influence diagrams has been sug-
gested for healthcare issues, there are limited examples of practical applications in the literature.

Figure 2 presents a simplified influence diagram for the hormonal therapy decision, which can be structured to identify preferred choices. The decision is represented by a rectangle, sources of uncertainty as ovals, and outcomes as diamonds. The choice of hormonal therapy strategy will influence health benefits and risks, and could influence other stakeholder values as well as economic costs. The "final" outcome of the decision is evaluated by a multi-attribute utility function, which represents the tradeoffs across health benefits, health risks, costs, and other stakeholder values.

In a more complete computational model, the intermediate outcomes (risks, benefits, other values, costs) would be represented in much more detail. There are several hierarchical, modular commercial computer modeling programs available and spreadsheets can be used.

In practice, healthcare decision analyses often seem to be driven by physician expertise and opinion, which might be appropriate in the clinical setting but which might not be appropriate in a multi-objective, multistakeholder issue. Use of preliminary scoping exercises and hierarchical modeling could facilitate appropriate representation of complex decisions. Characterizing healthcare decision making in these scenarios is a fertile area of research.

Presentation of the results of a complex model in a simple, understandable fashion is necessary if decision-makers with limited time and expertise are to actually consider the results of the analysis in their decision process. Use of clear tables and graphic representations (eg, as provided by Clemen) as opposed to exhaustive text discussions is desirable.

Assessment of Uncertainty

Uncertainty assessment quantifies what we do and do not know. Morgan and Henrion provide the following situations (among others) in which one should be concerned about proper and systematic assessment of uncertainty:

- When risk attitude is important (eg, rapidly increasing aversion to increasing levels of risk exists).
- When uncertain information from different sources must be combined.
- When a decision must be made regarding whether additional resources must be expended to reduce uncertainty.
All these considerations are important in healthcare decision analysis. However, it is quite rare to find risk aversion even addressed in published decision analyses, much less discussed in terms of importance to uncertainty assessment.18–20 Information from disparate sources, such as randomized trials, observational studies, surveys, and expert opinion, is routinely aggregated and incorporated into such analyses; yet formalized means of combining information (eg, Bayesian methods) and capturing uncertainty in appropriate statistical distributions are rarely used.6,21–25 Despite much available guidance, thorough uncertainty assessment is rarely practiced in published healthcare decision analyses. The argument “we don’t have enough data to model this decision” is not constructive; decisions will be made regardless in the face of uncertainty, and the opportunity loss for not evaluating uncertainty can be high.14

There are many sources of uncertainty in the hormonal therapy example. For example, women who use oral contraceptives at one time period in life and estrogen replacement therapy at a later time period of life could have a different endometrial cancer risk than women with just one of these exposures.26 The modeler ideally should address the uncertainty associated with these combined therapies (at different time points in a woman’s life) on cancer risk by defining statistical distributions and functions that address both positive and negative interactions. Bias can result when expected values or means are used to arrive at conclusions regarding the utility of an alternative (discussed later in this article), and these values are not estimated appropriately. Correct calculation of expected values is contingent on correct and “honest” representation of parameter uncertainty.14

There are 2 basic ways to determine the impact of uncertainty on a decision. One is by means of sensitivity analysis in which variable values are varied across a range and the impact on modeling results examined. Alternatively, sensitivity can be estimated by many different statistical methods, including rank correlation, contribution to variance, or regression techniques.14,27,28 The second way is by means of value-of-information analysis, which is a powerful method to take sensitivity analysis a step further by directly examining the impact of uncertainty reduction on the choice of an alternative.29,30

In the majority of published healthcare decision analyses and texts, uncertainty and sensitivity assessments usually involve either varying a single, deterministic input or use of Monte Carlo simulation (a random sampling technique). Detailed methods of determining sensitivity from Monte Carlo simulations are not usually discussed in the healthcare literature (eg, Hunink et al.31). We did not find any instances in the healthcare literature in which analytic or multiple simultaneous statistical methods were used to estimate sensitivity, although these are routinely used in other fields and have been found to be informative.14,27,28 Additionally, there are important technical aspects of Monte Carlo simulation that appear to be routinely ignored. These include estimation and presentation of the appropriate number of trials to reduce sampling error,32 as well as use of variance-reduction, importance sampling, and sampling strategies such as Latin Hypercube simulation, which provide more accurate sampling of the tails of distributions.28,33–35 The number of sampling trials, as well as use of sampling strategies such as Latin Hypercube simulation, become important when rare events are being assessed. In the hormonal therapy example, many diseases such as breast cancer have small probabilities but potentially large consequences. If the tails of distributions are not sampled properly, estimations of the expected value of variables might not be accurate, thus directly biasing estimations of expected values in decision calculations. A literature search (using the search term “Latin Hypercube”) revealed no instances in which Latin Hypercube simulation has been used in healthcare modeling, although it has been used in disease transmission modeling (eg, Seaholm et al.35) and is available in many software tools.

Value-of-information analysis6,25,36,37 compares preferred alternatives under a state of uncertainty with preferred alternatives given a state with more or better information. The value of conducting tests, or, more generally, reduction of uncertainty through research, is estimated by comparing the expected value of preferred alternatives in the 2 states. This process takes uncertainty assessment a step further by directly assessing the impact of uncertainty on choice of alternatives. Value-of-information analysis in healthcare decision analysis seems relegated to evaluations of clinical tests (for an example, see reference 31), although there is no reason that this powerful technique could not be applied to any particular uncertain variable.
Variability is distinguished from uncertainty. Variability represents the true heterogeneity across individuals in a population, or in an individual over time or over changes in health status. Variability, like in statistical analysis, is typically addressed in decision analyses either by stratifying the analysis or by defining distributions that represent heterogeneity. Many published healthcare decision analyses do not distinguish between uncertainty and variability, and thus, at the least, are quite confusing. Modern simulation software allows simultaneous modeling of uncertainty and variability.

Uncertainty and variability in healthcare decision problems can be addressed by 1) identifying a population of interest; 2) characterizing uncertainty and variability associated with variables and the model itself for the population; and 3) appropriate modeling of uncertainty and variability and their impact on decisions. Uncertainty assessment has been developed to a high degree in fields such as engineering and environmental safety because of the potentially large consequences of highly uncertain events in those fields. Proper use of Monte Carlo simulation and its variants provides efficient means to implement analyses, although in some cases, analytic solutions exist. Statistical means of conducting sensitivity analysis can be easily programmed into spreadsheets and applied to the output of simulations. There is debate with regard to the value of assessing population variability in preferences as opposed to using summary measures for a population. When possible, sensitivity to differences in stakeholder preferences should be assessed.

Much of the information used in healthcare decision analysis originates from epidemiologic studies. Little work has been performed on appropriate means of translating epidemiologic data into information suitable for inclusion into decision analyses, including the full range of uncertainty associated with these data. Some epidemiologic study designs are quite limited with respect to identifying and disaggregating specific sources of uncertainty; thus, subjective judgment might have to be used in lieu of information from such studies.

**Non-Expected Value Decision Analysis**

Utility functions quantify stakeholders’ values, risk attitudes, and preferences for outcomes. Most utility-based decision theory relies on the concept of the expected value or average probability-weighted utility for a particular choice. Under axioms of expected utility theory, the alternative with the greatest overall expected utility is the preferred choice. Expected utility decision analysis “averages out” probability-weighted utilities of consequences across alternatives and is a convenient way to evaluate decisions that involve alternatives with similar scales of risk and consequences. This is a rigorous and powerful technique, but one that assumes decision-makers have well-defined preferences and reasonable knowledge of the probabilities of different outcomes, which might be unlikely in many healthcare scenarios.

Suppose one is faced with 2 alternatives having a single consequence of interest (eg, quality-adjusted life-years [QALYs]), one with a large probability of a relatively minor consequence, and the other with a small probability of a large consequence. In expected-value decision calculations without utility functions, it is possible that these 2 alternatives would have the same expected value for the consequence, and thus a risk-neutral rational decision-maker (ie, utilities proportional to a scalar consequence) would be indifferent between them. This is obviously not the way that healthcare decision-makers make decisions; in the hormonal therapy example, a small increase in the probability of giving a patient life-threatening cancer is not likely to be viewed the same as a large probability of not preventing hot flushes. Thus, proper use of utility functions and methods that are not constrained to typical estimation of expected values might be warranted.

A modification of expected-value decision analysis called the partitioned multi-objective risk method can address this problem by estimating conditional expected-value functions, which represent risks given that consequences fall within specified ranges of probability. This method is useful for evaluating rare events with large consequences and represents a potentially more appropriate means of informing healthcare decisions; nonetheless, these methods do not appear to have been applied in the literature. Methods of nonexpected value decision making can allow decision-makers faced with low-probability, high-consequence events such as breast cancer to concentrate the analysis on evaluation of the impact of these events on a decision. Use of expected...
value decision making in such situations will tend to “dilute” the impact of these events.

Multi-Attribute Decision Criteria

Cost-effectiveness is a common decision criterion in healthcare decision analyses.31 “Effectiveness” generally refers to changes in health status and could be measured by disease avoidance, survival, or quality-adjusted survival as in the QALY. Resource tradeoffs are introduced with the inclusion of healthcare costs in either private or publicly funded healthcare systems; thus, there is a strong interest from payers’ perspectives in technical (intraprogram) and allocative (interprogram) efficiency.41 Incremental cost-effectiveness or cost-utility ratios have been routinely used as decision criteria in health care; however, reliance on cost-effectiveness or cost-utility as decision criteria is highly limiting outside of narrowly defined questions relating to technical (as opposed to allocative) efficiency.42,43

Net present value or other measures of benefit-cost comparisons have been proposed as more appropriate decision criteria,42,44,45 at least in terms of allocative efficiency. These criteria are routinely used in health services and public health decisions34,46–51; the specific reasons that these have not been used in health care are unclear. However, the issue of distributional equity is not addressed sufficiently by either cost-effectiveness or benefit-cost analysis in their traditional forms as applied in any field; this is an active area of inquiry.52

Individual women and many providers in publicly funded systems might not be concerned primarily with costs of disease or treatments; therefore, there is no particular reason that analyses from these perspectives should emphasize cost in decision criteria (the ethics of this stakeholder view are not discussed here). In the case of hormonal treatments, women might also be concerned with important outcomes that are not directly disease-related such as sexual function.53 Furthermore, important attributes from a healthcare system perspective other than patient health such as physician satisfaction are rarely included. It is unclear if or how the majority of healthcare decision analyses incorporate risk aversion, which is likely quite important in many healthcare decisions.54

A general, rigorous decision approach is MAUT, in which preferences and risk attitudes are expressed as utility functions of multiple consequence dimensions or “attributes.”4,55 MAUT has been used to a limited extent in health care, eg, to aggregate an “effectiveness” attribute from various “subattributes” relating to patient quality-of-life.56,57 However, full use of MAUT in choosing strategies is underused in health care, although it has been applied in health services38,39 and a notable exception in the work of Pellissier and colleagues.19,60 Furthermore, integration of MAUT into healthcare portfolio-based planning and resource allocation is notably absent in the literature.

Definition of multi-attribute functions in most software is straightforward, as is definition of risk aversion functions; therefore, evaluation of multiple attributes is not constrained by available software.

Pellissier and Venta60 posit that MAUT has not been used more in health care as a result of the lack of common use of preference assessment methods that incorporate multiple stakeholder viewpoints and the complexity of many healthcare decisions. They propose, along with others,19 a method of eliciting utilities, continuous-risk utility assessment that addresses this issue.

Another approach for addressing multiple attributes is multicriteria decision making (MCDM), in which “inferior” alternatives are eliminated in a systematic fashion rather than attempting to order all alternatives.61 MCDM methods allow for incomplete, ambiguous, and uncertain preferences. They recognize that, although some pairs of alternatives might not be comparable, it might be unnecessary to explicitly compare them to identify the preferred alternative. One important class of MCDM methods first scores or ranks each alternative in the set of alternatives according to each of several criteria, yielding a multivariate payoff table. Dominant alternatives are then identified.61 Other MCDM methods emphasize iterative interaction between a decision-maker and a software program (for an example, see reference 62). A popular MCDM method is the Analytic Hierarchy Process (AHP). It asks users to estimate ratios of importance weights for pairs of criteria or attributes, and then estimates a final set of weights that best represent these multiple, potentially inconsistent, pairwise ratio estimates. Despite theoretical and conceptual difficulties with AHP,63 the method is popular because of its simplicity.

In terms of formal MCDM methods, one of the few examples is Yu64 in which a computer-guided iterative optimization approach to MCDM in radia-
tion oncology was used, and competing treatment plans were ranked based on the decision-maker’s preference tradeoffs among several dimensions.

Primary research into true multi-attribute decision making in health care is lacking. Like with nonexpected value decision making, potential studies could compare analyses conducted with multi-attribute methods with “traditional” methods, and the relevance to decision-makers could be assessed using elicitation and survey techniques. The issue of distributional equity is particularly of interest.65–68 The use of MCDM methods in healthcare decision making could be fruitful, because many risk-cost-benefit comparisons involve multiple criteria with difficult value tradeoffs and conflicting, incomplete, or uncertain preferences that might not be easily addressed in the MAUT framework.

Discussion

Approaches to healthcare decision analysis might be improved by adopting methods from decision analysis and decision facilitation as practiced in other fields. Hormonal therapy decisions are among the many complex healthcare decisions that could benefit from such an approach.

A recent, typical example of healthcare decision analysis addressing hormonal therapies was conducted by Grann et al.69 This study examined the breast and/or ovarian cancer risk reduction provided by tamoxifen, raloxifene, or oral contraceptives, plus prophylactic mastectomy and surveillance. This study, like many similar studies, did not examine the potential effects of multiple combined therapies and did not evaluate the risk of a complete suite of serious side effects of these therapies (thus oversimplifying a complex analysis). No details were provided regarding uncertainty analysis or sensitivity analysis, and there was no value of BRCA1/2 testing evaluated. Many of the risks evaluated are low-probability, high-consequence events (such as breast cancer), yet risk aversion was not discussed. Tradeoffs across multiple objectives of hormonal therapy were not discussed. This study is simply an example; other similar studies have examined different aspects of this decision, yet all suffer from incomplete ascertainment of the question “what hormonal therapies should women take?” When the entire scope of decisions addressed in the healthcare decision analysis literature is considered, lack of systematic approaches and methodologic rigor becomes a serious issue.

All of the suggested improvements can be easily implemented using existing software tools (although all of these methods are not yet available in a single product). Suggestions regarding research studies are provided to assist in evaluating the reliability and appropriateness of the improved approaches over current approaches.

One problem with implementation of such an approach is lack of formal or practical training in modeling techniques on the part of many physicians and health researchers, yet powerful computer hardware and software tools exist for conducting decision analysis. We suggest that the level of modeling sophistication on the part of healthcare decision analysts should increase to a level commensurate with other fields, and the ability to communicate analytic techniques and results must correspondingly improve. The literature and methods from fields such as operations research and engineering have much to offer.

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