



One-Size Fits All Medicine? G'Day Mate

"One size fits all" rarely does. From clothes to shoes to hats, few people find that items carrying that label work with their individual bodies. So why do we entrust the health of our bodies -- one of the most important assets we have -- to a one-size-fits-all mentality?

Unfortunately, policies being advanced under the guise of "evidence-based medicine" (EBM) could do just that.

The idea behind EBM – empowering physicians with sound evidence to incorporate into their treatment decisions for individual patients – is a good one. Unfortunately, EBM now is being distorted by government bureaucrats and HMOs in ways that impose top-down, one-size-fits-all restrictions on patients and their healthcare providers.

This EBM model relies heavily on findings from randomized clinical trials. While these trials are essential to demonstrating the safety and efficacy of new medical products, the results are based on large population averages that rarely if ever will tell us which treatments are “best” for which patients. That is why it is so critically important for the physician to maintain his or her ability to combine study findings with their expertise and knowledge of the individual in order to make the optimal treatment decisions.

Evidence-based medicine in its present, distorted form emphasizes just one aspect of the clinical pie over all the others. This model, which began taking shape in the 1970s, is now broken and outdated.

For the most part it is a retrospective look at clinical studies and head-to-head comparisons of medicines and medical procedures. EBM may involve a careful look at the science. But in practice, it's very limited. All of these studies are population-based, have rigid exclusion criteria and can't integrate new information or innovations. Today evidence-based medicine means cost-based medicines.

The result is decidedly and transparently a narrow one: to eliminate "practice variation to what has been known to work – in the past.

Practice variation is any treatment that varies from the norm that EBM prescribes. The canard of evidence-based medicine is the belief that practice variation is bad and that one-size-fits-all medicine is good. EBM presupposes that all people respond precisely the same way to all medicines. But that's simply not true. Disease varies by individual, and selection of treatment must be driven by diagnostics, not just guidelines. At its core evidence-based

medicine is cost-based rather than patient-based. Disease varies by individual and selection of treatment must be driven by diagnostics and judgment not simply “guidelines.”

For evidence of the problems with the current EBM model, consider the Australian model of granting access only to treatments that government bureaucrats have decided are “cost-effective.” That is precisely what a new study by IMS Consulting study did. Consider the facts and be afraid – very afraid – of the implications. The IMS study included examination of two diseases, osteoporosis and Alzheimer’s, where the Australians are regularly denied access to medicines available to American patients. According to the new IMS study:

- By 2007, approximately 9.1 million patients in the United States with osteoporosis would be denied access to treatment choices if we adopted the Australian model of cost-based (aka, “evidence-based”) medicine. In Australia, for example, newer medicines for osteoporosis that are not “on the list” for reimbursement may be made available only after a patient suffers a fracture.
- IMS estimates that a 1.6 million Alzheimer’s patients could be impacted if we adopted the same system as Australia. The Aussie guidelines are identical for Aricept (donepezil), Exelon (rivastigmine), and Razadyne (galantamine) – and are highly restrictive compared with US guidelines. Australia also limits coverage of Alzheimer’s medicines to six months of treatment unless the patient shows “significant improvement.” In the US, the decision to continue treatment is based on patient (and care-giver) satisfaction – which includes maintenance of current mental status and prevention of mental decline – quite a difference from “significant improvement.”

Restrictive formularies (in the US both public and private) and health care systems (in Australia, the EU, Canada, and elsewhere) that deny access to the right drug for the right patient at the right time but pay for more expensive and invasive procedures later on have their priorities upside down.

Perverse in so many ways — not the least of which is that “savings” are entirely transitory.

Consider the facts. A longitudinal prospective study by Dr. Susan Horn, et al., examined the relationship between HMO cost-containment strategies and utilization and total cost of health care for a number of medical (non-psychiatric) illnesses. The study showed that the tighter the formulary restrictions, the higher the overall cost of care — and what drove the increased costs was the association between formulary restrictions and utilization of care.

A blunter way of putting this is that “efficient,” as the term has come to be defined, has nothing to do with patient care. “Efficient” is a cost-based word and patient-centric concerns be damned. Welcome to the world of health technology assessment, aka evidence-based medicine, aka rational use of medicine.

The existing philosophy and practice of evidence-based medicine try to eliminate practice variation. **But, since they are population-based, EBM’s analyses and the guidelines that flow from them are so flawed that the generally eliminate about 60 percent of the**

variation that actually exists in individuals. The effort to develop *predictive* approaches to health care using *retrospective* evaluation of population-based studies is outdated, *dangerously outdated*, in an era where new medical products use the integration of complex biology in both the screening of patients and the development of medicines.

This approach to evidence-based medicine uses the tools and concepts of the last century when a new toolkit is required that reflects this century's new personalized medical technologies and empowered healthcare consumers.

The triumph of modern medicine is that it can be so precisely targeted to a single patient's needs. It is a dramatic leap forward from EBM's sweeping approach, which sees only through the broad lens of population-based studies, and the individual patient is kept out of focus.

Unfortunately, the current, misguided EBM model is enjoying growing support among HMO and government bureaucrats. Why? Because it puts a veil of pseudo-science around blunt, one-size-fits-all cost controls that rely on denying patients access to effective treatments. This is extremely shortsighted, and out of step with emerging science and health care delivery models. The fact that HMOs and government bureaucrats are signing on so readily should, in and of itself, should be a warning signal that “money” and not the “patient” is at the center of the decision making process.

Evidence-based medicine may provide transitory savings in the short term, but the same patient who takes the cheapest available statin today may very well be the patient costing you -- the taxpayer, the policymaker, the thought-leader, the sister, the spouse -- big bucks when that patient ends up in the hospital because of improperly treated cardiovascular disease.

The repercussions of choosing short-term thinking over long-term results and cost-based medicine over patient-based are pernicious to both the public purse and the public health.

As Mark McClellan, the chief architect of American health care policy said, “Looking at a gigantic uniform solution for everything is never going to work.” We must move forward by looking ahead.

Today, the science of genomics is ushering in an age of personalized medicine where people can be screened with a variety of molecular diagnostics to both reduce side effects, increase compliance, improve outcomes, *and even prevent* various forms of cancer, depression, hypertension, Alzheimer's and immune disorders. This is 21st century evidence-based medicine -- ***patient-centric and cost-efficient***. Evidence-based medicine and policy must be adapted to reflect this emerging science. Many providers are currently testing genotypes that predispose someone to coronary disease and “tailor” their medical therapy to what is going on in their body (i.e. personalized medicine). Grandpa was right when he stated “an ounce of prevention is worth a pound of cure.” We are now truly at the threshold of this thinking in medicine if EBM in its current form doesn't stop progress in its tracks.

Today and increasingly in the future, clinical outcomes can be monitored more precisely and computers can control for hundreds of variables to help doctors and researchers identify what treatments steps matter most in improving care. This is 21st century evidence-based medicine – *patient centric and cost-efficient*.

We need a new approach to evidence for a new era of medicine We must develop the studies, tools, and metrics that will provide policymakers (not just inside the Beltway, but also in Brussels and the capitals of Europe), payors (public and private), and providers with the ability to evaluate, support and use new medicines and medical technologies to improve patient well-being in a patient-based, cost-efficient environment.

We must provide the 21st century health care evidence so crucial to the decision process of policy-makers and the policy debate of thought leaders – and must do so in a coordinated, rigorous manner via evidence-based policy approaches that:

1. Recognize The Value of Medical Innovation

We need studies to evaluate the cost-effectiveness of molecular diagnostics and new medicines in terms of more rapid access to critical (often lifesaving) new drugs. Not just how much they “cost” versus alternative therapeutic choices.

Policies should support adoption of new medicines, molecular diagnostics, and health information technology as part of the solution to our health care challenge. We must recognize and address the fact that one of our single biggest health system failures – as demonstrated by evidence-based medicine – is the failure to make sure that patients receive and adhere to needed therapies.

This also requires policies that consider cost impacts across the health system rather than in narrow budget silos. Increasingly, evidence is revealing ways in which increased spending on medicines yields cost offsets in other parts of the system.

It also means we must recognize the limitations of single, centralized, decisions about the value of innovation based on population averages. Health policy must reflect the heterogeneity of treatment effect – that is, the fact that patients are not averages but will respond differently to medicines; and the ways that use and value of innovation evolve over time.

2. Support the Healthcare provider and Patient as Empowered Technology Assessors

We have a growing capability for rapid communication of medical evidence and information, and an increasing emphasis on the role of the consumer in health care decision-making. This creates new opportunities for EBM done right – that is, EBM that delivers useful, accurate, and balanced information to Healthcare providers, patients and consumer at the point of decision-making.

3. Reflect the Emerging Science of Personalized Medicine

There is a growing body of evidence that tiered and restrictive formularies discourage appropriate utilization of medicines designed to prevent or delay onset of chronic disease.

In an era of personalized medicine, the goal should be (for example) to identify people at risk for metabolic syndrome and prescribe a medicine to proactively protect their heart and delay diabetes -- rather continue to prescribe the “least expensive” medicine.

We must compare preventive and personalized formulary outcomes with the tiered approach to demonstrate the impact in terms of health outcomes and total health care costs. We must evaluate the impact of restrictiveness on mortality, morbidity and social costs (disability, long term care, welfare benefits).

Policy-makers must work closely with the leading bioinformatic companies and health systems to develop state of the art approaches to both documenting and improving patient well-being, allowing us to demonstrate that the “looking backwards” approach and heavy reliance on randomized clinical trials is often overly burdensome and outdated.

There must be a partnership, at the federal level, a partnership of states, health systems, hospitals and health plans to develop actual programs that can be implemented to improve outcomes and the processes/best practices to achieve these results -- *patient-centric, cost-efficient results.*

A rapidly aging society demands a new health care capable of providing for its needs in the 21st century. **Equality of Care** must now be matched with **Quality of Care**. We need a new approach. The health care community must work together to develop new cost-efficient programs that account for modern genomics and individual screening. Because "one size fits all" policies are dangerously outdated in this era of patient-centric medicine.

As John Adams said, *“Facts are stubborn things; and whatever may be our wishes, our inclinations, or the dictates of our passion, they cannot alter the state of facts and evidence.”*

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