Health Technology Assessment 1999; Vol. 3: No. 6 (Executive summary)

Review

Executive summary

Assessing the costs of healthcare technologies in clinical trials

K Johnston[1]
MJ Buxton[1]
DR Jones[2]
R Fitzpatrick[3]

[1] Health Economics Research Group, Brunel University, Uxbridge, UK
[2] Department of Epidemiology and Public Health, University of Leicester, UK
[3] Division of Public Health and Primary Health Care, University of Oxford, UK

Health Technology Assessment
NHS R&D HTA Programme

Executive summary

Background

In the economic evaluation of healthcare technologies, costs are estimated by multiplying the quantities of resources used by the unit costs of the resources. When economic evaluations are conducted alongside clinical trials, the opportunity arises to collect comprehensive and detailed information on resource-use quantities. For example, resource use such as days in hospital can be measured for each individual in the trial. This then allows the estimation of cost data at the individual level, referred to as 'patient-specific' data. The advantage of such data is that it allows statistical analysis of costs to be performed.

There is, however, a legitimate concern not to overburden the trial data collection process with the gathering of such detailed resource-use information. Consequently, the choice of resource-use items for data collection needs to be considered very carefully. This report identifies and examines the range of methodological issues concerning the collection of resource-use data for costing purposes and its analysis.

Objectives

The overarching objective is to challenge investigators to think through their study design in order to collect appropriate resource-use information in the most efficient way. Specifically, the objectives are:

- to identify methodological issues concerning the collection of resource-use data for costing purposes and its analysis
- to classify methodological issues into: (1) those where there is general agreement about how they should be handled; (2) those remaining open because of legitimate differences in values or perspectives; and (3) those where further empirical testing could resolve how the issue should be handled
- to demonstrate how existing data can be used to inform the design of costing studies in trials
- to develop a framework or decision aid within which decisions about costing in specific trials can be made.

Methods

The methodological issues were identified through a review of several strands of relevant literature, including methodological review articles, empirical articles and guidelines on performing economic evaluations. In developing the review, comments from relevant experts were sought with the aim of identifying further issues and opinions. The methodological issues identified are structured under four broad headings:

- study design
- data collection
- data analysis
- presentation of results.

The two final objectives listed above are achieved through empirical analysis and the development of a framework or decision aid, respectively. Further detail on the methods is presented in the main report.

Results

Design issues address the types of cost to be included, such as health service, trial, future and productivity costs. The decision on which types of cost to include depends on seven key factors:

- possible links to economic welfare theory
- the perspective to be adopted
- the form of economic evaluation
- the avoidance of double counting
- the quantitative importance of the type of cost
- whether the cost can be attributed to the intervention
- the time horizon of the study.

The collection of detailed data on resource use for all patients may not be necessary; key cost-generating events can be measured. These can be defined as where there is variation in the frequency of events between arms of the trial or between patients within arms. Determining sample sizes for detecting differences in costs or cost-effectiveness involves identifying an economically important difference and having information on the variability of cost data from previous studies or

from pilot studies. A further sampling issue to be addressed in multicentre trials is the selection of centres and whether resource-use and unit cost information should be collected from all centres.

Data collection issues involve deciding on the appropriate resource-use data collection method. Resource-use data can be measured on a patient-specific basis by using, for example, interviews, questionnaires, case record forms or diary cards. In selecting a method, potential sources of bias have to be addressed, including recall bias, evasive answer bias, non-response bias, selection bias and question format. The validity and reliability of resource-use data collection methods have not been tested fully and are therefore not reported in the literature.

Data analysis may also influence the design of the study. In summarising and synthesising cost data, issues such as how to pool data and how to handle missing and censored data have to be addressed. It is important to take into account the variability in cost data and its distribution. It is generally agreed that mean costs convey more useful information than medians because they relate to total cost. The methods used to address uncertainty in methods and results include both statistical and sensitivity analyses; these have complementary roles. Sensitivity analysis can also be used to generalise results.

The presentation of results addresses reporting formats. Results should be presented in a disaggregated manner, for example, by separating resource use from unit costs and reporting the contribution of different types of cost to total costs. The development of a common reporting format for economic evaluations would increase the transparency of both methods and results. The design of future studies relies on transparent reporting in earlier studies so that issues such as the variability in cost data can be determined.

There are two additional elements of the review. First, an existing data set on costing from a clinical trial was used to illustrate how evidence relating to costs from a completed study can be used to inform the design of data for costing. By examining the results of detailed data collection, the exercise illustrates that, in the example at least, it is possible for simpler data collection methods to be adopted to produce comparable results. The exercise demonstrates the usefulness of having access to, and analysing, existing data sets in order to address design issues.

Secondly, a decision aid, or structured framework, has been developed within which decisions can be made about designing a costing study alongside a clinical trial. In effect, the decision aid requires answering a set of explicit questions. It is recommended that it should be tested in future studies.

Conclusions

Methodological issues on which there is general agreement include identifying perspective, measuring units of resource use, and applying appropriate unit cost. Those issues remaining open because of legitimate differences in values or perspectives concern which perspective to adopt and whether to base decisions on economic welfare theory. Finally, methodological issues requiring further empirical study include:

- exploring optimal sampling approaches
- questions surrounding multicentre clinical trials
- testing the validity and reliability of resource-use data collection methods
- handling missing and censored data
- methods used to generalise results.

By presenting issues in this way, the review recognises the inevitability of some issues remaining unresolved while at the same time allowing the specification of a future research agenda.

Recommendations

Four sets of recommendations are provided, for: investigators, funding bodies, those responsible for ensuring high standards in reporting of studies, and further research. The review and its associated appendices serve to challenge investigators to think through methodological issues and to decide how best they can be handled in their own circumstances. For those issues requiring further empirical investigation, researchers should build empirical testing into their studies. In this way, methodological standards in the next generation of studies can be improved, and the future research necessary to develop further and refine methodology can be undertaken. In the short term, however, the review will provide users of currently available studies with information having a critical basis against which to assess the cost information presented.

Publication

Johnston K, Buxton MJ, Jones DR, Fitzpatrick R. Assessing the costs of healthcare technologies in clinical trials. *Health Technol Assess* 1999;**3**(6).

NHS R&D HTA Programme

The overall aim of the NHS R&D Health Technology Assessment (HTA) programme is to ensure that high-quality research information on the costs, effectiveness and broader impact of health technologies is produced in the most efficient way for those who use, manage and work in the NHS. Research is undertaken in those areas where the evidence will lead to the greatest benefits to patients, either through improved patient outcomes or the most efficient use of NHS resources.

The Standing Group on Health Technology advises on national priorities for health technology assessment. Six advisory panels assist the Standing Group in identifying and prioritising projects. These priorities are then considered by the HTA Commissioning Board supported by the National Coordinating Centre for HTA (NCCHTA).

This report is one of a series covering acute care, diagnostics and imaging, methodology, pharmaceuticals, population screening, and primary and community care. It was identified as a priority by the Methodology Panel and funded as project number 93/48/05.

The views expressed in this publication are those of the authors and not necessarily those of the Standing Group, the Commissioning Board, the Panel members or the Department of Health. The editors wish to emphasise that funding and publication of this research by the NHS should not be taken as implicit support for the recommendations for policy contained herein. In particular, policy options in the area of screening will be considered by the National Screening Committee. This Committee, chaired by the Chief Medical Officer, will take into account the views expressed here, further available evidence and other relevant considerations.

Reviews in *Health Technology Assessment* are termed 'systematic' when the account of the search, appraisal and synthesis methods (to minimise biases and random errors) would, in theory, permit the replication of the review by others.

The editors have tried to ensure the accuracy of this report but cannot accept responsibility for any errors or omissions. They would like to thank the referees for their constructive comments on the draft document.

Copies of this report can be obtained from:

The National Coordinating Centre for Health Technology Assessment,
Mailpoint 728, Boldrewood,
University of Southampton,
Southampton, SO16 7PX, UK.
Fax: +44 (0) 1703 595 639 Email: hta@soton.ac.uk
http://www.soton.ac.uk/~hta ISSN 1366-5278