Where are the Limits of Cost-Effectiveness Analysis and Health Technology Assessment?

Anthony J Culyer, CBE, BA, Hon DEcon, Hon FRCP, FRSA, FMedSci*

* Professor at the University of Toronto, Canada and University of York, England

The advent of a second edition of the Thai guidance on HTA, with its admirable stated aims of clarifying, updating and widening the scope of HTA in order to include budget impact analysis and assessments of social and ethical impact, seems a good time to ask what the limits of the scope of HTA truly are, and whether they are inherently conceptual in character or empirical and practical. One might think it a bit late to be reflecting thus after the revisions and all the brainstorming and consultations have been completed—rather like shutting the stable door after the horse has bolted. On the other hand, to have reached conclusions about some of the extended scope is not necessarily to have concluded any discussion of what the scope can or ought to be. In this short editorial I aim to set out a few ideas that may serve at least in part as an agenda for the third revision!

The authors have an excellent definition of HTA:

Health technology assessment is a [form of] policy research which integrates multidisciplinary fields in a systematic way in order to determine the effects on the use of health technology in short and long terms, direct and indirect effects, intentional or unintentional effects, effects of the development and diffusion of technology, and the group of related technologies and issues related to the application of technology.

The trouble with this definition is that it is a statement of aspiration rather than a statement of what HTA can or does do. There is nothing wrong with the aspiration. On the contrary there is a good deal that is right about it. However, HTA, as currently practiced, does not “integrate multidisciplinary fields” nor does it approach the issues in trying to do so in “a systematic way”. The list of short and long-term intentional and unintentional consequences is a very thinly populated list in all examples in practice.

My own view is that the reasons for this state of affairs are partly inherent and conceptual and partly empirical and practical. If the only difficulties were empirical and practical, I do not doubt that solutions would be found and the central issue would be one of assessing the value of the additional information. An appropriate way of thinking about those issues is readily available.

The inherent and conceptual difficulties have an altogether different character. The main problem here is that we do not have an all-encompassing theory of human welfare that integrates the various categories of effect that might properly be appraised. There are many lesser conceptual problems too, such as the metrics by which “more” or “less” of some of the entities of interest might be measured (“distributional fairness”? “fairness of what”? health, health care, social care?... “enhanced dignity”? “patient autonomy”? and so endlessly on). I do not wish to state that deriving measures that have construct validity and an appropriate degree of cardinality is impossible. For most of these items, however, it remains still to be done. But the prime difficulty lies in the combining. The economist’s idea of a social welfare function is not quite what we seek, for its arguments are utilities and utilities derive from preferences. It is far from clear that the expanded set of variables that would meet the aspiration cited above can be limited to people’s preferences alone.

The evidence on the limited range of cost and benefit variables commonly embraced in HTA already poses considerable problems of interpretation: the evidence may be partial, it may be scientifically controversial, it often has high internal validity but low external validity, it may in some important respects be missing, it may not all be of comparable quality, some of it may be highly quantitative and other elements may be qualitative. Some may be based on observational...
studies with poor controls for confounding factors, some may be based on professional and probably biased opinion, much will have unknown and even unknowable biases.

How much more complex is the combining of a much wider set of desired elements. They will not combine themselves to produce health system guidance, instead, combining and interpreting them requires a deliberative process. A deliberative process is participative and often follows a period of consultation with relevant stakeholders. It entails both the eliciting and the combining of various types of evidence in order to reach an evidence-based judgment. There is little evidence on the effectiveness of deliberative processes, though there is much to be said in favor of them on grounds of principle. The design of a deliberative process is not neutral and may well influence the relative weights assigned to different types of evidence, thus influencing the extent to which the eventual guidance is “evidence-based”. Characteristics of a deliberative process likely to ensure evidence-based guidance include consultation with all parties affected by the outcome, fair representation of scientists and stakeholders, high-quality syntheses of the scientific evidence, and skilful chairing.

The way ahead thus seems to me to be one in which our prime focus should be less on refining and expanding our calculus of choice and more on the decision process and the design of processes that enable decision-makers to weigh up entities that are not only measured or indicated with varied precision and unknown biases, and not only incomplete and contestable, but that also belong in different layers of understanding and difficulty of comparison: variables arising from preferences, from notions of duty, from religious observance, from historical circumstance. Variables that may not be continuous but binary, like “good” and “bad”. The focus should not, moreover, be only on process. It should also be on the people who engage in the process. The processes we need are ones in which (unprejudiced) people can exercise an informed judgment. People do not come ready for these tasks. They need training in the testing and interpretation of information and in the exercise of good judgment.

The authors have hardly begun to address this agenda but, if progress can be made in these respects, then the boundaries of CEA and HTA will no longer be set by the limitations of the HTA algorithm but by the limitations of the human imagination. That is to say, they become virtually limitless!

References
1. Teerawattananon Y, Russell S, Mugford M. A systematic review of economic evaluation literature in Thailand: are the data good enough to be used by policy-makers? Pharmacoeconomics 2007; 25: 467-79.
5. Bergson A. A reformulation of certain aspects of welfare economics. Q J Econ 1938; 52, 310-34.