

Session Title: Cost-effectiveness thresholds for decision making about health care technologies: conceptual and empirical underpinnings

Chair : Mark Sculpher (University of York)

Organizer (1): Mark Sculpher (University of York)

Session Description (259 words)

Many jurisdictions use cost-effectiveness analysis to support decision making regarding the reimbursement, coverage or funding of new medical technologies; some systems use such methods to determine whether to continue a commitment to an existing intervention. Any decisions based on such methods require an estimate of the threshold above which the costs of improving health outcomes are considered prohibitively high. Although some decision making authorities are becoming more explicit about the threshold they employ, this is rarely based on empirical analysis. It is likely that organisations will be increasingly pressurised to be more explicit about the underlying conceptualisation and derivation of the thresholds they employ, but the appropriate methods for doing so are contested.

Some have argued that, in a budget constrained health care system, the cost-effectiveness threshold should reflect the health forgone as services are displaced by new technologies imposing additional costs (e.g. NICE). Others have suggested that its value should represent the willingness to pay for health improvement of those ultimately funding the system (e.g. tax or insurance payers). Some have suggested that the threshold should be inferred from previous decisions within specific clinical areas (e.g. IQWiG). Finally, interventions could be compared across sectors beyond health care (e.g. with education, transportation, defence). The chair and panellists have extensive experience working with decision making authorities in Europe and elsewhere. The session will begin with an overview of the cost-effectiveness threshold, its role and the alternative approaches to derivation. Three Panellists will debate the appropriate approach to characterising and estimating the cost-effectiveness threshold in the context of resource allocation decisions.

Key Terms: cost effectiveness analysis, cost effectiveness threshold, economic evaluation

Presenter: Mark Sculpher (University of York. Centre for Health Economics)

Abstract (313 words)

Mark Sculpher will chair the session. He will begin with an overview of the alternative ways in which the cost-effectiveness threshold has been characterised in the literature and the

implications of this for its empirical estimation. He will also review how the threshold has been described in international methods guidelines of decision making organisations. Mark Sculpher is Professor of Health Economics at the Centre for Health Economics, University of York, UK where he is Director of the Programme on Economic Evaluation and Health Technology Assessment. Mark has worked in the field of economic evaluation and health technology assessment for over 20 years. He has researched in a range of clinical areas including heart disease and various types of cancer. He has also contributed to methods in the field, in particular relating to decision analytic modelling and techniques to handle uncertainty. He has over 170 peer-reviewed publications and is a co-author of two major text books in the area: *Methods for the Economic Evaluation of Health Care Programmes* (OUP, 2005 with Drummond, Torrance, O'Brien and Stoddart) and *Decision Modelling for Health Economic Evaluation* (OUP, 2006 with Briggs and Claxton). Mark is a member of the UK National Institute of Health Research (NIHR) College of Senior Investigators. He has also been a member of the National Institute for Health and Clinical Excellence (NICE) Technology Appraisal Committee and the NICE Public Health Interventions Advisory Committee. He currently sits on NICE's Diagnostics Advisory Committee. He chaired NICE's 2004 Task Group on methods guidance for economic evaluation and advised the Methods Working Party for the 2008 update of this guidance; he has also advised health systems internationally on HTA methods including those in Ireland, Germany, Portugal and New Zealand. He has been a member of the Commissioning Board for the UK NHS Health Technology Assessment Programme and currently sits on the UK NIHR/Medical Research Council's Methodology Research Panel.

Key Terms: cost effectiveness analysis, cost effectiveness threshold, economic evaluation

Author (1): Mark Sculpher (University of York. Centre for Health Economics)

Presenter: Karl Claxton (University of York. Centre for Health Economics and Department of Economics)

Abstract (261 words)

Claxton will advocate the opportunity cost approach as a means of estimating the threshold and discuss ongoing work in the UK. He will report on the UK MRC Methodology Research Programme funded research to establish appropriate methods to estimate the NHS threshold based on routinely available sources of data. Karl Claxton is a Professor in the Department of Economics and the Centre for Health Economics at the University of York. He was a Harkness Fellow at the Harvard School of Public Health and, until 2007, he held an adjunct appointment at Harvard as an Assistant Professor of Health and Decision Sciences. His research interests encompass the methods for the evaluation of health care technologies. He has served as a member of the National Institute for Health and Clinical Excellence Appraisal Committee from 1999 to 2010 and, more recently, as a member of the newly formed Medical Technologies Appraisal Committee. He has represented the committee at a number of appeals and was an expert witness for the Institute during judicial review. He is also a member of the National Decision Support Unit and continues to contribute to the development of the NICE Guide to the Methods of Technology Appraisal. He has contributed in a number of ways to recent policy debates such as pharmaceutical pricing and innovation, including as an expert witness at the House of Common Health Select Committee and the Kennedy review of

Innovation in Health Technology. As well as NICE he has also advised, Department of Health, HM Treasury, Department of Innovation and Skills and Office of Life Sciences.

Key Terms: cost effectiveness analysis, cost effectiveness threshold, economic evaluation

Author (1): Karl Claxton (University of York. Centre for Health Economics and Department of Economics)

Presenter: Adrian Towse (Office of Health Economics)

Abstract (257 words)

Towse will argue that a broader societal perspective, including willingness to pay, needs to inform the threshold estimates. Working from first principles of an individual's willingness to buy health care (the individual cost-per-QALY threshold) through health insurance schemes based on representative consumers, he will look at how heterogeneity of consumer and patient preferences could be accommodated in competing and single-payer systems. He will also explore how opportunity cost concepts should be efficiently applied in a fixed global budget health care system. Adrian Towse is the Director of the Office of Health Economics. The OHE commissions research, publications and meetings on the economics of the pharmaceutical industry, health care systems and the use of health technology assessment. It also advises the ABPI (who provide research funding to the OHE) and undertakes research and consultancy for the public and private sectors in the UK and internationally. He is a Visiting Professor at the University of York and a visiting Senior Researcher at the Department of Public Health and Primary Care at the University of Oxford. He served for ten years as Non-executive Director of the Oxford Radcliffe Hospitals NHS Trust, one of the UK's largest hospitals, where he chaired its Governance Committee. His current research interests include the use of "risk-sharing" arrangements between health care payers and pharmaceutical companies; the economics of pharmacogenetics for health care payers and the pharmaceutical industry; economic issues around access to, and R&D for the development of, treatments for less developed country diseases; the economics of medical negligence; and measuring productivity in health care.

Key Terms: cost effectiveness analysis, cost effectiveness threshold, economic evaluation

Author (1): Adrian Towse (Office of Health Economics)

Presenter: Jaime Caro (United BioSource Corporation). [Dr Caro was unable to attend so he was replaced by Dr Andreas Gerber (IQWiG, Germany).]

Abstract (339 words)

Caro will discuss the IQWiG proposals for threshold estimation and argue that a general threshold is unnecessary and not well supported by theory. He will argue that efficiency does have a role in priority-setting but only in therapeutic areas where there are alternatives. This talk will present a needs-based approach to value-based pricing, which avoids the need for a

threshold, together with the proper role of efficiency and how unnecessary the QALY is. Jaime Caro is an expert in advanced modeling techniques used to inform complex health care decisions. Dr. Caro trained at McGill University, where he practiced internal medicine and is now Adjunct Professor of Medicine, and of Epidemiology and Biostatistics. He has established the graduate-level course in pharmacoeconomics at the University and also lends his teaching ability to other academic institutions, government organizations, and professional associations such as the International Society for Pharmacoeconomics and Outcomes Research (ISPOR). After founding and leading the Caro Research Institute for more than a decade, he is now Senior Vice President for Health Economics at United BioSource Corporation. His initial work in theoretical epidemiology led to applied research for the Health Technology Assessment Council of Quebec, a pioneer in the then nascent field. This early interest in the proper approach to evaluating health care interventions has resulted in efforts to help government organizations in countries as diverse as Germany and Colombia develop and implement their Methodologies; and to appointments on the steering committees of various global initiatives to address specific diseases. In addition to Dr. Caro's work and publications on more than 100 disease models, he has continued active research on methods for modeling diseases and the application of epidemiologic concepts to economic studies. This has included the development of approaches for dealing with complex time dependencies and the transfer of modeling techniques from other fields to medicine—modifying discrete event simulation for use in economic and risk-benefit analyses and the modeling of adaptive clinical trials. More recently, he has focused on the development of a suitable metric to guide health care decisions.

Key Terms: cost effectiveness analysis, cost effectiveness threshold, economic evaluation

Author (1): Jaime Caro (United BioSource Corporation)