

## **Technology and Outcomes Assessment: a view from both sides of the Atlantic**

### **Commentary on:**

**Skinner J, Chandra A, Fisher E. The Role of Outcomes Assessment in Health Care Reform**

**Pearson S. Health Technology Assessment and Comparative Effectiveness: Recommendations for Improving Health Care Value in the United States**

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### Introduction

The backdrop to the workshop was recognition that the high and rising cost of health care is a growing threat to the stability of the US health care system. Health care insurance premiums have increased by 54% over a 5 year period, and Ken Thorpe and colleagues<sup>i</sup> have estimated that approximately 63% of the increased spending is linked to “treated disease prevalence” with the remaining 37% linked to rise in spending for “treated cases.” Thus the introduction of new health care technologies (i.e. drugs, devices and medical procedures) is a major factor contributing to health expenditure growth in this and other countries. The focus for the papers by Skinner et al and Pearson is, appropriately, then technology coverage policy; i.e., decisions by health care organisations to adopt or recommend for use a new drug, device or therapy. Coverage can be understood to involve setting limits on available health care services<sup>ii</sup>. Although historically there has not been much of an appetite in US health care for explicitly setting limits on services and technologies, a recent report by Neumann et al to AHRQ suggests that US decision makers are increasingly open to this direction<sup>iii</sup>. However, Neumann along with many others has noted a number of cultural and system-related barriers that will need to be overcome to move forward.

In the UK, where coverage decisions are made in the context of a health care system that is predominately tax-payer financed with explicit ‘cash constraints’, limit-setting has long been part of the territory. At the national level this responsibility is now largely seated at the National Institute for Health & Clinical Excellence (NICE). Established in 1999, NICE is an independent entity within the National Health Service (NHS) charged with appraising new and existing health technologies. Technology appraisal ‘guidance’ from NICE is issued to the NHS and is mandatory – if a technology is not approved for use by NICE then it will not be available as part of NHS care. Coverage decisions made by NICE are based on explicit criteria and are informed by an independent assessment of evidence, including a cost-effectiveness analysis (CEA). Evidence is considered and interpreted by Technology Appraisal Committees that formulate recommendations to the Institute on the use of the technology.

This response to the two papers draws on our experience of NICE and UK policy in this area, and our work with decision makers and the public in the US.

## Comments on Skinner et al

Skinner et al are champions of “outcomes research” as a vital component of reforming US health care. They argue that in addition to building “technology assessment” more emphasis need be placed on monitoring the efficiency of the health care system overall. In their paper, they provide a rather compelling example of cost-effectiveness analysis conducted at the program level looking at survival from, and expenses related to, treatment of acute myocardial infarction at several different hospitals. They suggest that outcomes assessment may be more acceptable and provide greater information on balance than technology assessment does. This is because health care is ultimately a local event, dependent on a complex interaction of institutions, providers, the characteristics of the populations served, and a wide panoply of treatment options. Therefore, what is demonstrated in clinical trials may break down at the level of service delivery, robbing technology assessment of some of its promise. They argue also that progress in accepting technology assessment has been slow, and may remain so, because it is more often couched in terms of limit setting, and meets resistance because of that. The authors believe that institutions in the US are neither willing nor able to make the hard decisions about what should be paid for.

However, there are examples of US institutions that do make coverage decisions explicitly, with due consideration of costs and CEA. An example of this is the VA<sup>iv</sup>, a US health care system that fails to feature prominently in either paper despite its numerous successes.<sup>v</sup>

The authors also argue that a prominent role for CEA in US health care is lacking because of the public’s unwillingness to accept limit setting in health care on the basis of CEA. They suggest that the literature reveals that “voters ... just don’t agree with the guiding principle of cost-effectiveness analysis.” Although the empirical base here is limited, recent testing of this perception amongst Californians and New Yorkers suggests that members of the public may be more open to this view that is commonly promoted<sup>vi</sup>.

Skinner et al also highlight methodology weaknesses in CEA, including their concerns with the lack of generalisability of results from clinical trials to ‘real world’ settings. Methodology advances in CEA over recent years have been focussed on addressing the very points raised by Skinner et al. It is undoubtedly true that procedures found to be highly cost-effective in academic medical centres may not be so in community hospitals. However, as Pearson points out in his paper, CEA is increasingly turning toward decision analytic modelling as a platform for analysis with data drawn both from trials (especially in relation to relative effect) and non-trial (i.e. real-world) settings too. Thus, it would be a mistake to suggest that the limitations of clinical trials are necessarily limitations of CEA or technology assessment.

The central and most powerful message of the Skinner et al paper is that the value of a given technology is not inherent in the drug or device but is tied to who performs the procedure and the characteristics of the patient who receives it. To which we say ‘Quite right!’ but the implication of this is not that CEA should be rejected but that CEA must be able more effectively to consider these issues rather than focus

exclusively on technologies. We wholeheartedly agree that “Differences in spending aren’t due to ‘what’ is provided (angioplasty vs. aspirin), but ‘how’ (the labor and associated services that are bundled with it in higher cost systems).” And this can and should be captured in a good CEA.

The paper concludes with the vision of “comprehensive technology assessment aligned with a rigorous program of provider performance measurement based on longitudinal outcomes and costs”. We share with the authors the view that this offers an intriguing approach and would represent a major shift forward if it could be achieved. This is far from what occurs now in the US and the provider performance aspect is not well established in the UK. We see this as a highly promising area for development.

### Comments on Pearson

We agree with a great deal of what is argued by Pearson, with some important exceptions to which we will return.

One of the central issues highlighted by Pearson is that US decision makers have no framework within which costs and cost-effectiveness analysis can explicitly and legitimately be discussed. Few would disagree with this statement but the challenge lies in how change in this situation might be achieved. The paper provides us less of a roadmap in this respect. It is key to recognise that the problem cannot effectively be addressed simply by providing more and better technology assessment; the organisational context of health care in the US and the workings of health care entities needs also to be reconsidered.

The pluralistic nature of US health care is very different to the situation in the UK and as Pearson acknowledges some of the lessons of the UK system are not freely translated. The exception to this would be components of US health care where there are strong parallels with the UK NHS. These would include Medicare, given its tax-funded (single payer) base, and the VA health care system, given both its tax-funding and its delivery mechanism.

The NICE-NHS solution to ensuring use of CEA and implementation of resulting policy has been to make its guidance mandatory – implementation of NICE ‘guidance’ happens because it is a legal requirement. The flip side of this is that the true local opportunity cost (i.e. the value of services that are sacrificed to facilitate implementation of NICE guidance) is not considered when the coverage decision is taken. An attempt is made to manage this problem by using a cost-effectiveness threshold indicating ‘good value’ (typically between £20,000 and £30,000 per QALY gained). However, there is no theoretical basis for this threshold, and local health authorities<sup>vii</sup> and academics have been vocal and persuasive in pointing out the difficulties associated with such new mandates for coverage, in a budget that is fixed and has pressures on it to deliver other valued services in a timely fashion<sup>viii</sup>. This issue of opportunity costs associated with adding new technologies while maintaining the status quo is likely to have relevance within any institution’s constrained budget. In a managed care organization, purchasers (employers and consumers) will wish to keep premiums affordable; in Medicare, tax payers (employers and the public) may balk as taxes become too high.

We will now consider Pearson's recommendations in turn.

*Recommendation 1: Improve rigour and consistency of evidence base*

Moves to improve the rigour, consistency and transparency of pretty much anything will gain widespread support. A great deal of work has been done in this area already by a number of different entities, and we would urge that there not be an over-emphasis on reinventing the wheel. Rather we think that harmonization of practice can occur through a federally convened consensus panel of expert participants that have established track records in evidence-based reviews. Although we agree that the framework needs to make sense to both developers and users of the reviews, we believe that the commercial sector should serve in a consultative, rather than in a decision making mode. We return to this later.

*Recommendation 2: Develop mechanisms for incorporating patients' and society's perspectives on effectiveness and value*

We fully agree that it is important to understand the impact that provision of therapies (or their lack of provision) have on patients' lives, as well as the priorities patients place on addressing different aspects of the morbidity they experience. However, in coverage decisions, we believe that patient perspectives are best incorporated within a formal technology assessment. Two considerations drive this view. First, there is a long history of irrational coverage decisions of interventions that lack an established science base (e.g. witness Congress' decision to add PSA coverage to Medicare) that have been motivated by emotional appeals from patients. Persons suffering illnesses are understandably drawn by new treatments for feared diseases and this has been documented as making them vulnerable to industry-supported advocacy for devices and drugs. Aligned industry and patient interests can act to derail evidence-based policy.

A second and related concern is that of opportunity costs. In providing coverage for very high cost treatments within a fixed budget scenario, the ability to cover other interventions that may be more efficient and effective can be lost. We have seen this in the U.K. in debates triggered by intense patient and media pressure for coverage of high cost cancer drugs, notably Herceptin. Concern was raised both locally and nationally that other, possibly higher value services may be foregone or delayed if coverage of some high cost drugs were to become mandatory. In the U.S., Medicare's longstanding policy of providing immediate coverage for persons under 65 with the diagnosis of renal failure, while requiring that persons equally disabled from other diseases wait two years to establish Medicare eligibility is an example of how some populations who are first to the table have benefited in a manner that most would agree is inequitable. For these reasons, we favour detaching patient perspectives from coverage decisions.

NICE appraisals use the quality-adjusted life year (QALY) to capture patient perspectives within cost-effectiveness analyses. While QALYs are not without methodologic challenges, we think it is right to take account of patients' health-related quality of life as an integral part of the CEA calculation and not as some additional 'add on'.<sup>ix</sup>

With respect to societal values, we are fully in favour of gaining public perspectives “behind a veil of ignorance”<sup>x</sup> on an engaged citizenry’s perspective regarding prioritizing resources within a health care delivery system. NICE here offers some ways forward. In 2003 NICE’s leadership formed a Citizens Council, advisory to the appraisal committees, that was intended to reflect public opinion in areas that are not easily captured by CEAs. The membership, rotating every 2 years, is made up of 30 individuals drawn from all sectors of society, and selected to capture broadly the make up of the population of England and Wales. Members of the council listen to evidence and debate key social values that are of importance in coverage decisions. To date, bi-annual 3-day meetings have taken place on topics and issued reports on areas such as: how age should be taken into account in coverage; how orphan drugs should be handled; the role of the “rule of rescue”; and whether NICE guidance should attempt to decrease the gap in health outcomes between more and less advantaged members of society.

*Recommendation 3: Develop role for decision analytic modelling; Foster collaboration with manufacturers*

We (along with countless others) support fully the inclusion of formal CEA as a core element of TA in the US and believe that the wider use of decision analytic modelling will address a number of the concerns raised by Skinner et al.

We have serious reservations, however, about the more central role Pearson proposes for manufacturers within analysis development. Certainly it is appropriate for manufacturers to lay out the uses of new products and the interventions they are intended to replace (or gaps intended to fill), but a role beyond this invites worries of conflicts of interest. By way of example, in a largely congratulatory review of NICE conducted by the World Health Organization in 2003, a key reservation was the perception of too close a relationship with industry. This concern is supported by a recent comparison of manufacturer-sponsored and independent CEAs submitted to NICE where Miners et al demonstrate marked differences in CEA ratios; in the majority of studies, manufacturers’ ratios were more favourable to their products than were those of the independent reviews<sup>xi</sup>. Further, in our own work in recent workshops conducted with decision makers in California health care, lack of trust in commercially sponsored CEAs was viewed as a key barrier to their credibility and use.<sup>xii</sup>

*Recommendation 4: Include assessments of comparative value; Increase the comprehensibility and utility of economic evaluations to decision makers*

Again, we agree that any comparative effectiveness determination should have an economic evaluation component attached to it. Further, efforts to make comparative effectiveness evaluations useful and comprehensible to their end users are rather like ‘apple pie and motherhood’ and hard to critique. We are intrigued by the new ICER development proposal. Together with ongoing AHRQ-sponsored projects, an IOM initiative, and many regional activities – both publicly and privately sponsored – should provide fertile territory to move forward in this regard.

*Recommendation 5: Work with key stakeholders to use TAs to improve the value of health care*

This all sounds like the right direction of travel. We think that insurance coverage that limits care on the basis of poor effectiveness and/or value has a greater likelihood of achieving better health outcomes than coverage that limits care via co-pays and deductibles, thereby limiting access to cost-effective services for lower income enrollees. The immediate questions are: How will all this be achieved? (The devil lies in the detail!) For those things that have been tried before, why are they more likely to be successful this time? The pricing and reimbursement route for using CEA might offer some early successes if health plans are willing and able to take this forward. The jurisdiction where this is applied successfully, and where lessons might be learned, is Australia and the work of their Pharmacy Benefits Committee.

*Recommendation 6: Create new federal entity to coordinate and conduct TA as a public good*

We share Pearson's view that a federal entity should bear responsibility for the coordination and conduct of technology assessments as a public good. However, as we suggest earlier, absent other concerted efforts to increase the acceptability of economic analyses to health care organizations and the populations they serve, this work runs the risk of being under-utilized.

We wonder also if it is necessary to build a new federal entity to accomplish this agenda, when the Agency for Health care Research and Quality (AHRQ) has already built an infrastructure that is designed to address and support most of the types of work that these papers endorse. The Agency has a tradition of funding the development of new methods and conducts studies in evidence-based medicine and cost-effectiveness analysis and has had experience in establishing evidence-based centers throughout the United States. AHRQ has been markedly underfunded for the tasks it has been given, but we think that, with appropriate funding for this initiative it could represent a good home, for the activities outlined. The added value of establishing a new federal agency needs fuller justification.

Conclusions

The papers highlight the importance of the challenge to health care organisations of making explicit use of cost and CEA information. There is a clear need for a change of approach in health care organisations such that technology assessments are more readily employed. There are some important recommendations put forth here as to how to better constitute and package comparative effectiveness and economic information, but the way forward, unfortunately, is not as simple as providing more timely and cogent studies. We believe that a good deal of work needs to be done in preparing the ground for the public and its decision makers to understand the contribution of economic analyses to a better performing and more equitable health care system. This will need strong leadership and something of a change in culture. We suspect that this will be best accomplished by CMS working through the Medicare program, a tax-funded system which has historically served as the health care program that advances change within the US health care system.

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- <sup>i</sup> Thorpe KE, Florence CS, Howard DH and P Joski. The rising prevalence of treated disease: effects on private health insurance spending. *Health Affairs* 2005;W5 317-325
- <sup>ii</sup> Daniels N, Sabin JE. Setting limits fairly. New York: Oxford University Press, 2002
- <sup>iii</sup> Neumann PJ, Palmer JA, Daniels N, Quigley K, Gold M, Chao S. "Integrating Cost-Effectiveness Analysis into the U.S. Healthcare System" Report to the Agency of Healthcare Research and Quality, Tufts-New England Medical Center, 2007
- <sup>iv</sup> Aspinall SL, Good CB, Glassman PA, Valentino MA. The evolving use of cost-effectiveness analysis in formulary management within the Department of Veterans Affairs. *Medical Care* 2005;43(7 Suppl):20-26
- <sup>v</sup> In some respects the VA is a mini-NHS with ownership of hospitals by the VA, in this way more akin to the NHS that Aneurin Bevan (UK Health Minister, 1945-51) envisioned, given the NHS's now increasing contracts with the private sector for provision of services.
- <sup>vi</sup> Gold MR, Franks P, Siegelberg T, Sofaer S. Does providing cost-effectiveness information change coverage priorities for citizens acting as social decision makers? *Health Policy* 2007;83(1):65-72
- <sup>vii</sup> Davies E and Littlejohns P. View of directors of public health about NICE appraisal guidance: results of a postal survey. *J Public Health Med* 2002;24(4):319-325
- <sup>viii</sup> Birch S, Gafni A. Economists' dream or nightmare? Maximising health gains from available resources using the NICE guidelines. *Health Economics, Policy and Law* 2007;2(2): 193-202; and Gold M, Bryan S. A response to Birch and Gafni: some reasons to be cheerful about NICE. *Health Economics, Policy and Law* 2007;2(2): 209-216
- <sup>ix</sup> NICE has commissioned work on social value judgments as they play out in QALYs and is in the process of reviewing what, if any, additional considerations need to guide the use of QALYs.
- <sup>x</sup> Russell L, Gold MR, Siegel JE, Daniels N, Weinstein MC. The role of CEA in health and medicine: Panel on Cost-effectiveness in Health and Medicine. *JAMA* 1996;276(14):1172-7
- <sup>xi</sup> Miners AH, Garau M, Fidan D, Fischer AJ. Comparing estimates of cost effectiveness submitted to the National Institute for Clinical Excellence (NICE) by different organisations: retrospective study. *BMJ* 2005; 330:68-68
- <sup>xii</sup> In our work with health care decision makers in California, although the vast majority felt that CEA should be used in coverage decisions, nearly all said that commercial sponsorship of CEAs was a key barrier in preventing its uptake. (Bryan S, Sofaer S, Siegelberg T, Gold M. "Rehabilitating rationing: has the time come for cost-effectiveness analysis?" Report to the Californian Health Care Foundation, City College, City University of New York, 2007)