

Health Technology Assessment and Evidence-Based Medicine: What Are We Talking About?

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This *Value in Health* Special Issue describes technology assessment (TA) programs in five countries. The primary purpose is to provide information about the ways coverage and related decisions are made in each country, and how TA is used to support those decisions. For this purpose each article stands on its own. Individually they are helpful to understand the decision-making processes, as well as the methods used to assess technology in these countries. But when the articles are considered together, they tell us much more.

One immediate observation is the extent to which TA is being used. These articles describe a very impressive range of programs. These are not intellectual exercises; they are integral parts of national decision-making processes. TA has clearly come a long way from its origins about 30 years ago.

A second observation relates to the diversity of the programs. Given that all the programs stem from a common source; this is more surprising. They all have the same motivation, which is to understand the effects of health-care technologies on important outcomes. And they all share a commitment to the same approach, which is that to the greatest extent possible the understanding should be based on rigorous evidence and analytical methods. From this, one might expect the programs to be very similar, differing only in details dictated by local conditions such as committee structures. But the differences we see are considerably deeper than just committee structures; they affect such things as the types of decisions to which TA is applied, the mandates given to those applying them, the types of outcomes they should consider, the criteria for drawing conclusions, and the extent to which the conclusions are binding.

Looking across the spectrum of programs one might wonder whether the authors are really talking about the same thing. Some emphasize an analysis of evidence and for all intents and purposes stop there. Some look more like health economics and outcomes research. And for others the centerpiece is a cost-effectiveness analysis. Just what is TA, if it can morph into so many different forms? Are the programs actually doing very different things—evidence-based medicine (EBM), outcomes analysis, cost-effectiveness analysis—and are we being polite to call them all TA? Does the interpretation of the term “technology assessment” vary so widely that in fact there is no such thing? Or does TA encompass all of these as well as other disciplines, and the different programs just vary in the extent to which they use them?

Health TA as defined in International Society For Pharmacoeconomics and Outcomes Research’s (ISPOR) *Health-Care Cost*,

Quality, and Outcomes: ISPOR Book Terms [1] is “a form of policy research that examines short- and long-term consequences of the application of a health-care technology. Properties assessed include evidence of safety, efficacy, patient-reported outcomes, real world effectiveness, cost and cost-effectiveness as well as social, legal, ethical, and political impacts” [1]. The breadth of this definition clearly argues for the third interpretation in the previous paragraph—that TA is an umbrella term that encompasses a wide variety of applications and methods. But if we accept this, then what are we to make of the fact that some of the programs described in this Special Issue only do, for example, an evaluation of evidence for safety and efficacy? Some of the programs not only fail to perform a cost-effectiveness analysis; they are actually forbidden to do so. Should we cast these programs out of TA, and say that they just practice EBM?

The most obvious resolution to these questions, I believe, is to recognize that a “full” TA has several different parts, and that different programs emphasize different parts. But the programs described in this Special Issue illustrate something more interesting than this. There is an order to the parts methodologically, making it reasonable to consider them “stages” towards a full TA, and the different programs have progressed to different stages. Thus some programs might be considered not just “different,” in some arbitrary sense, but “further along” toward a full TA.

The four main stages are apparent in ISPOR’s definition. The first is an evidence analysis—a systematic evaluation of evidence for a technology and a requirement of good evidence for such things as coverage, placement on formularies, and affirmative guidelines. This stage corresponds to the evidence-based guidelines (EBGs) part of EBM. The second stage is an outcomes analysis. In this stage there is an estimation of the magnitude of the effects of the technology on the desired clinical outcomes (the “benefits”) and on potential harms such as side effects and risks (the “risks”). This stage also includes a comparison of benefits and risks, to determine if the “benefit-risk ratio” is sufficiently high to justify the technology. The third and fourth stages are analyses of costs and cost-effectiveness; here the researcher estimates the effect of the technology on costs and compares the clinical effects against the costs to determine if the ratio is sufficiently high. The last stage is the analysis of the ethical and legal implications of the technology.

The methodological progression of these stages is apparent. One can not estimate the magnitude of clinical outcomes (stage 2) without first evaluating the clinical evidence (stage 1). One can not compare the costs and cost-effectiveness (stages 3 and 4) without estimating the effects on the clinical outcomes (stage 2). And one can not think about ethical and legal implications of the technology (stage 5), until one knows something about the costs and cost-effectiveness (stages 3 and 4).

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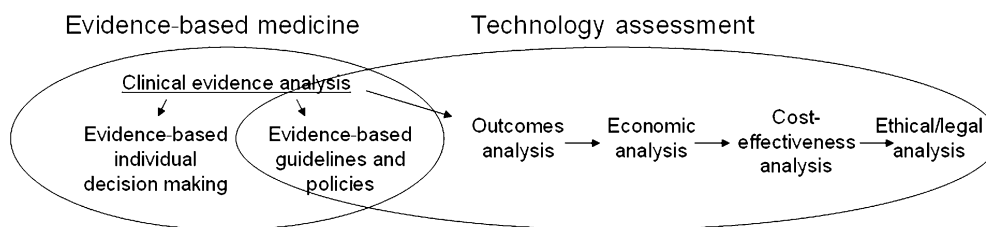


Figure 1 Relationship of evidence-based medicine and technology assessment.

That seems fine. But it still begs the question of why different programs stop at different stages? The most obvious reason is that as a TA progresses from stage to stage there is more work to be done. It is easier, faster, and less expensive to simply do an analysis of the evidence and stop there. Another reason is that the methods for estimating the magnitudes of clinical benefits, estimating the magnitude of costs, and comparing them, are not nearly as straightforward or universally accepted as the methods for evaluating evidence (the first stage).

But there is also a third reason. It is that these stages are not only ordered methodologically, they are also ordered in terms of their political and social acceptability. Until very recently the appropriateness of a technology was determined pretty much by whatever physicians wanted to do. No further information was needed. From the point of view of physicians and patients, this is clearly a highly desirable “methodology” for determining the coverage of a technology. It puts virtually no restrictions on what can be done or paid for. Furthermore this method has prevailed for hundreds of years. People are not only used to it, they are addicted to it. The first stage of a TA, the analysis of evidence, is not only younger (about 20 years old) but considerably more restrictive. It says that before a physician can do something there must be a systematic evaluation of evidence, and only those things that are supported by good evidence will be paid for. The second stage, which calls for estimating and comparing the magnitudes of benefits and harms, is even more restrictive. It implies that there is some threshold beyond which even effective treatments might be denied if the benefit risk ratio is considered too small. Finally, an explicit consideration of costs is the most restrictive of all. It explicitly states that effective technologies can be denied if they are deemed to cost too much. In societies where for decades few have had to pay directly for their health care, this is the most obnoxious of all.

So the fact that TA programs in different countries have progressed to different stages reflects not only methodological concerns but social and political concerns as well. The fact is that different countries have reached different stages in their social and political acceptance of the different parts of a TA. Correspondingly, the TA programs in different countries have reached different stages. In many settings the profession and the public are not ready for full TAs and the difficult choices (rationing) they imply. Given the strengths of the methodologists working around the world, it is almost certainly these social and political constraints, and not the resource or methodological constraints that have caused different programs to get stuck at different phases in their TAs.

A final element of TA illustrated by these articles is its relationship to EBM. As already noted, every TA begins with an evaluation of the evidence for the technology being assessed. In that sense EBM is a part of TA, the other parts being the estimation of the magnitudes of the clinical outcomes (outcomes analysis), the

evaluation of costs (economic analysis), the comparison of health and economic outcomes (cost-effectiveness analysis or CEA), and an evaluation of ethical factors. But EBM itself has parts [2]. One is evidence-based individual decision-making (EBID). As the name implies this type of EBM focuses on the evidence pertaining to an individual patient and his or her management. As originally proposed, it emphasizes the education of physicians in how to bring evidence to bear on decisions about individual patients [3], and the synthesis of evidence with clinical judgment [4]. The other part is evidence-based guidelines (EBGs) [5] or more generally evidence-based policymaking. This part describes the importance of basing population-based policies like guidelines, coverage policies, formulary decisions, and performance measurement on evidence and it is this part that stresses the principle that before any population-based policy can be promoted there should be good evidence that the policy will be effective and beneficial. Thus the relationship between TA and EBM can be nicely visualized as a Venn diagram as is shown in Figure 1.

EBM consists of EBID and EBG, whereas TA consists of the evidence used in developing EBG, outcomes analysis, economic analysis, cost-effectiveness analysis, and ethical/legal analysis. The two overlap at EBM; the principles and methods for developing EBGs are an integral part of both TA and EBM, but both TA and EBM use other principles and methods to be complete.

Those of us who believe in TA have been fairly successful in gaining acceptance for the evidence part of a TA. Every program described in this series of articles starts off with this aspect of EBM. The fact that only a subset of programs goes beyond that, to explicitly estimate the magnitudes of benefits and harms, and to explicitly incorporate costs, tells us that we have not been nearly as successful in helping the profession and public understand the value of these other parts of TA. This must be addressed if TA is to continue to progress and reach its full potential.

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