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Economic evaluation and EBM

Glenn P. Salkeld
University of Wollongong, gsalkeld@uow.edu.au

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Abstract

In the world of textbook economics, the "sovereign" consumer weighs up the (freely available) evidence on the costs, risks, harms and benefits before purchasing health care. The value that consumers then attach to the evidence and the expected outcomes is revealed through their purchasing decisions in the market. Ultimately, the consumer's decision represents the best or benefit maximising choice given the available information. The notion of this evidence-based market is however a long way from the reality of health care in Australia. Consumers (that is, patients) generally do not have current best evidence to hand. The same could be said of their agent (doctor) prior to worldwide interest in evidence-based medicine (especially through the Cochrane Collaboration). If the market is not capable of integrating external clinical evidence from systematic research and clinical expertise such that consumers (or their agents for that matter) can assess the quality of information easily then a mechanism is needed to perform that function. One such mechanism is economic evaluation. This approach describes a set of techniques, such as cost-effectiveness analysis and cost-benefit analysis, that require the systematic comparison of the costs and benefits of the full range of health care activities. Economic evaluation performs what individual consumers would otherwise do in a competitive market; it weighs up the costs and benefits of the available choices. That still leaves many questions about whose values count in the aggregation of costs and benefits and whether the value of the total is greater than the sum of individual values. Nonetheless, if one of the aims of a health care system is to be efficient, then choosing those programs that provide the greatest benefits for the resources available will delivery an efficient allocation of health care resources. Allocating health care resources is seldom simply a matter of choosing efficient programs; the 'fairness' or equity of resource allocation is also a desirable economic goal. The aim of this paper is to provide a brief account of what economic evaluation has achieved and could achieve in cancer control within an EBM environment. The first part looks at funding lor health services based on evidence of economic evaluation. The following section of the paper highlights some innovative research into the use of EBM to elicit consumer preferences for colorectal cancer screening.

Keywords

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ECONOMIC EVALUATION AND EBM

G Salkeld

Social and Public Health Economics Research Group, Department of Public Health and Community Medicine, University of Sydney, Sydney, NSW.
Email: glenns@pub.health.usyd.edu.au

Introduction

In the world of textbook economics, the "sovereign" consumer weighs up the (freely available) evidence on the costs, risks, harms and benefits before purchasing health care. The value that consumers then attach to the evidence and the expected outcomes is revealed through their purchasing decisions in the market. Ultimately, the consumer's decision represents the best or benefit maximising choice given the available information. The notion of this evidence-based market is however a long way from the reality of health care in Australia. Consumers (that is, patients) generally do not have current best evidence to hand. The same could be said of their agent (doctor) prior to worldwide interest in evidence-based medicine (especially through the Cochrane Collaboration). If the market is not capable of integrating external clinical evidence from systematic research and clinical expertise such that consumers (or their agents for that matter) can assess the quality of information easily then a mechanism is needed to perform that function. One such mechanism is economic evaluation.

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The aim of this paper is to provide a brief account of what economic evaluation has achieved and could achieve in cancer control within an EBM environment. The first part looks at funding for health services based on evidence of economic evaluation. The following section of the paper highlights some innovative research into the use of EBM to elicit consumer preferences for colorectal cancer screening.

Funding for Success

If EBM focuses on the use of "current best evidence in making decisions about the care of individual patients"[2] then economic evaluation tends to focus on the use of current best evidence about resource allocation for groups of patients. Such evaluation is relevant to the care of individual patients but that is not where it has been most successful. Success has come more readily at a systems wide level. For example, in 1987, the *National Health Act, 1953*, was amended to require the Pharmaceutical Benefits Advisory Committee (PBAC) to 'take account of comparative effectiveness and cost in recommending drugs as pharmaceutical benefits . . .'[3]. Although the legislation did not specify how this was to be achieved, in practice it happened within an EBM framework.[4-5] For the first time in the world, government funding for the reimbursement of drugs depended on a systematic review of all relevant evidence of acceptable scientific rigour. The Pharmaceutical Evaluation Scheme (PES) is supported by an institutional structure that critically appraises each cost-effectiveness submission. The effect of this scheme over the last six years has been to reward drugs that demonstrate clinical superiority with a higher price than the comparator drug. There is a very clear incentive for drug companies to pursue research and development of drugs that do offer a clinical advantage over current therapy and then to evaluate their cost-effectiveness in a systematic and scientifically rigorous manner.

In the area of cancer drugs, the emergence of Interferon (IFN) as an anticancer agent predates the PES but the potential gains to be made from the proper exploitation of IFN are great. But so too are the costs of unrestricted and inefficient use of IFN. The EBM framework for the reimbursement of pharmaceuticals allows drugs such as IFN to be subjected to proper economic scrutiny.[6]

Using Evidence to elicit Consumer Preferences for Cancer Screening and Cancer Treatment

In December 1996, The Clinical Oncological Society of Australia (COSA) and the Australian Cancer Network (ACN) auspiced a process to develop evidence-based guidelines for the prevention, detection and management of colorectal cancer (CRC).[7] Guidelines are a necessary step in improving medical decision making. The question then arises, how do patients and citizens best use evidence-based information for their own treatment and screening choices? Interest in measuring patient preferences, patient participation in screening and treatment choices, the use of decision aids and communication of information between doctor and patient has grown with the worldwide interest in EBM. In conjunction with my colleagues Jeanette Ward, Michael Solomon and Leonie Short, I am conducting a study to elicit consumer preferences for colorectal cancer screening. A measurement technique, known as discrete choice modelling, is being used to provide a quantitative estimate of which factors of CRC screening and its outcomes matter most to citizens and by how much.[8-9] The technique presents an individual with a series of pairwise choices, each offering different combinations of harms and benefits.[10] An example of a typical scenario is presented in Figure 1.

Q1. Could you please compare the two programs and tell me whether you would prefer Program A, Program B or No Screening?

Example Scenario	Program A	Program B	No Screening
Number of bowel cancer deaths prevented	3	14	0
Number of unnecessary colonoscopies	900	11,200	0
Notification of a negative test result	Yes	Yes	–
		(please tick one box)	
Which would you prefer?	Prefer A ☐	Prefer B ☐	No Screening ☐

Figure 1

In this example, the respondent has been told that the questions are based on a CRC screening program where 10,000 men and women aged 50-69 years have a faecal occult blood test every second year for 10 years. In Figure 1, the subject is being asked to trade-off an extra 9 bowel cancer deaths averted for an extra 10,300 colonoscopies (due to a false positive result). Prior to being offered the choice, subjects are given a lay description of what is involved in the screening process and in having a colonoscopy. By altering the level of harms and benefits in subsequent choice questions, a point is reached where the respondent is indifferent to a combination of harms and benefits. The process of trading involves weighing up the evidence presented in each of the scenarios. The information contained in each of the scenarios is based on the mean value and 95% confidence limits for the harms and benefits as reported in the Nottingham and Funen trials of biennial CRC screening.[11-12] In this way, individuals are being asked to weigh up the best available external evidence on screening. The next stage of the project will introduce cost as a choice variable in screening.

Conclusion

The application of economic evaluation within a clinical/EBM framework has been most successful at the system wide level. It is encouraging to see that the principles of economic evaluation will be incorporated into the activities of the new Medicare Services Advisory Committee. However, the impact of economic evaluation on clinicians making decisions about the care of individual patients or on people being offered cancer screening services is less than impressive. New approaches such as discrete choice modelling are needed to help consumers weigh up the costs, risk, harms and benefits of screening or treatment options so that the consumer can make the best (evidence-based) choice.

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PSYCHOSOCIAL CARE AND SUPPORT FOR CANCER PATIENTS

P Butow

Medical Psychology Unit, University of Sydney and Department of Psychological Medicine, Royal North Shore Hospital, Sydney, NSW

The diagnosis and treatment of cancer can present a major challenge to patients both in the short and long-term, and anxiety and depression are not uncommon responses. Reported prevalence rates of psychological distress in cancer patients range from 20-66%[1], in comparison to a 5.8% prevalence rate of depression in the general population, estimated in the US[2]. A considerable literature has developed addressing questions concerning optimal methods to prevent and ameliorate this distress.

Studies on psychosocial care and support for cancer patients have tended to focus on either (i) the provision of support and counselling or (ii) the provision of information and facilitation of decision-making[3]. Psychosocial clinical practice guidelines encompassing both these areas have recently been developed by the National Breast Cancer Centre, and these are currently being reviewed under the auspices of the NHMRC. While focusing on breast cancer, much of the content of these guidelines is generalisable to the wider cancer context.

Support by both the treatment team and specialist providers, such as psychiatrists, psychologists and social workers, has been widely studied. There are now a number of meta-analyses of randomised controlled trials (RCTs) in this area showing that psychological interventions improve the well-being of cancer patients. For example, in a meta-analysis of 45 RCTs with adults with cancer, those receiving psychological therapies had on average a significant improvement of 12% in emotional adjustment, 10% in social functioning, 15% in treatment- and disease-related symptoms and 14% in overall improvement in their quality of life, compared to those not receiving psychological therapy[4].

Greater effects have been observed when therapies were provided over longer periods, and conducted by more highly trained therapists, such as a specially trained counsellor, nurse, psychologist or social worker[5]. However, few differences have been observed between different types of therapy (such as cognitive-behavioural therapy, family and/or couple therapy, or psycho-educational therapy) or formats of therapy (such as group or individual sessions), suggesting that features common to all psychosocial therapies, such as empathy, listening, affirmation and reassurance, have the greatest impact.

More controversially, some naturalistic, prospective studies have demonstrated an association between patient coping style and length of survival[6], and a small number of randomised controlled trials have produced a higher level of evidence for an association between psychosocial factors and outcome. Speigel[7] reported that women with metastatic breast cancer randomised to a psychosocial intervention group survived for twice as long as those receiving standard treatment, while Fawzy et al[8] reported significant changes in immunological