

ETHICS, EVIDENCE, AND PUBLIC POLICY

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ABSTRACT The growth of evidence-based medicine (EBM) raises a number of ethical issues that have too often been overlooked. These include issues that arise when clinicians make judgments under uncertainty, new challenges for the clinician-patient relationship, new duties for institutional review boards, issues in physician autonomy and reimbursement, and challenges for disclosure and informed consent. Ethics and EBM must be addressed by policy makers and integrated into medical education.

WHEN SCIENTIFIC INSIGHTS BECOME social movements, they often expose themselves to opposition and even ridicule. Perhaps the best example of this is the sad story of Ignaz Semmelweis, the Austrian physician who rediscovered the route of transmission of puerperal fever and ordered that physicians and medical students clean their hands between obstetrics cases (Bridson 1996). This insight, which has saved countless lives, was met at the time with vehement criticism and ridicule; some senior physicians refused to follow the order, thinking hand washing “undignified.” Semmelweis fled Vienna in 1854 and eventually, by some accounts, went mad and took his own life. (Apparently unable to resist irony, others suggest he died from an infection caused by dirty dissection instruments.)

To be sure, *insights* here implies that someone has gotten something right. A scientific qua social movement based on falsehood or misunderstanding might actually deserve criticism and ridicule: think of creationism, certain (but not all) forms of alternative and complementary medicine, and, say, Lysenkoism. (Between

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the clearly or even likely right and the clearly goofy, we have many scientific disputes that will only achieve closure after more time and research: string theory, estrogen replacement therapy, and, again, some forms of alternative medicine.)

One would have thought that evidence-based medicine (EBM) falls under the “clearly or likely right” heading. Its core precept is or should be uncontroversial: Clinicians should practice in light of the best available evidence. Surely no one disagrees with this. Neither should anyone disagree with the British epidemiologist Archie Cochrane (1979), who provided the scientific-moral injunction that has inspired much subsequent work in evidence-based practice: “It is surely a great criticism of our profession that we have not organised a critical summary, by specialty or subspecialty, adapted periodically, of all relevant randomised controlled trials.” Assuming that such trials are correctly to be regarded as the gold standard for evaluating the efficacy of treatments, drugs, and various interventions, then it is nothing less than irrational to argue that physicians, nurses, and other clinicians need not bother learning about the results of these experiments.

Why, then, is there a counterrevolution? Why have apparently rational critics come to oppose EBM, in some cases with great vehemence? The answer—well, *an* answer—to these questions lies in a misunderstanding about the ethical challenges posed by the evidence-based project. At ground, an effort or movement aimed at the moral target of reducing uncertainty in the care of sick people has come to be seen by some as a professional, financial, and legal albatross.

GUIDELINES VERSUS COOKBOOKS

One way that Cochrane’s critical summary can be instantiated is in the form of practice guidelines. Developed by government panels, professional societies, and academic researchers, practice guidelines attempt to gather, evaluate, interpret, synthesize, and then make plain all the research that bears on problems or issues in clinical and public health practice (see National Guideline Clearinghouse 2005). This is no small task, especially given that biomedical research is, sometimes, all over the map. We find ourselves in a very interesting pickle: either we surrender and apologize to Cochrane’s ghost for our inadequacy, or we press on, knowing full well that any research synthesis will be imprecise and probabilistic. Surrender is nuts: doctors, nurses, and others need to learn *something* and need to do *something*, and we should give our best shot at helping them get it right. Uncertainty is unavoidable to one degree or another in any scientific inquiry, but to use uncertainty as a justification for skepticism or relativism is like deciding to stop eating everything forever because last night’s burrito engendered dyspepsia.

Uncertainty is reduced by more and better science. The randomized clinical trial evolved over the past half-century to eliminate individual observational bias and introduce rigor into the collection of biomedical data. It is, apparently, the best we can do,

But even our best tools for reducing bias fail to eliminate it; and some of these tools even present new sources of bias. This—coupled with the facts that (1) nature gives up its secrets slowly and not always in a stepwise, orderly fashion and (2) human variation introduces aspects of great complexity into the business of biomedical discovery—means that even our best evidence will be inconclusive in many cases, and always probabilistic. We sometimes want the world to be simple, and it fails to comply. We apparently want our studies to point in a direct and unambiguous way to a set of beliefs, when the task that is actually before us requires that we use critical judgment to make inferences based on our observations, tests and experiments. (Goodman 2003, p. 43)

What this means is that criticisms of EBM itself cannot hope but to prove too much. That is, if the foundations and goals of evidence-based practice are to be rejected or disdained because the project fails to eliminate uncertainty, then the whole game is lost. Medicine and the other health professions enter a Wild West in which anything goes and one opinion is as good as another, more or less. Are we really prepared to be sanguine about physicians who actually believe these things?

In apparent recognition of the fact that such a stance is untenable, the opposition to evidence-based practice has tended to emphasize not the science but the use or applications of the science, namely, practice guidelines. The guidelines are the distillations of what are often vast amounts of research—research unparseable by individual clinicians. The guidelines generally direct clinicians, when presented with certain maladies, to take particular courses of action. At their best, they guide and inform clinicians about what has worked best for most people. At worst, they are seen and used as rules that brook no variation, “cookbooks” from which one deviates at no small peril.

Now, this is an interesting problem. It is as old as any in the sciences and, for that matter, ethics: how and when should one take a lesson learned in aggregate and apply it in particular instances? The science of epidemiology or the health of populations guides us in addressing the health problems of groups of people; it does not always provide good advice for the individual, here, now, in front of me. Vaccinations have nearly eradicated polio, even as a vanishingly small number of children once got polio from the vaccine. This tension is unavoidable: it is irrational to ignore the guideline, even while following it might cause someone to come to grief.

But note that I say “ignore,” not “slavishly follow.” Those who develop guidelines are aware that there are instances in which they apply and instances in which they do not. The challenge is to tell the difference. Consider the following:

In fact, the EBM guidelines are not guidelines at all. These so-called “best practices” are poised to become coercive mandates imposed by government agencies and third-party payers with political and financial incentives to ration health care—and the power to do it. . . . The public should be alarmed. Despite the

positive ring of terms like “evidence-based medicine,” “best practices,” and “guidelines,” EBM is aimed at stopping the heart of health care—the compassionate, first-do-no-harm, to-my-own-patient-be-true ethics of medicine. . . . Fully implemented, EBM will lead to a limited list of approved health care services—“best practices”—as determined by the agendas and values of a small cadre of politically-motivated, personally biased individuals sitting around a table making treatment decisions somewhere far from the patient’s bedside. (Brase 2005, p. 18)

While this invective is actually written cautiously—guidelines are “poised to become,” “aimed at” and “will lead to” various sources of unhappiness, but have not yet done so—the gist is clear. The instantiations of EBM’s core idea are seen as bad, erosive, even corrupt. How did this come to be?

REIMBURSEMENT AND LIABILITY

Sooner or later it was bound to be about money and lawsuits. In the United States, EBM and managed care were fledged at about the same time. The latter, clumsily applied in vastly many contexts, has at its center two reasonable precepts: (1) health care costs will decline (or not increase so fast) if health care providers have to compete on the basis of quality, outcomes, and price; and (2) there is no good reason to pay for treatments that do not work. It was perhaps inevitable that managed care would adopt evidence-based criteria (Mechanic 2000). If those criteria were applied without exception, and if reimbursement of physicians and hospitals were contingent on adherence to practice guidelines, then EBM would become a tool not exclusively for reducing error and improving quality, but also a cudgel for beating down costs. Worse, at least from the physician or hospital perspective, bad outcomes in cases in which care deviated from the guidelines become risk management nightmares. If you deviate, you die, legally or economically speaking.

Some of this is guilt by association. Managed care, as rolled out, justifiably infuriated half a generation of physicians who were refused admission to “provider panels” that were necessary if they were to continue taking care of long-standing patients, humiliated by having to ask nonclinicians for permission to treat, and silenced under gag orders that forbade telling patients about more expensive treatments (Goodman 2003, p. 104). If evidence-based principles were to grease this engine, then surely they should be scorned, too.

But the entire dispute is based on a mistake—a conceptual and, ultimately, ethical mistake. As epidemiologists have known for decades, the absence of effectiveness is not the same as evidence of ineffectiveness. Sometimes we just do not know with certainty whether a drug or device or treatment will work for a particular patient—no matter how much evidence exists that the drug, device, or treatment tends to work according to the clinical trials and other data that were used to build the practice guidelines. To require 100% adherence to a practice

guideline in exchange for reimbursement is to fail to recognize the probabilistic nature of medical evidence. To win a malpractice suit because a physician did not hew to a guideline is to fail to see that such uncertainty applies to *anyone* who would have treated the same patient.

But none of this should be construed as supporting or constituting an argument in favor of not following guidelines or not availing oneself of the best available evidence:

The goal of effectiveness studies and [clinical practice guidelines] is not, despite what some physicians may believe, to remove all elements of discretion and professional judgment from medical care. There will always be the need—and, one would hope, the latitude—for the exercise of professional judgment. Still, as the body of what is knowable and what is known grows, the degree of latitude will inevitably be [affected] by the extant knowledge base. When one does not know what is right or wrong, everything is fair game to do. Knowledge brings limitations, or at least, the basis for limitations to be imposed. (Rosoff 1995, p. 375; see also Rosoff 2001)

Moreover, policy makers who want to improve individual clinicians' behavior have the obligation to refine descriptions of conditions for applying the guidelines, that is, to make explicit that guidelines do not eliminate uncertainty, and that rationality permits and sometimes requires exceptions—a sort of guideline for following guidelines. And the rest of society needs to join both groups by demanding higher quality, not clearer apportionment of blame.

ETHICS AND UNCERTAINTY

The epistemological issues here are interesting both for their intrinsic importance and for the moral challenges they raise. Three of these challenges are especially significant, and may be put as questions:

1. How should one make an empirical decision under uncertainty when an error might harm someone?
2. How should clinicians communicate about this uncertainty and risk to patients?
3. How ought society respond to the problem of scientific or clinical uncertainty, as well as to the tensions raised as money and malpractice challenge efforts to incorporate more evidence into daily practice?

Much of the discussion so far bears directly on the first question. Although most data and evidence are *from* individuals but *about* populations, we want this evidence to bear on the question how best to treat individual patients:

Our work would be done—the problem solved—if biomedical science, human biology, and the various organisms, toxins, and sharp objects that cause it to

come to grief were simple. None of this is simple. Randomized studies of the same phenomenon sometimes conflict, and their data are not always analyzed correctly or reported with adequate rigor. (The gold standard sometimes needs polishing.) Moreover, clinical trials often reveal subgroup variation or point to our ignorance about groups that were not included in the research in sufficient numbers. Research reduces uncertainty; it rarely eliminates it. . . . It should therefore not surprise us that practice guidelines are themselves works in progress. It would be nothing less than extraordinary if a guideline got it right for all patients and for all time. Why ever would we presume that the moral imperative to do systematic research and make study results easily available would eliminate a feature of all scientific inquiry, namely, that knowledge rarely accretes in a straight line, or that empirical “closure” is once and for all. (Goodman 2004)

Depending on one’s perspective, this is either maddening or exciting. Yet it comes to this: if uncertainty is unavoidable, ordinary clinicians must deal with it. Such a coming to terms will not be achieved either by refusing to use or know of practice guidelines or by following them without exception. This is an ethical problem because it bears on standards of care, which are designed or have evolved in part to protect patients. If a physician errs by failing to read a gardening book and then plants bulbs in the wrong season, the flowers will not grow. But if she errs by not knowing about research that applies to a specific patient’s malady, the patient might not improve, get sicker, or worse. In cases in which guidelines conflict, evidence is scanty, or it seems that the patient is somehow unlike those in whom the efficacy of a particular intervention was demonstrated, the physician has license (even a duty) to come up with something else. But—and here is the nut of it—she must have some sort of reason, warrant, or justification to make this move. Playing hunches or going with intuition or relying on the “art of medicine” are inadequate as reasons, warrants, or justifications.

The demand for reasons is a moral demand in part because of the stakes, the consequences. Under uncertainty, reasons are not morally required to be infallible, dispositive, or even right; but they must be motivated by some sort of inference at the nexus of scientific knowledge and the facts of the case. (One might conceivably make a decision without such an inference if the risk of the consequences of an adverse event were very low.)

The second question—regarding communication with patients about uncertainty and risk—is, ethically, equally important. The standard view of valid consent is arguably one of the great accomplishments of applied ethics. This requires that before a patient may be treated or a subject exposed to the risks of an experiment, he must have adequate information about the risks, benefits, and alternatives; must assent voluntarily, or without coercion or undue influence; and must have adequate mental capacity, or be able to understand and appreciate the information shared during the consent process. Surely, now, “adequate information” ought at least tacitly to include information that helps a patient form a judgment about whether to proceed. Information about the best evidence, the lack of good

evidence, the physician's confidence in the evidence, and many other things might be salient. If there is a practice guideline that counsels A and a physician has reason to believe that B is superior, then no good case can be made to suggest that a patient should not know about both. Put differently, valid consent helps ensure that medical decisions are shared decisions, and a patient's consent emerges as a decision based on some level of understanding of the best evidence or the lack of good evidence or the physician's confidence in the evidence. It will also be shaped by the patient's confidence in the physician, his aversion to risk, his preferences for various tradeoffs, and so on.

Indeed, patient values have been included in the best formulations of evidence-based medicine: "Evidence-based medicine . . . is the integration of best research evidence with clinical expertise and patient values. . . . By *patient values* we mean the unique preferences, concerns and expectations each patient brings to a clinical encounter and which must be integrated into clinical decisions if they are to serve the patient" (Sackett et al. 2000, p. 1). The upshot for us is that patients must share in the management of uncertainty. Strategically and intellectually, this might be difficult—it might even be asking a lot of both physician and patient. But these difficulties are not ethical difficulties. When challenged, moral philosophy produced a practical tool—valid consent—for ethically optimized patient care and human-subjects research. (This is not to ignore imperfections in or challenges to the utility of valid consent; it is only to say that it is a powerful and generally useful moral tool.) How much more is really wanted? One is tempted to say that these remaining difficulties are certainly important and they are clearly interesting—but they are not ethics' problem.

The third question, recall, was this: how should society respond to the problem of scientific or clinical uncertainty, as well as to the tensions raised as money and malpractice challenge efforts to incorporate more evidence into daily practice? Not merely in the domain of policy makers, this issue has come explicitly to include political considerations applying to individual medical procedures (mammography, coronary artery bypass grafts, etc.; Gelijns 2005; Goodman 2003). This is perhaps the most difficult question of all. We can, however, begin to answer it with a suite of recommendations.

Reward Quality, Not Compliance

The move from a "mother-may-I" form of decision review, in which managed care organizations required physicians to seek case-by-case permission to do what they thought best, has been replaced in many instances by a population-based approach that assesses aggregate outcomes. In this model, physicians are evaluated by analyses of their performance over a period. If, all other things being equal, a physician has worse outcomes than colleagues with similar practices and patients, and has deviated from practice guidelines frequently enough to correlate with the difference, then there is reason to insist on revised decision procedures. This approach has the virtue of vetting and rewarding performance

with the same kind of statistical mechanism that was used to generate the evidence in the first place. It preserves a large measure of clinical judgment and what has been called “physician autonomy.” It allows deviation and variation from guidelines, as long as such deviation and variation are within limits supported by *some* scientific data.

Fix the Malpractice Liability Problem

In the United States, at least, medical liability torts are a “through-the-looking-glass” social mess, if not a catastrophe. Long before managed care, and ever since, a physician could lose a lawsuit for an outcome that might have occurred for anyone. The very idea of a “standard of care” is itself evidence-based, but rarely regarded as probabilistic under the law. Policy makers and legislatures need to ensure that practice guidelines are not regarded as possessing more certainty than appropriate. Too, they ought to reward or otherwise promote the development of systems in which bona fide errors that cause bad outcomes are responded to with adequate compensation for patients. They should eliminate the blame mongering and dueling experts that turn so many malpractice suits into carnivals of invective and finger-pointing.

Do More and Better Science, and Improve Education

Uncertainty is reduced by research, and research that is hidden or unknown does no one any good. Institutional review boards or research ethics committees should help ensure that if humans are going to be put at risk for the sake of improved treatments, drugs, and devices, then investigators must do their duty to publish accurately and comprehensively, and to conduct studies that answer biological and medical questions instead of secure market share. Moreover, the ethical challenges of EBM should be included in medical school curricula. Students must be made to understand the probabilistic nature of evidence, that rationality and morality require reasons, and that the overarching goal of the healing professions is healing—a goal that often demands of healers that they resist an ensemble of epistemic, social, and perhaps even economic temptations.

CONCLUSION

EBM has evolved from a professional insight to a social movement to a *bête noire* to a political hot potato. It guides (or vexes) the physician at the bedside and inspires (or confuses) the policy makers and educators who must generalize its best features. And, too often overlooked, it raises interesting and important ethical issues, issues that might be different in form or substance from those usually identified in clinical practice, research, and public policy.

One reason for this is as old as any in the sciences: namely, the conceptual and epistemological challenge inherent in making inferences from individual data sources to collective evidence, and then back to individuals—patients. Most such

inferences are probabilistic and are shaped by some degree of uncertainty. If we were concerned with the evolution of galaxies or the daily life of Phoenicians or the best computer program to parse a sentence in Urdu, it would matter less if we got it wrong. The ethical issues raised by EBM come to constitute an imperative to include in education and practice consideration of individual and collective responsibility for making sound inferences.

This can be quite difficult, especially for those who protest that they want to be healers, not logicians. But in making explicit the moral duty to make sound decisions—not impossible even in the face of uncertainty—we lay bare the fact that the healing professions were never about and ought never be about mere technical competence, simple practice, or the quotidian laying on of hands.

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