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Modeling for Policy Decisions: Potential and Problems

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Because decision modeling involves the construction of an explicit, mathematically describable structure of the pertinent elements of a clinical problem, the relative effectiveness of alternative approaches to care can be identified; costly procedures become apparent; new technologies can be assessed in relation to the old in terms of effectiveness and costs; the marginal benefit to be achieved by duplicative or alternative practices can be determined. All of this can be accomplished in terms of patient outcome and without the bias and self-interest of which the profession has been accused. Furthermore, if a resource allocation or reimbursement decision made in the name of cost containment eliminates or limits access to effective diagnostic or therapeutic technologies, the impact of that decision on effective care can be explicitly and quantitatively expressed through decision modeling. When such analyses are based on patient outcome, they are difficult to ignore and provide a pivotal point for discussions and eventual compromise.

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Role of Quantitation in Quality Care

> Medicine is the art of understanding diseases and of curing or relieving them when possible. Under this acceptation our sciences would, at least, be exonerated from reproach and would stand on a basis capable of supporting a reasonable and durable system for the amelioration of human maladies.
>
> *Dr. Jacob Bigelow (1) 1852*

In paraphrase of what Bigelow (1) stated 137 years ago, medical practices and technology utilization presented in terms of patient outcome and with quantitative demonstrations of effectiveness would provide the structure for a reasonable and durable system of health care.

It is the thesis underlying this presentation that quantitative analyses of those practices most likely to achieve optimal patient outcome and application of the findings to patient care are basic to "quality" care. Quantitation is the key word. Although there is a voluminous number of published reports on quality care, what it comprises and how to assess it, and physicians have always understood it to be synonymous with effective and efficient care, without structure, it is difficult to reach agreement on its definition, content, delivery mechanisms and criteria for assessment. When quality care is presented as a quantitative model based on effectiveness, however, a basis is provided for highlighting and discussing areas of agreement or disagreement without ambiguity and bias and, thus, for laying a foundation for rational decision making and resource allocations by physicians, nonmedical health care personnel and policymakers alike.

Benefits of assessing care in relation to effectiveness (patient outcome). The advantages accruing from assuming preeminence for effectiveness, quantitatively described in terms of patient outcome, as the most important descriptor of quality care are: optimal outcome is that which the public desires and considers to be quality care, and a common language is created that professionals, the public and policymakers can understand; quantitative data on the options and choices that were considered in the decision-making processes relative to their care help patients understand their care and its costs and provide a basis for their choices; quality care can be best defended when management alternatives are quantitatively related to outcome because the issues become clear and accusations of self-interest or other secondary gain are nullified; adequacy of diagnosis, physician skill and discrimination in obtaining appropriate and complete information, soundness in evaluating data, choice of specific therapeutic regimens with due regard to risks, patient understanding and adequate surveillance can be related to optimal patient outcome. Most importantly, the need for specific technologies in order that quality care can

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JACC Vol. 14, No. 3
September 1989:7A–11A

be provided is best understood when discussion is oriented toward patient outcome and best defended when the quantitative impact of the technology on patient morbidity or mortality can be identified.

An illustration from history. An illustration serving to highlight the benefits to be derived from assessing care on the basis of effectiveness may be useful at this point. The illustration that follows is frivolous, but was chosen because it is unlikely to provoke any medical biases that might distract from the message.

There is a quotation from L.C. MacKinney (2).

> Once upon a time a king, while shaving, fell unconscious in his bedroom. The following treatment was employed by the royal physicians. A pint of blood was extracted from his right arm: then 8 ounces from the left shoulder: next an emetic, 2 physics and an enema consisting of 15 substances. Then his head was shaved and a blister raised on the scalp. To purge the brain, a sneezing powder was given, then cowslip powder to strengthen it. Meanwhile, more medics, soothing drinks, and more bleeding. Also, a plaster of pitch and pigeon dung applied to the royal feet. Not to leave anything undone, the following substances were taken internally: melon seeds, manna, slippery elm, black cherry water, extracts of lily of the valley, peony, lavender. Pearls dissolved in vinegar, gentian root, nutmeg and finally, forty drops of extract of human skull. As a last resort, bezoar stone was employed.
>
> But the royal patient died.

One would presume that this was quality care for the time in which it was delivered. After all, the patient was the king. Or, was it desperation that drove the royal physicians? Or, were they making more profit because of charging for each intervention; or, was the threat of being beheaded motivating them? Was their care influenced by the need to impress the royal family members? Was there minimal redundancy in the therapeutic procedures or was technology utilization in excess? Did the royal physicians fully exploit the medical knowledge and technologies available at the time? Did they overexploit them? Should they have asked for consultation from specialists?

Limitations of structural and procedural standards of quality care. Given the problem of whether King Charles II of England received quality care in 1785, only 200 some years ago, several approaches might be taken. One group of analysts, the structuralists, would look at the education and training of the royal physicians and whether or not they were certified by the proper governing boards. They also would assess whether the facilities were adequate and met standards for the management of the problem of fainting while shaving. Presuming that the physicians and facilities passed inspection, it might be said that quality care had been provided, the presumption being that proper structure assures quality care.

Advocates of the process of care as an indication of quality care would check to see if guidelines or norms were available for the management of conditions similar to the king's, and if not, would ask a panel of experts to formulate such criteria. Equating quality care with normative behavior is established by tradition. All that is required is that the physician provide what is believed by the "experts" to be quality care. This approach has led to the classic definition:

> Good medical care is the kind of medicine practiced and taught by the recognized leaders of the medical profession at a given time or period of social, cultural and professional development in a community or population group (3).

It is improbable, however, that such structure or process standards without demonstration of a direct relation between them and patient outcome could serve to establish whether the king received quality care. Nor is the current need for explicit and quantitative data on what constitutes quality care well served by structure and process standards. They become just another form whereby the profession says, "We say so. Therefore it is." The public, legislators, regulators, third party payers and managers are no longer receptive to what they consider to be "professional elitism." A more direct and adaptive approach to the provision and assessment of quality care would seem to be to define quality care as effective care, with effectiveness referring to the change in patients' current and future health status attributable to that health care (3).

Back to King Charles II. As ludicrous as the example may seem, it is almost certain that the royal physicians were acting under the belief that the therapies they were using held some hope for benefiting the king, and it is doubtful that all of the therapies applied to the unfortunate king were selected at random. Surely, the physicians had used the remedies in previous cases with manifestations of disease similar to the king's, and probably the physicians, if asked, would have had an "option" of the percent of times the therapies had been efficacious (or detrimental).

Advantages of decision models based on probability of increased chance of survival. Suppose, for the sake of illustration, that the royal physicians could have structured a decision model and assigned a quantitative value to each intervention as to its probability for increasing the king's chance for survival. Presuming that each intervention would have been judged to have a positive value, the following advantages would have resulted from such an approach: it could have been demonstrated that care had been provided that was directed at optimizing patient outcome and could be so defended; the family would have understood the reasons for the various therapies and would have accepted that the king received quality care even though he died; the royal treasurer would have understood that bezoar stones and

extracts of human skull, although expensive, were essential for quality care because they had significantly increased the king's chances for survival; if bezoar stones, extracts of human skull or dissolved pearls were denied to the peasants by the Royal Health Service because of cost, a good case could be made by their physician advocates that quality care was being infringed upon. On the other hand, if bezoar stones accounted for the greatest percent cost of the total care, but contributed only a 1% additional probability for survival over the other therapies, singly or in combination, the policymakers in the king's court would have data on which to structure their discussions and eventual decisions on the provision of bezoar stones for general population use on whether or not quality care would be significantly affected by the denial of bezoar therapy. Certainly, other physicians reading about the king's care would have the benefit of having what the royal physicians thought was optimal care and their own thinking would have been influenced.

Although the effectiveness of care approach is based on process of care criteria in that the selection of management alternatives and their execution are processes, the processes are more directly outcome-oriented than are process of care "standards" that are disease-oriented rather than patient-oriented. The "best" care is care that gives the greatest probability, in quantitative terms, of achieving the desired patient outcome, the values of which may be as diverse as mortality, morbidity, patient satisfaction, rehabilitation or cost, among others. The validity of the model would, of course, need to be assessed for accuracy through comparisons with outcome data.

The strongest argument for outcome-based criteria, over and above the fact that improved health (and patient satisfaction) are the goals of health care, is that the definition of quality care as effective (and efficient) care will "work." Because it allows an unbiased quantitative explanation of why it is physicians do what they do, it will have a profound effect on "how doctors behave, on relations within the profession . . ." (4), and through improving relation among the profession, government, third party payers and managers on reimbursement and resource allocation decisions.

A Current Management Problem: Early Acute Myocardial Infarction

Moving rapidly forward in time to our present decade, it is not difficult to identify high cost clinical practices, particularly in cardiology, that should be subjected to decision modeling not only in defense of quality care, but to provide guidance as to the cost-effectiveness of alternative management strategies. One that comes immediately to mind is the management of the patient early in the course of acute myocardial infarction.

There is increasing evidence that thrombolysis or other forms of revascularization are effective therapies and achieve a significant reduction in short-term mortality. However, with wide application, the costs will be enormous. Furthermore, the complications are not trivial: bleeding of enough severity to require transfusion, cerebrovascular accidents, acute occlusion requiring emergency bypass surgery, and death. In addition, the effectiveness of revascularization attempts may be only in the 65% range or as low as 30% to 35%, depending on the time thrombolysis or other revascularization attempts are made relative to the onset of symptoms and other variables. In addition, there is a significant incidence of restenosis and reinfarction after interruption of the initial acute event so that early interventions may need to be followed up by coronary cineangiography and revascularization, if feasible.

The clinical problem presented by early acute myocardial infarction is one that is ripe for decision modeling. The government and other reimbursement agencies are going to want information on the cost-effectiveness of thrombolysis or angioplasty, or both, and cardiologists' "opinion," no matter how many experts participate in its formulation, is not going to satisfy. The costs are simply too enormous.

A Decision Model: Early Acute Myocardial Infarction

A still evolving decision analysis of early acute myocardial infarction done in conjunction with Robert Dittus at the Regenstrief Institute for Health Care serves to illustrate the advantages (and disadvantages) of decision modeling. The analysis modeled 50 to 60 year old men with myocardial infarction who presented to a medical facility within the first 4 h after the onset of symptoms. The model characterized patients with respect to coronary anatomy and estimated cardiovascular function, based on previous history of myocardial infarction, at the onset of their infarction and modeled the probabilities for living or dying and for the development of angina, congestive heart failure and reinfarction, while at the same time accruing costs for each management strategy in the hospital and at 1 year.

The management alternatives were medical therapy, streptokinase, recombinant tissue-type plasminogen activator (rt-PA), angioplasty or coronary artery bypass surgery. The strategies also included options if symptoms recurred after the first management choice. Therefore, there could be streptokinase followed by medical therapy; rt-PA and later medical therapy; rt-PA and later angioplasty; streptokinase and later angioplasty and so forth.

The data used were derived from large clinical trials and expert opinion. The preliminary results are shown in Table 1.

If one eliminates bypass surgery as an exceedingly costly and morbidity-inducing strategy, the initial use of angioplasty followed by angioplasty for recurrent symptoms or to maximize the probability for maintaining patency achieved

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Table 1. One Year Mortality Rate Probabilities With Differing Interventions and Combinations of Interventions During the Actute Phase of Myocardial Infarction

Initial Therapy	Second Therapy Probability (if required for recurrent symptoms)	1 Year Mortality (%)
Coronary artery bypass	Coronary artery bypass	4.9
Angioplasty	Angioplasty	5.8
Angioplasty	Coronary artery bypass	6.1
Angioplasty	Medical	6.5
rt-PA	Angioplasty	6.5
rt-PA	Medical therapy	7.0
rt-PA	Coronary artery bypass	7.0
Streptokinase	Medical	9.0
Streptokinase	Angioplasty	9.0
Medical	Medical	10.1
Streptokinase	Coronary artery bypass	10.2

rt-PA = recombinant tissue-type plasminogen activator.

the lowest mortality rate of all strategies both in the hospital and at 1 year. Compared with medical therapy, the angioplasty strategy reduced the 1 year mortality rate from 10.1 to 5.8% and increased the 1 year complication-free survival rate from 58 to 71% at a cost, however, of $246,000 per life saved. The thrombolytic strategies (averaged between rt-PA and streptokinase) reduced the mortality rate to 7.7% at a cost of $115,000 per life saved compared with medical therapy.

Compared with the thrombolytic strategies, the angioplasty strategy was more costly in 88% of patients, was more than double the cost in 24% of patients and had a marginal cost per life saved exceeding $400,000. In this model, angioplasty is an effective but expensive approach to care; thrombolytic therapy is a less expensive but effective alternative. The policy issues that could be addressed by this model are obvious.

Discussion

Why physicians distrust decision modeling. Despite the advantages for policy making as well as for clinical decision making that may accrue through the use of decision modeling, its application has been difficult to foster.

Lack of physician acceptance has been a major factor in delaying incorporation of decision modeling into clinical medicine. Although part of the problem may be a lack of familiarity among physicians with the techniques, the major concerns are a mistrust of probability theory and what are conceived to be "made up " data.

The early infarction problem serves to illustrate why physicians have reservations about decision modeling. A decision model of the early myocardial infarction problems requires probabilities of death, congestive heart failure and lethal arrhythmias with and without intervention; probabilities of successful reperfusion; probabilities of significant myocardial salvage; probabilities of complications; probabilities of death from complications and probabilities of reocclusion. Furthermore, these need to be assigned for each strategy employed (medical therapy, streptokinase, rt-PA, angioplasty, coronary artery bypass surgery) and for multiple patient characteristics such as, age, gender, concomitant disease, left ventricular function, "culprit" artery, type of lesion and size of infarction that would be likely to result, among others.

Physicians say the necessary data are simply not available and they do not even want to play the game to get the point where the advantages to be gained from sensitivity analysis can be demonstrated. In addition, it is true that a decision model that incorporates all of the variables having predictive significance becomes quite complex, and estimation of the probabilities and utilities required to complete the structure becomes subject to increasing subjectivity and, potentially, decreasing reliability.

Future requirements of decision modeling. If decision modeling is to achieve professional support, provisions must be made for the incorporation and validation of "real data," such as those derived from clinical trials or observational data bases, or consensus derivations must be refined to the point of acceptability as alternatives for hard data. Until this happens, many physicians will continue to question the validity of the decision models, certainly relative to the individual patient care, but also, unfortunately, for socioeconomic purposes where the requirements for precise subgroup classifications may not be as stringent as for the management of the individual patient.

A second problem that has delayed the growth and application of decision modeling is that it is costly, time-consuming and labor- and expertise-intensive.

The problems of execution of decision modeling coupled with the concern among the participating cardiologists about data reliability have made the progress slow and the utility uncertain. Most government agencies or third party payers want an opinion on the effectiveness of technologic advances in a short period of time. They do not want to wait a year while a decision model is generated and tested for consensus.

Summary

Despite the problems, the rewards of the decision modeling technology in the present and projected economic and political climate can be great because any test or management strategy can be assigned a quantifiable "value" based on its contribution to clinical decision making and resultant patient outcome.

There is no substitute for an informational approach to cost containment. Doctors, government and reimbursement

officials need vastly more information about medical costs as related to the long- and short-term beneficial and detrimental effects of alternative management strategies on patient outcome.

Information needs to be provided as to which care practices are possible at given reimbursement rates and which effective practices would need to be foregone with alternative reimbursement decisions. We need to know if the trade-offs make sense for patients and for society, and we need a system flexible enough to enable us to act on this information.

If it can be accepted that decision modeling can serve a useful role in achieving a balance between cost containment and the preservation of quality care, means and methods need to be developed so that decision models are available in a reasonable time frame, and to make their development an ongoing, appropriately funded and staffed operation. To achieve acceptability, the models must be data-driven either by consensus derivation techniques or preferably by appropriately analyzed observational data bases.

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