Whither trial-based economic evaluation for health care decision making?

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The randomised controlled trial (RCT) has developed a central role in applied cost-effectiveness studies in health care as the vehicle for analysis. That is, data are collected on all or a sample of patients in the trials on resource use and health effects. This proves the basis of a trial-based estimate of cost-effectiveness of the technology of interest relative to a comparator. Increasingly, economic evaluation is being used as an explicit input into formalised decision-making about new technologies by specific agencies (e.g. NICE in the UK). This paper considers the role of trial-based economic evaluation in this era of explicit decision making. It is argued that any framework of economic analysis can only be judged insofar as it can inform two key decisions and be consistent with the objectives of a health care system subject to its resource constraints. The two decisions are, firstly, whether to adopt a health technology given existing evidence and, secondly, an assessment of whether more evidence is required to support this decision in the future. We argue that a framework of economic analysis is needed which can estimate costs and effects, based on all the available evidence, across the full range of possible alternative interventions and clinical strategies, over a relevant time horizon and for specific patient groups. It must also enable the accumulated evidence to be synthesised in an explicit and transparent way in order to fully represent the decision uncertainty. These requirements suggest that, in most circumstances, the use of a single RCT as a vehicle for economic analysis will be an inadequate and partial basis for decision making. The paper concludes that RCT evidence, with or without economic content, should be viewed as simply one of the sources of evidence which must be placed in a broader framework of evidence synthesis and decision analysis.