

Efficacy, Risk, and the Determination of Value

Shared Medical Decision Making in the Age of Information

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Patients once left medical decisions to physicians. Physicians told patients what was best for them, and the patients listened. Physicians advised or prescribed, and the patients followed. The patients were as sure of the physicians as the physicians were of themselves.

Times have changed. The value of medical care is in question, and patients have claimed a share of the medical decision-making process.⁸ Against this background and during a period of increasing fiscal constraint and accountability, physicians are struggling to determine what services to offer, as patients try to decide which services to use. Each group's deliberations turn on the quantification and perception of risk.⁹⁻¹¹

How do physicians decide which screening tests, diagnostic procedures, and treatments are worth their patients' consideration? How do patients decide whether they should take an action that might lead to the prevention, palliation, or resolution of a particular symptom, disease, or injury? Both are looking for information that will allow them to say, "If this, then that."⁹⁻¹¹ What they ultimately do with such information is a matter of individual judgment and circumstance.^{9,12}

When calculating the value of an intervention, physicians want to know what percentage of the target population benefits from it—to what degree, at what cost, and at what risk of harm—and, more important, how the intervention's value is modified by a specific patient's age, sex, health status, family history, and so forth. Patients want to know to what extent they are at risk for a particular condition, what the personal significance of that condition would be, what they can do to offset their risk, and what costs and possible harms would be associated with that action.⁹⁻¹¹ Both sets of questions demand evidence that is too often nonexistent. When available, the evidence is frequently misleading, difficult to interpret, or of uncertain clinical significance.¹³⁻¹⁶

This is not to say that when they have the facts in hand physicians and patients make medical decisions solely on the basis of scientific proof. There are and always will be times when fact yields to opinion, just as there will always be debate about the meaning and deter-

mination of medical necessity, proof, value, and effectiveness. Nonetheless, the coherent and impartial presentation of evidence remains a desirable starting point.

PROBLEMS WITH AVAILABLE DATA

Medical decision making is particularly confounded by the tendency of special interest groups—including physicians, scientists, medical journals, pharmaceutical companies, and the mass media—to frame health care information in a way that maximizes its perceived significance.^{5,13-17} For example, treatment benefits are frequently presented without reference to absolute or baseline risks. Telling a man that a medication will reduce his risk of a heart attack by 30% may impress him, but it will not inform if his risk in the absence of that intervention is not specified.

Even when absolute risks are stated, the data often beg further interpretation. The oft-cited calculation that 1 out of every 9 women will develop breast cancer is especially confusing.¹⁸ In fact, the incidence of breast cancer during any given year of a woman's life never rises above 1 in 200.¹⁹ It is less than 1 in 1000 at age 40.¹⁹ The 1-in-9 statistic, which may be readily misconstrued as a woman's chance of having breast cancer at any given moment, offers little or no help when it comes to sorting out the possible harms and benefits associated with breast cancer screening, diagnosis, and treatment.

When a possible benefit is associated with a potentially harmful intervention, decision making can be exceptionally troublesome. This is especially so when the risk of harm in the absence of the intervention is low and the likelihood that the intervention would benefit a specific patient is unknown.^{20,21} Financial costs add yet another dimension to the problem. For example, should a 40-year-old woman's chance of having breast cancer¹⁹ compel us to recommend—or her to request—a mammogram? Should a 50-year-old man have a prostate-specific antigen test? Should either undergo fecal occult blood screening for colon cancer?

INTERPRETING DATA

Uncommon risks may become public health issues when aggregated across large populations. When such issues are brought into the examination room, we often ask ourselves and our patients to confront probabilities that defy intuitive analysis.^{10,22-24} The fact that an individual's tolerance for risk is generally higher than that of society further complicates doctor-patient decision making

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about infrequent events of social concern

Consider the value of a screening, treatment, or prevention option in terms of the following hypothetical study. Twenty thousand patients are randomly assigned to 2 groups and followed up for 5 years. The outcome of interest is death. Treatment group 1 receives drug A. Treatment group 2 receives a placebo. At the conclusion of the study, it is found that 200 people in group 1 died, compared with 300 people in group 2.

Assuming that the study was well designed and executed, the data suggest that the incidence or absolute risk of death over a period of 5 years (X) would be 3% among people similar to those in the study, and that this base-line risk could be reduced to an incidence or absolute risk (Y) of 2% for drug A.

The relative risk reduction (RRR) attributable to drug A $[(1-Y/X) \times 100\%]$ is 33%. The absolute risk reduction (ARR) attributable to drug A (X-Y) is 1%. The number of patients that need to be treated (NNT) with drug A in order to prevent 1 death in 5 years $[1/(X-Y)]$, or the inverse of the ARR] is 100.

If the population at risk contained 50,000,000 people, it could be argued that treatment with drug A would save 500,000 lives over a period of 5 years (the number of people treated times the ARR). Conversely, it could be said that 49,500,000 people, or 99% of those treated with drug A (100% minus the ARR), would receive no benefit from it, benefit having been defined as a reduction in mortality.

If this study were yours, and you received funding from the pharmaceutical company that made drug A, how would you present the results for publication? If you owned the company, how would you present the results to physicians? If you were a news editor, how would you present the results to the public? As a physician, how would you present the results to your patients?

It has been shown clearly and repeatedly that the manner in which data on the costs and benefits of a given intervention are presented influences the user's perception of the intervention's effectiveness.^{8,13,25-31} Studies^{26,29-31} indicate that both patients and physicians (including physicians with advanced training in research design and analysis²⁸) will find a given intervention most effective when supporting evidence is presented in terms of RRR. They will find it less effective when the same evidence is presented in terms of ARR and least effective when it is presented in terms of the NNT.

Research reports that emphasize RRR while downplaying or excluding discussion of absolute risk are particularly disturbing, especially when the initial risk is small. The question of intention aside, reports of this type may drive physicians and patients to the prescription or use of products and services that are of marginal utility in an effort to avoid or minimize what is falsely perceived to be a high-risk situation.

Even when they have the numbers and the ability to decipher them, physicians find the subjective assess-

ment of grave but unlikely threats difficult.^{20,21} It is also difficult for physicians to choose between screening or treatment options associated with small differences in outcomes.³² We should not expect patients to find these tasks any easier, although we may reduce or inflate their perception of risk by various means and thereby induce them to avoid or pursue a particular course of action.

FINDING AND SHARING ESSENTIAL DATA

In an ideal world, a physician trying to help a patient make health care decisions would first access data that clearly supported or rebutted each of the patient's choices. The physician would then share that information with the patient in an objective, unbiased, and readily understood manner. If asked to make decisions for the patient, the physician would be prepared to act on the basis of the best available evidence. The patient's preferences for care would be respected and, under all circumstances, the physician's overriding objective would be the provision of hope and comfort.

Although these ideas may be widely accepted, all but the last are not widely practiced. The root of the problem is a paucity of data that are both meaningful and patient-specific. In an age of seemingly unlimited information, this paradox makes it difficult to sort the wheat from the chaff. Fortunately, multiple efforts to generate and assemble a comprehensive database of readily accessible, scientifically valid, and clinically relevant information are now under way. We are also beginning to explore how, where, and when such information might best be presented to physicians and patients. First approximations of these goals include the *Guide to Clinical Preventive Health Services*,⁴ the Cochrane Collaboration,^{33,34} balance sheets,^{35,36} the interactive computer-based patient education programs produced by the Foundation for Informed Medical Decision Making,³⁷⁻³⁹ and the work of the Agency for Health Care Policy and Research.^{40,41}

In the end, medical decision making that is truly shared and informed will depend on our willingness and ability to support and direct long-term research and development in these and related areas. We must also be willing to take a stand in the marketplace. I encourage all of us — especially those in academic and other agenda-setting positions — to question the value of everything we do in the name of medicine and to challenge the various private and public health care industries, including our own, to provide scientific justification for their goods and services in terms that we and our patients can understand.

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