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ESSAYS ON TRENDS, INNOVATIVE IDEAS AND CUTTING-EDGE RESEARCH IN HEALTH CARE

Medical Decision-making: Why it must, and how it can, be improved

David Eddy MD, PhD

One of the few things about which everyone can agree is that our health care system is not performing the way we want it to. In the public arena, most of the attention is on the structure and financing of care. However, there is another deeper problem. It concerns the content of care – the tests, treatments and other things providers do to try to prevent and manage diseases. In the end, it is content of care that determines the actual quality and generates the actual costs of the care that people receive. We can rearrange the financing of care all day long, but until we address the content of care – the way that medical decisions are made – we will never get the health care system we want.

The Fundamental Assumption. For centuries, the practice of medicine has been based on a fundamental assumption: physicians know the right things to do and that they do them.[1] We call it clinical judgment or the *art of medicine*. It is the basis for the sacrosanct *doctor-patient relationship* and the belief that physicians should be left alone to make their decisions while the other parts of the health care system are there to play supporting roles (e.g., hospitals to provide the facilities, insurance companies to make the payments, and so forth).

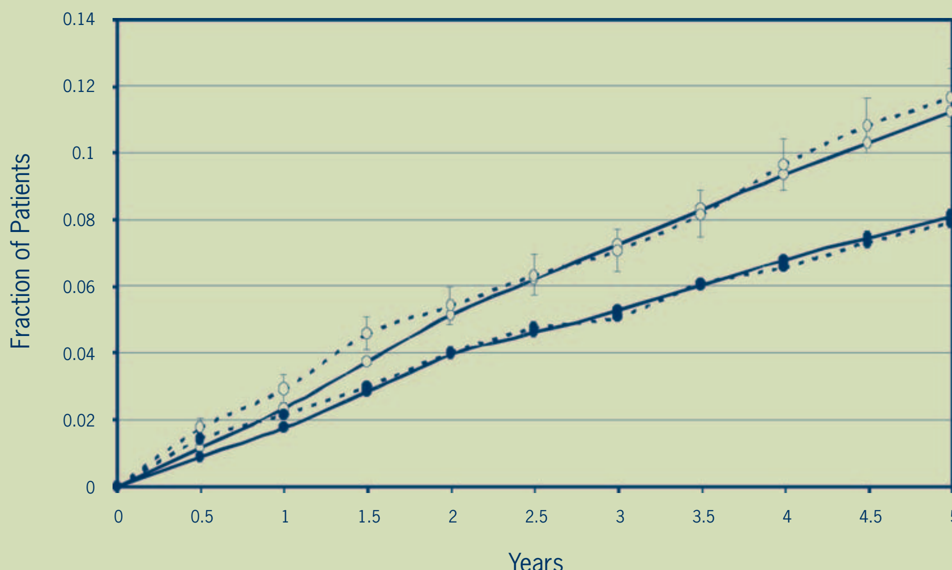
Failures of the Fundamental Assumption. Unfortunately, over the last 40 years unequivocal evidence has demonstrated that this fundamental assumption is wrong. Physicians do not instinctively know the right things to do, their perceptions of the effects of treatments are filled with uncertainty, their decisions are influenced by a wide variety of extraneous factors, their actual choices can vary widely and inexplicably, and even when everyone appears to agree on what should be done, high proportions of physicians do not do it. The literature is now filled with hundreds of studies of wide variations in practice patterns, high rates of inappropriate care, decisions based on little or no evidence, and distortions of decisions by inappropriate incentives.[2] These are the sources of poor quality and high costs.

How do we fix it? The first step is to realize that this problem is not the fault of physicians. The real culprit is that the complexity and pace of modern medical decisions far exceed the limitations of the human mind. No one, not even physicians, can possibly know and process all the information and get it right. So to solve the problem, we need to invent new ways to support the decisions physicians and patients must make.

Guidelines. One way to do this is to improve the quality of guidelines. Guidelines are simple rules of thumb that help transform complex problems that can involve dozens of variables into simple if-then rules (e.g., "if a woman is over 40, recommend an annual mammogram").[3] Guidelines have been part of medical decision-making for centuries and are ubiquitous and very powerful aids to decision-making. As such they provide an obvious and natural way to improve the content of care; the quality of decisions is directly linked to the quality of the guidelines on which the decisions are based. Unfortunately, historically the quality of the guidelines has been very poor; they have done little more than codify current beliefs – however uninformed, uncertain, variable, or inappropriate those beliefs

Figure 1: Example of a simulation of a clinical trial by a mathematical model.[9]

The Heart Protection Study compared Simvastatin (black circles) to a placebo (open circles) in people at high risk of coronary artery disease. The results of the simulated trial are shown as the dotted lines. The results of the real trial are the solid l



might be. Thus, the guidelines of the past did not correct the problems created by the fundamental assumption, they reinforced them. So the strategy now must be to change the way guidelines are created.[4]

Evidence-based medicine. One of the most obvious places to begin is to switch the basis of guidelines from subjective beliefs to actual evidence. This is done by insisting that if a guideline is to recommend a treatment, there should be good evidence that the treatment is actually effective. That is, we should insist that all guidelines either should be *evidence-based*,[5] or should explicitly describe the lack of evidence and admit that the treatment's effects are unknown.[6]

This insistence on evidence may seem simple, but it has profound effects on the quality of medicine. In addition to steering guidelines and other types of decisions towards treatments that we know actually work, it also retards the use of treatments for which there is no evidence of effectiveness, and creates a strong stimulus to improve the collection of evidence. For example, the insistence on evidence stopped the juggernaut toward high dose chemotherapy and bone marrow transplantation and turned a morbid and expensive error into a much more accurate understanding of cancer treatment.[7]

However, evidence-based guidelines do not correct all of the problems we face in modern medical decision-making. As it is currently formulated and applied, evidence-based medicine is largely qualitative. The primary question it asks is, "Is there good evidence that this treatment will cause a beneficial outcome?" But, it does not systematically quantify the magnitude of the benefit. Nor does it estimate the magnitudes of the risks or costs.

Outcomes-based medicine. This is a very important limitation. Without being able to answer *how* beneficial, risky or costly each treatment is, it is not possible to compare benefits versus risks, compare net benefits versus costs, make comparisons across treatments, or optimize the use of resources. Difficult and important decisions require that we make tradeoffs, tradeoffs require numbers, and evidence-based medicine by itself does not systematically provide them. To improve the content of care we need to go beyond evidence-based medicine and get the numbers – the probabilities and magnitudes of all the important outcomes associated with different choices. This is outcomes-based medicine.

The limits of clinical research. How are we going to do this? At first thought it might seem that the route to such quantitative information is through clinical trials. This is indeed a necessary step, but it is not sufficient. The reason is again the complexity of medical decisions. A well-designed trial costs tens to hundreds of millions of dollars and takes five to ten years to compare two or three treatments for a single patient indication in a single setting. When we consider all possible treatments, patient indications, outcomes and settings, it is painfully obvious that we can only conduct clinical trials for a tiny fraction of the questions we face. Even worse, the pace of technological development in medicine is far faster than the pace at which we can do clinical trials. You can't use clinical trials that require five years to study technologies that are changing every year.

Mathematical Models. What then are we to do? It is tempting to fall back on expert judgment, but we already know from past experience that that won't work. The solution is to bring in a third method: mathematical modeling. Mathematical models have proved to be enormously valuable in virtually every other field of human endeavor – from designing bridges and airplanes, to creating virtual worlds for sci-fi movies. The same power can be brought to medicine. Recent advances in the biological sciences, mathematical modeling, and computer hardware and software now make it possible to build models of physiological pathways, clinical events, physician and patient behaviors, and health care systems at a high level of detail and accuracy.[8] Models like this can then be used to calculate the outcomes of a wide variety of potential decisions. Clinical trials that typically take years to complete can now be simulated in a week (not years) for thousands (not millions) of dollars (Figure 1). Physicians and administrators can calculate the effects of new guidelines, disease management programs, benefit designs, pay-for-performance incentives, and other types of policies in the virtual world of the model before deciding on the best strategies. The effects of a new treatment on utilization and costs can be anticipated, optimized and monitored. This progress has been made in only a decade. Over time, as more groups take up this work and more and better data become available, the scope, accuracy, and power of these models will accelerate.

The Future. If we are to achieve the quality, efficiency, and cost of health care we all desire, then human minds, empirical research, and mathematical models are going to have to work together in a much more balanced way than they have in the past. All three are necessary. Empirical research is the foundation of any science and the anchor to reality. However, research can only observe and record results; it can not think. Human minds can think and identify patterns, generate hypotheses, plan experiments, make value judgments, and talk to patients about delicate topics. But human minds have very limited computational skills and are hopelessly outmatched by the volume and complexity of modern medicine. This is where mathematical models come in. They can process virtually unlimited amounts of data and perform extraordinarily complex calculations. But they require human minds to design them, and they are nothing without data.

Historically we have relied on the human mind to do almost all the work in medical decision-making. In the future, the strengths of all three will need to be woven together to improve the quality and cost of care. If we do this well, and we will, the reward will be improvements in health care that can only be imagined today. ■

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