

Executive summary

An introduction to statistical methods for health technology assessment

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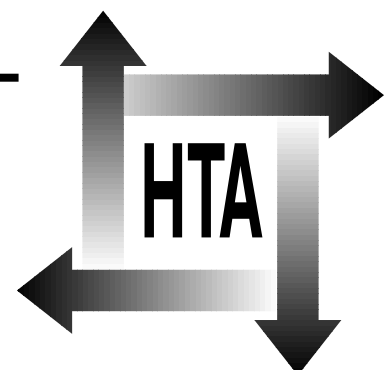
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**Health Technology Assessment
NHS R&D HTA Programme**





Executive summary

Objectives

The ability of the Health Technology Assessment (HTA) programme to answer questions about the effectiveness and cost-effectiveness of new technologies relies on the availability of appropriate methodologies including statistics. The aims of this report were to:

- document recommended practice in relevant and related areas
- document current practice critically
- map current methodological research
- identify areas relevant to health technology assessment where statistical methodology is either inadequate or not being employed to full advantage, and
- identify suitable areas for further research.

Methods

To meet these objectives a series of linked reviews were undertaken. These were of:

- textbooks used in the training of medical statisticians on three MSc courses
- guidelines covering statistical aspects of evaluation of technologies
- publications appearing in the statistical literature during 1994–95
- publications on various study designs using MEDLINE for 1993–96
- publications on methods for analysis of follow-up studies using MEDLINE for 1993–96
- the needs of the HTA programme as evidenced by current work.

Findings

Statistical training

The review of textbooks from MSc courses showed that students are being offered courses in statistical theory and methods, design of experiments, linear models and generalised linear models, survival analysis, repeated measures, spatial statistics, multivariate methods, multilevel models, distribution-free statistics, Bayesian inference and methods,

measurement errors, computational statistics, clinical trials and epidemiology. This represents a wider range than any one person can learn in a year. Much is relevant to health technology assessment, but the links are not yet very explicit, and there are no directly relevant textbooks recommended.

Statistical guidelines

Statistical guidelines have been developed in areas relevant to health technology assessment, in particular drug regulation, and in systematic reviews of randomised trials, through the Cochrane Collaboration. The linchpin technology in both of these areas is the randomised controlled trial. However, they mostly emphasise principles and ways of working rather than detail, with only meta-analysis covered in depth.

Publications

A review of the papers potentially relevant to health technology assessment, published in statistical journals in 1994–95 yielded 505 papers. These were predominantly about new methodology rather than discussion or review papers, mainly used classical rather than Bayesian approaches, and largely used re-analysed or simulated data, rather than primary analyses. Most related to preclinical or clinical trials rather than other kinds of studies.

Study designs

Much of the statistical literature on study designs that relate to health technology assessment comes from clinical trials; there are relatively few publications that cover the more complex experimental designs, meta-analysis or studies of drug safety. Within the medical literature, of the more complex experimental designs, (bio-)equivalence and crossover studies can be identified but not other designs in large numbers.

Methods for analysis

Of the statistical literature relevant to health technology assessment on analysis of follow-up studies, both survival and longitudinal data feature regularly, and repeated measures (non-longitudinal) occur less often. Within the medical literature, survival analysis is extremely common, particularly proportional hazards and Cox regression. Much of this work is in the context of cancer and heart disease. Identifying longitudinal studies

in the medical literature is straightforward, and they cover a range of conditions, but identifying the use of longitudinal methods of analysis is much harder.

Needs of the HTA programme

In health technology assessment the question 'Does the technology work?' is most easily answered using standard statistical methods. 'For whom?' raises statistical questions of subgroup analysis and interactions, and wider questions of generalisability. 'At what cost?' raises questions of identification and measurement of costs, with appropriate handling of associated uncertainty. 'How does it compare with alternatives?' brings a need for more formal decision analysis, and revisiting work on complex experimental design.

Recommendations

- The NHS R&D programme could consider training strategies for continuing professional development of statisticians, for example by allocating a fund to allow attendance of relevant courses.
- The NHS R&D programme could consider commissioning induction courses for statisticians working in health technology assessment. The purpose of such courses would partly be to give an introduction to health technology assessment

and associated disciplines, and partly to focus on reinforcing statistical methods particularly pertinent to health technology assessment.

- Researchers in health technology assessment could avail themselves of existing guidelines, specifically those in drug regulation, and, if involved in meta-analysis, those of the Cochrane Collaboration.
- The NHS R&D programme could sponsor workshops to bring together statisticians and others who have been working in health technology assessment to identify and develop future statistical issues in health technology assessment.
- Case studies are needed on decision making under uncertainty using established Bayesian methodology to integrate health outcomes with wider costs.
- Established statistical methodology on design of experiments is potentially relevant to complex questions in health technology assessment. The development of specimen protocols explicitly using such methodology could be commissioned.

Publication

White SJ, Ashby D, Brown PJ. An introduction to statistical methods for health technology assessment. *Health Technol Assess* 2000;4(8).

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 Group and funded as project number 93/50/02.

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.

Criteria for inclusion in the HTA monograph series

Reports are published in the HTA monograph series if (1) they have resulted from work either prioritised by the Standing Group on Health Technology, or otherwise commissioned for the HTA Programme, and (2) they are of a sufficiently high scientific quality as assessed by the referees and editors.

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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.

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