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Issue Backgrounder

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Compulsory Evidence-Based Medicine:

An Unproven Idea That Shouldn't Be Law

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I. What is evidence-based medicine (EBM)?

Proponents like EBM originator David Sackett say evidence-based medicine is simply a tool to further the "conscientious, explicit and judicious use of current best evidence in making decisions about the care of the individual patient. It means integrating individual clinical expertise with the best available external clinical evidence from systematic research."

II. So, who could possibly have a problem with using "the best evidence from systematic research?"

No one. That is what medicine does. The last 300 years of medical progress haven't come about because physicians use Ouija boards to make decisions. But what, exactly, is "the best?" Real science is a messy social endeavor. It mixes observation, experiment, controlled trials, and seemingly far-fetched proposals into an ongoing conversation that takes place in papers, meetings, talks, informal conversations, and formal education. Progress occurs when the good ideas are separated from the bad ones through a process of ruling out other possible explanations. Over time, informed judgment, careful observation, repeated trials, elegant experiments, inelegant experiments, and pure serendipity have uncovered the medical miracles we now take for granted.

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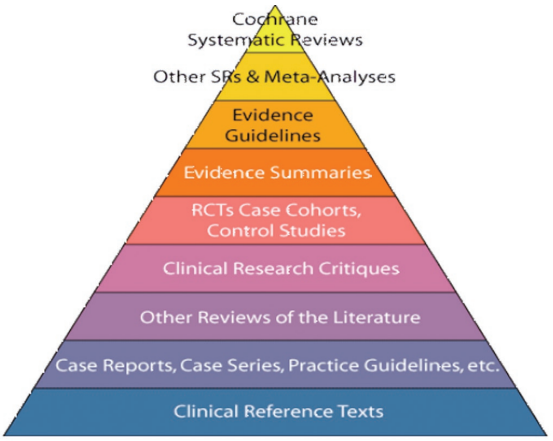
Evidence-based medicine proponents think that imposing their standards on physicians will improve clinical treatment. In the normal course of scientific inquiry, physicians will voluntarily adopt EBM standards when they are convinced that they are an improvement. But converting physicians takes clear and convincing evidence, and amassing that takes time. Evidence-based medicine proponents are impatient with the messy discursiveness of real scientific inquiry. They seek to streamline it by having the law empower a handful of experts to dictate which sources of information clinical practitioners must revere and which can be safely ignored. A typical information hierarchy from the University of Washington's evidence-based toolkit is shown below.



Cochrane systematic reviews are systematized meta-analyses, studies in which researchers use a specific process when they combine a number of different study results into one overall conclusion. "Other SRs and Meta-Analyses" refers to combination reviews done by organizations other than the Cochrane Collaboration.

Evidence guidelines are papers containing their recommendations for best practices. Evidence summaries explain what is and is not known about a particular problem. RCT stands for randomized controlled trials, studies that use statistical techniques to compare results when two similar groups of patients receive different treatments.

Evidence-based medicine proponents are comfortable discarding evidence that does not come from randomized controlled trials and meta-analysis. In fact, Sackett and his co-authors advised people to ignore observational studies. "If you find that [a] study was not randomized," they wrote, "we'd suggest that you stop reading it and go on to the next article."[1]

III. But Aren't Randomized controlled trials, and informed review of them, the "Gold Standard" of Evidence in Evaluating the Efficacy of Clinical Interventions?

Only if well designed and appropriately used, which is difficult to do and fiendishly expensive. Even then, the results may hold only for a like group of patients. For a variety of reasons, an individual might not be the same as the group an intervention was tested on. In that case, imposing the treatment recommendations from the group trial on a particular individual could do harm.

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Small sample sizes are a major problem with randomized controlled trials. In statistical parlance, such studies are "underpowered," not large enough to detect differences between treatments unless the difference is very large. One study of RCTs for surgery found that of those that reported sample size calculations, only half of them had sample sizes large enough to detect treatment differences as large as 50%.[2] This is of particular concern in drug and other trials that compare the relative efficacy of old and new treatments. Underpowered RCTs are biased towards finding no difference, a fact that makes evidence-based medicine an attractive justification for arbitrary rationing decisions. Meta-analyses can compound the problem by introducing other biases. Research shows that meta-analytic conclusions vary substantially depending on how trials are selected for inclusion, and how outcomes are defined.

Randomized controlled trials are simply an investigative tool that uses statistical controls to help sort out imperfect human perceptions of causality. But statistics are not science. In many cases no reasonably likely bias would affect a conclusion, and requiring a RCT wastes money that might be better spent on more narrowly focused improvements. In cases in which observation clearly shows improvement, demanding a RCT may also be unethical.

IV. Blah, blah, blah, statistics, blah, blah, blah. Better safe than sorry. Give one concrete case in which forcing physicians to follow guidelines based on a randomized controlled trail would do harm.

The ALLHAT trial was a large clinical trial on 42,418 patients conducted by the National Heart, Lung, and Blood Institute (NHLBI). It was designed to compare how well four drugs from different classes controlled high blood pressure, lowered lipids, and prevented heart attack. At a reported cost of $125 million, it found no evidence that a calcium channel blocker, an alpha-blocker, or an ACE inhibitor did a better job of preventing heart attacks or fatal coronary heart disease than a simple diuretic. The press, with lurid headlines like "Common water pills outperform pricey drugs in big study," and "Diuretics' value drowned out by trumpeting of newer drugs," enthusiastically reported the study results in December 2002.

By May 2003, the NHLBI had released the express JNC 7, new clinical guidelines for treating hypertension that were based on the ALLHAT study. These recommended that "thiazide-type diuretics should be used in drug treatment for most patients with uncomplicated hypertension."

By mid-2003 the first papers questioning the applicability of the ALLHAT results began appearing. They were stimulated by the fact that ALLHAT study results contradicted those from other large trials, including the Second Australian National Blood Pressure Study and the Anglo-Scandinavian Outcomes Trial. The Australian trial compared a diuretic with an ACE inhibitor and found that ACE inhibitors lowered deaths in its sample of Caucasian men by about 11%.

Since then, a number of problems with the ALLHAT study have been identified. The study design was biased in favor of the diuretic. One reason was that African-Americans made up 35% of the patient sample. As a group, they tend to be less responsive to ACE inhibitors than Caucasians.

Another reason was that if the first drug given a patient failed to control blood pressure, the research protocol prohibited physicians from adding a drug from one of the other study classes. This made beta blockers the main option for controlling recalcitrant hypertension. Though they are known to help in combination with diuretics, they are not normally used with ACE inhibitors. As a result, the diuretic group had better blood pressure control than the ACE inhibitor group, a difference that would be expected to affect outcomes in its favor. One British critic wrote that the drugs used in ALLHAT would "more commonly [be] found in a pharmaceutical museum than in our patients."

At five years, the group on the diuretic had a significantly higher risk of developing new-onset diabetes, and though investigators claimed that people on the diuretic had fewer heart attacks and less heart failure, this did not affect the overall death rate. In fact, for nonblack patients, overall mortality was 3 to 6 percent lower in the groups not on the diuretic. One recent paper dismisses ALLHAT as uninformative due to inadequate power, lower than projected sample size, and poor compliance.

The National Heart, Lung, and Blood Institute both conducted the ALLHAT study and appoints the members of the National Committee on Prevention, Detection, Evaluation, and Treatment of High Blood Pressure, the group that published the JNC 7 guidelines. As Michael A. Weber has pointed out, "Although not presented in the ALLHAT report, the relative cost of drugs somehow became part of its published conclusion."[3]

It specifically avoided handing down rigid rules designed to constrain clinical judgment concerning individual patients, and instead concentrated on offering the most balanced information it could for all involved.

The Reference Card published as a brief summary of the new JNC 7 guidelines makes no mention of race as a compelling indication for choosing among initial drug classes. If physicians were compelled to follow this "evidence-based" guideline, nonblack men would be put on the same drug treatment that increased their risk of death by 3 to 6 percent in the ALLHAT study alone.

Unsurprisingly, the new European guidelines for treating hypertension differ significantly from the American ones. The European committee noted that controlled randomized hypertension trials last for only 4-5 years while patients are on antihypertensive medications 20 to 30 years. It specifically avoided handing down rigid rules designed to constrain clinical judgment concerning individual patients, and instead concentrated on offering the most balanced information it could for all involved.

V. But if compulsory EBM isn't good, why are so many people promoting it?

Money and power. Physicians who deviate from compulsory evidence-based medicine guidelines would be prime targets for trial lawyers. In practice, this means that compulsory EBM shifts the power to determine medical practice from clinicians who deliver care to patients to people in government and academia who manage to get assigned to the right committees. As currently designed, EMB has high overhead costs and hard evidence showing that its benefits exceed its costs is conspicuously lacking. In the last decade a number of groups have been developing evidence-based practice guidelines using grant funds. If EBM is statutory, they could make considerable amounts of money selling those guidelines.

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Finally, under model EBM statutes, medical decision-making is collaborative. Patients no longer have final authority to decide on the kind of care they receive, and physicians who deviate from guidelines will be sued. Compulsory EBM politicizes medicine by ceding control to small committees, decreases the likelihood that research disagreeing with guidelines will be funded, and increases the likelihood that decisions about treatment will end up in the courts.

Footnotes

[1] Sackett DL, Richardson WS, Rosenberg W, Haynes RB. *Evidence-based medicine: how to practice and teach EBM.* New York: Churchill Livingtone, 1997. Quoted in Benson K, and Hartz A, June 22, 2000. A Comparison of Observational Studies and Randomized, Controlled Trials. *New England Journal of Medicine*, Volume 342, 25.1878-1889. Online version as of January 7, 2004.

[2] Maggard MA, O'Connell JB, Liu JH, Etzioni DA, Ko CY. "Sample size calculations in surgery: are they done correctly? *Surgery.* 2003 Aug; 143(2):275-9. Vickers AJ. Underpowering in randomized trials reporting a sample size calculation. *J. Clin Epidemiol*. 2003 Aug; 56(8):717-20.

[3] Michael A. Weber. 2003. "The ALLHAT Report: A Case of Information and Misinformation. *J Clin Hypertens* 5(1): 9-13.

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