VALUE IN HEALTH 18 (2015) 865–875

Available online at www.sciencedirect.com

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journal homepage: www.elsevier.com/locate/jval

Comparative Effectiveness Research/Health Technology Assessment (HTA)

Unifying Research and Reimbursement Decisions: Case Studies Demonstrating the Sequence of Assessment and Judgments Required

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ABSTRACT

Background: The key principles regarding what assessments lead to different types of guidance about the use of health technologies (Only in Research, Approval with Research, Approve, or Reject) provide an explicit and transparent framework for technology appraisal. **Objective:** We aim to demonstrate how these principles and assessments can be applied in practice through the use of a seven-point checklist of assessment. **Methods:** The value of access to a technology and the value of additional evidence are explored through the application of the checklist to the case studies of enhanced external counterpulsation for chronic stable angina and clopidogrel for the management of patients with non–ST-segment elevation acute coronary syndromes. **Results:** The case studies demonstrate the importance of considering 1) the expected cost-effectiveness and population net health effects; 2) the need for evidence and whether the type of research required can be conducted once a technology is approved for widespread use; 3) whether there are sources of uncertainty that cannot be resolved by research but only over time; and 4) whether there are significant (opportunity) costs that once committed by approval cannot be recovered. **Conclusions:** The checklist demonstrates that cost-effectiveness is a necessary but not sufficient condition for approval. Only in Research may be appropriate when a technology is expected to be cost-effective due to significant irrecoverable costs. It is only approval that can be ruled out if a technology is not expected to be cost-effective. Lack of cost-effectiveness is not a necessary or sufficient condition for rejection.

Keywords: cost-effectiveness, coverage with evidence development, health technology assessment, only in research, reimbursement decisions, research decisions.

Introduction

In an effort to stem the rising health care costs, many health systems now require that a new technology demonstrate value (i.e., that the expected additional health benefits of the technology justify its additional costs). In publicly funded health systems such as the UK National Health Service (NHS), this is achieved by comparing the additional health gained from the new technology to the health expected to be forgone elsewhere in the system (opportunity cost, which is often assessed through the use of a cost-effectiveness threshold); that is, the technology is considered cost-effective if it offers positive net health benefits.

Even in health systems in which there is an absence of firm budget constraints or those that do not explicitly consider cost, there is often a focus on the magnitude of health benefits of the technology, which are informally weighed against costs. In this case, the existence of opportunity cost remains but it may manifest in terms of nonhealth expenditure. Therefore, decisions about health care technologies should consider including an assessment of the value of access to the technology, typically relying on evidence about clinical effectiveness, impact of the technology on long-term health and potential harms, costs, and some assessment of the opportunity cost of health that is likely to be forgone if the technology is approved for use.

These evidential requirements present a challenge to such decisions because often decisions are made earlier, shortly after regulatory approval, when the evidence base is least mature. Consequently, the assessment of value is uncertain and subsequent decisions about the use of the technology are likely to be uncertain. For example, approval of the technology may result in resources being wasted if the expected positive net health effects are not realized in practice, whereas rejecting the

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1098-3015$36.00 – see front matter http://dx.doi.org/10.1016/j.jval.2015.05.003

Table 1 – Checklist for coverage with evidence development decisions.

Point	Assessment	Judgment (based on estimates of expected net health benefit)	
	Types of analyses required	Yes	No
1	**Is the technology cost-effective?** • Estimate of expected cost-effectiveness at population level		
2	**Are there significant irrecoverable costs?** • Estimate of capital investment costs, upfront costs of treatment, learning and training costs, other potential irrecoverable costs • Assessment of whether decisions are irreversible • Assessment of whether costs are sufficiently significant to influence guidance		
3	**Does more research seem worthwhile?** • Probability that technology is cost-effective • Estimate of expected consequences of uncertainty		
4*	**Is the research possible with approval?** • What type of evidence is required? • Can the research be conducted if the technology is approved for use?		
5	**Will other sources of uncertainty resolve over time?** • Estimate of changes in the price of technology and comparators, new technology entering, other evidence underway, other potential sources		
6	**Are the benefits of research greater than the costs?** • Estimate of the likelihood that the research will be conducted, how much uncertainty will be resolved, when the results will become available, and the impact of other sources of uncertainty • Estimate of the expected costs of research		
7	**Are the benefits of approval greater than the costs?** • Comparison of the benefits of approval and the opportunity costs (e.g., value of research forgone as a consequence of early access)		

* For technologies not expected to be cost-effective at point 1, point 4 becomes "Is the research possible without approval?"

technology may risk failing to provide access to a valuable intervention if the net health effects prove to be greater than expected. Therefore, the need for and value of additional evidence is an important consideration when making decisions about the use of technologies [1–3]. This is even more critical when approval of a technology for widespread use might reduce the prospects of conducting the type of research that would provide the evidence needed [4]. In these circumstances, there is a trade-off between the net health effects to current patients from early access to the technology and the net health effects to future patients from withholding approval until valuable research has been conducted [5]. In making these trade-offs, consideration should also be given to uncertain events in the near or distant future, which may change the value of the technology and the need for evidence [6].

Generating additional evidence through research also consumes valuable resources that could be devoted to improving health outcomes elsewhere. Importantly, implementing approval of a new technology may commit resources that cannot subsequently be recovered if guidance changes at a later date [7–9]. Therefore, guidance about a technology will depend on whether the benefits of research are likely to exceed the costs of research and whether the benefits of early approval of the technology are expected to be greater than the loss resulting from withholding approval until valuable research is conducted or other sources of uncertainty are resolved. Until recently, decisions in many health care systems have been largely binary (i.e., approval or rejection of the technology). However, new decision options that allow patients early access to promising new technologies while limiting the risks associated with making wrong treatment choices until more evidence is established have emerged. Examples include conditional coverage options such as "Only in Research" (OIR) and "Approval with Research" (AWR) decisions: The former restricts the use of new technology to only those patients who are involved in research, whereas the latter approves the technology for widespread use on the condition that additional evidence to support its continued or expanded use be collected.

A review of different health care systems' policies for coverage decisions linked to evidence development has been presented elsewhere [10]; this review identified a lack of clear guidance on the specific circumstances under which an OIR or AWR scheme may be an appropriate policy option. Therefore, Claxton et al [10] set out to establish the key principles of what assessments are needed to inform OIR and AWR recommendations. The assessments identified fall into four broad areas: 1) expected cost-effectiveness and population net health effects; 2) the need for evidence and whether the type of research required can be conducted if a technology is approved for widespread use; 3) whether there are sources of uncertainty that cannot be resolved by research but only over time; and 4) whether there are significant (opportunity) costs that, once committed by approval, cannot be recovered if guidance were to change at a later date. A conceptual framework and algorithm has been developed that identifies the sequence of assessment and decisions leading to a particular type of guidance (OIR, AWR, Approve, or Reject) regarding the use of health technologies [10].

The sequence of assessment from this algorithm can be summarized using a seven-point checklist (Table 1). A judgment at each point of the checklist (based on estimates of expected net health benefits at each point) leads to a particular type of guidance (see Appendix Table S1 in Supplemental Materials found at: 10.1016/j.jval.2015.05.003 for the complete list of possible pathways). All seven assessments do not necessarily need to be undertaken because sometimes earlier decisions will lead directly to guidance.

The purpose of this article is to demonstrate how these principles and assessments can be applied in practice to inform