Economic analysis and national clinical guidelines: a methodological opportunity?*†

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Abstract

Commonly, economic analysis is directed at the policy level and aggregates complex treatment attributes to influence simple notions of efficiency. Our wares now have a ready audience through the work of the the National Institute for Clinical Excellence (NICE), appraising new technologies. NICE also commission a second kind of guidance called clinical guidelines. There are similarities between appraisals and guidelines, notably in their use of health technology assessment and both have a common intended audience, namely the NHS, clinicians and patients. However, there are some very important differences, which expose the issue of where our discipline is going.

Should national guidance be replaced by clinical protocols (telling clinicians what to do), based on our economic models? Should we just tell policy-makers what is cost-effective and then we have done our bit? Should we teach patients to understand QALYs? Should we provide the right kind of information so that patients can rank their options and make informed choices? What is the right kind of information, and what would you as a patient want to know?

We might desire our economic analyses to be: robust, valid, informative, participatory, transparent, comprehensive, relevant, but are they, and to whom? Some elements are offered to provide a context for discussion: the methods used to develop national guidance; the nature of clinical agency; and the values underpinning alternative economic analyses. The gist of my argument is that economic evaluation should be about helping society (and that means its individuals) to value healthcare. In the tradition of HESG, this paper is work-in-progress, and a number of the ideas are not fully formed: I would value your comments.

Preamble

It intrigues me how from disease models, with numerous uncertainties and complexities about the value of treatment and prognosis, such robust answers of cost effectiveness often seem to emerge. I think models are great for exploring uncertainties but that their best guess answers are largely fictional, they are unlikely in any statistical sense to be average or provide meaningful comparisons between technologies. In the past our models have been largely for academic or commercial pursuit and ignored in medicine [1] so no problem. Now the stakes are raised with bodies such as the National Institute for Clinical Excellence (NICE) writing guidance heavily influenced by the modelling approach. We know the calculations are speculative but managers want simple answers, and rest their decisions on our numbers as 'the experts'. With the decree that guidance arising from NICE's appraisal process must be fully funded in the NHS, we have the situation where funding is directed to the more marginal technologies (that is why they were appraised) and away from other unevaluated activity. The opportunity cost of enacting such partial guidance is potentially horrific, and may exacerbate local variations in (the rest of) healthcare for the sake of fully funding appraised technologies. Previously, high cost new technologies were rationed reflecting a balance of budgetary constraint and clinical judgement. This undoubtedly led to variation in delivery of new technologies but left room for common sense. Some clinicians now talk about 'appraisal blight', when there is no new drug in their field then NICE won't be 'guaranteeing' extra funding for their speciality. Whether the drug is any good doesn't appear to be a factor.

The appraisal process appears to have absorbed a lot of available health economic and health service research capacity in England. Furthermore, new academic courses are springing up tailored to produce more workers. Should we be concerned that are discipline is being driven and shaped by political goals and funding? Consistent with its political and managerial aims, NICE funding is targeted at producing answers using its prescribed methods, not at improving our understanding of the NHS and how to interact with it. Now seems to be a good time to reconsider our paradigm, as it may cause us to argue to change the way healthcare treatments and delivery are evaluated. Much of the healthcare we provide has quite modest benefits and (for me) the real challenge is about improving rationality of resource use in the NHS.

A note on terminology for those unfamiliar with this field: A *protocol* is a management process that must be followed unless overwhelming circumstances dictate otherwise. See 'A' do 'B' has real value in acute circumstances and can save lives: clinicians may be censored and/or sued for not following authoritative protocols. A *guideline* sets out standards and expectations and is intended to promote a consistent quality of care, working with the continuing education ethos of medicine. Regardless of the GP you see for your raised blood pressure there should be common elements of discussion and care offered. Although guidelines have no legal status in this country the lines can become blurred with negligence claims being brought on the basis of guidelines in more litigious societies. The final point is that NICE produces two kinds of *guidance* arising from the appraisal process and the guideline

process (more on this below)

What follows is some brief information about NICE, about how our economics evolved, and about clinical decision making. Then, I offer some opinions…

The National Institute for Clinical Excellence

NICE disseminates two types guidance to the NHS, clinicians and patients. Firstly, appraisal committees investigate selected new technologies, informed by rapid health technology assessments, commercial submissions and advocacy from stakeholders reflecting commercial and patient interests. Each item of guidance is written by the NICE executive under the guidance of one of its appraisal committees. In concept appraisals began with a narrow focus, but the scope of appraisals is becoming increasingly broad to answer more relevant questions. The guidance is legally underpinned and effectively a reimbursement decision for the NHS. Uniquely, the appraisal committee listens to advocacy from interest groups (commonly manufacturers and patient groups), but has no counter-advocacy.

Secondly, NICE has commissioned seven national collaborating centres to develop clinical guidelines. These centres are groupings of Royal Colleges, national bodies and academic units. The guidelines have very broad scopes (for example how to manage patients with raised blood pressure). The guidelines and subsequent recommendations are developed independently of NICE although there is a stakeholder process providing input at various stages in guideline development. Guidelines currently have no legal status, and it would be methodologically disastrous if this were to change. Guidelines work to augment clinical autonomy, not to subjugate it. Currently guidelines are not intended to address controversial reimbursement decisions for new technologies: instead they address the spectrum of treatment options and care pathways that exist in routine care.

Both processes are underpinned by review of relevant evidence through a health technology assessment (HTA) process, although by the nature of the process guideline groups have greater scope to shape and interpret the evidence. Both processes take submissions from stakeholders (parties from industry, the NHS, academics and patient groups who register with NICE) although guidelines do not deal with commercial in confidence material or unpublished economic analyses. Both processes are intended to include assessments of cost-effectiveness and the final summary guidance and patient versions produced by NICE are similarly packaged such that you might not realise they were produced by different processes.

The Relenza debacle demonstrates that the appraisal process is a politically vulnerable (or perhaps convenient) process. There is neither separation in the NICE Executive between the political expediencies of NHS management and the authorship process, nor recognition that this is a problem. It is not transparent how the original HTA, stakeholder submissions and committee discussions are linked to the final guidance. By contrast a guideline provides an audit trail: definition of relevant

evidence, tabulation of studies, evidence narrative and quantitative analysis, evidence statements, recommendations. Anyone can see (in principle) where a recommendation comes from and why.

Both appraisals and guidelines will experience variations in quality: any form of research is vulnerable to inadequate resources or skills. Consequently, the opportunity to manage core-funded units developing guidance is a genuine advance in terms of developing and retaining skills. However, there is an unresolved make-or-break debate waiting for NICE about the methodological rigour required when developing national guidance. The independence of the guideline process frees it from the perception of political or industry tampering. Conceptually this appears an important difference between the processes and is closer to the traditional relationship between research sponsors and academics.

Economic beginnings

For me the beginning was with welfare economics which seeks to 'formulate propositions by which we may rank, on the scale of better or worse, alternative situations open to society' [2]. This general statement is intriguing and has just as much to do with social justice as with efficiency. Indeed the Paretian tradition argues that an increase in efficiency is good as long as there is no decrease in distributional fairness. Thus, some key issues for any form of social decision-making are (1) how do we value alternatives and (2) whose values are to count? Welfare economics evolved to set out strictures for efficiency in the demand and supply of commodities and overall efficiency of an economy. The application was to explore alternative allocations of healthcare use by varying patterns of production and distribution to ascertain whether these produced more overall wellbeing in society than the current one. One of its virtues and stumbling blocks was the need to preserve the valuations of individuals when assessing the aggregate social valuation of alternatives. Arrow's Impossibility Theorem demonstrated the impracticality of this elegantly in 1951 [3].

Cost benefit analysis sought mechanisms to financially compensate losers of social policy changes so that some people are better off and none are worse off. This approach appeared largely still-born in healthcare given the tremendous problems getting monetary valuations of health gains in insurance-based or publicly funded systems. However, the balance of challenges for economic analysis may be shifting as healthcare becomes increasingly elective and medicines become available over-the-counter or in pharmacies. Currently, there is an application for low-dose omeprazole, the leading prescribed drug for dyspepsia, to be sold directly from pharmacies (of 1535 chemical entities prescribed in primary care in England in 2001, omeprazole was ranked 5th on expenditure and 22nd by volume). Healthcare may be becoming more like other marketplace commodities: with improving information about its attributes, repeated use by individuals and development of price signals beyond co-payments. This trend has some interesting social implications. OTC medications are often sold in smaller quantities, lower doses and higher prices than prescription drugs. Relatively wealthy patients may choose the convenience of self medication relieving pressures on NHS resources, although this

adds to the potential long term challenge for a free-at-the-point-of-use National Health Service.

We are inherently economic in our thinking: we achieve complex budget-constrained maximisations every time we go shopping. Informed shopping mean we know and can value the attributes of commodities. Informed patient choice can be developed by describing the good and bad attributes of healthcare alternatives. Where the choice is made is the point where resources are actually committed, so it seems to me economic evaluation should be at the forefront of informing patient choices and understanding their interface with policy objectives.

However, the main thrust of economic evaluation appears to have been to make the social valuation task manageable for policy-makers by estimating the rate of health output per unit of resource. The range of good and bad consequences of treatments is amalgamated into a simple measure of health gain, the Quality-Adjusted Life Year (QALY). Thus NICE can compare the health-related preference for different types of treatments and disease. Thus defined, efficiency dictates that NICE should maximise QALYs with the available NHS budget and this directs funding to the most cost-effective care. The task is simplified by ignoring equity – how health benefits are distributed – and the complexity of clinical decisions by focussing on simple buy-don't buy decisions at a managerial or policy level. We also need to ignore any routine, unevaluated care that may be displaced by such decisions. Cost/QALY league tables first emerged about a decade ago [4], and the strengths and weaknesses of this approach have been discussed [5]. The apparent simplicity of the cost/QALY approach is understandably attractive at the policy level when needing to make a reimbursement decision. It is the gold standard methodological approach identified by the NICE appraisal process, and operates a 'fuzzy' threshold of about £30,000/QALY.

Along the way we ditched the notion of individual valuations as too difficult, although theoretically the sum of individual valuations should inform prioritisation. Our energy appears narrowly directed at our own notions of efficiency with no attempt to understand real peoples' valuation of healthcare. Consequently we continue to argue over complex and speculative models for which just a few have any understanding. The industry says it is £20,000/QALY, the academics say £100,000/QALY and both models if robustly explored would give such a range of values that we would realise they are uninformative. How about we go back to the trials and thinking about whether the additional physical benefits and harms feel like good value-for-money? We would accept nothing less in our personal lives.

An example would be the way different groups have assessed the cost-effectiveness of the Alzheimer's disease drug donepezil. From a physical viewpoint the average improvement from therapy is 2 to 3 points on the Alzheimer's Disease Cognitive Subscale over 12-24 weeks, a change which at the individual level is not clinically important. Trials found no improvements on quality-of-life scales. Nonetheless if you assume that there really is an underlying and unmeasured quality-of-life gain and you assume that there is a delay in time to institutional care, then an expensive, equivocal drug becomes an acceptable buy using a cost/QALY approach. As the drug came to market, huge expectation was generated in clinicians, patients and carers by company-sponsored academic

detailing and media coverage with little counter argument. It would take a politically independent, scientifically rigorous and determined policy process to stand up to such pressures and explain why further long-term studies should inform the considerable uncertainties before the drug could become routine care.

It seems we are quite happy to chide clinicians when their attitudes to patients appear inadequate but we require no public mandate in the way we value currently healthcare on behalf of society. Our NHS is something most in our society values tremendously, and a shift from the current vogue for central planning to the opposite end of the spectrum (consumerism) is not the solution. However an open dialogue around the known facts has transparency and feels more credible. The delivery of the idea hangs on an agent-mediated model and for that we should brush up on clinical decision making.

Describing clinical choice

Under certain conditions individuals are good at maximising their welfare and there is no need for external interference in their choices. These conditions include rationality (e.g. familiarity with commodities, consistent and ordered preferences); an absence of externalities (e.g. valuing positively or negatively other individuals' choices; and, an adequate budget to achieve social norms of consumption). The consumer is said to be sovereign, each person is the sole judge of his own welfare.

Healthcare is an unusual commodity [6]: it may be rarely used and can be devastatingly expensive. If illness is chronic we may become progressively less able to pay for healthcare. Consequently, some form of insurance or social fund emerges. To protect and regulate, clinicians alone are licensed to practise medicine: they have greater knowledge due to training and experience and often treat patients at a time of need and vulnerability. Consequently clinicians have control not just of the supply of healthcare but also substantial influence over patient demand. Clinical decision making is not characterised by consumer sovereignty but an agency relationship in which the clinician as the agent has considerable influence upon healthcare demanded. The appropriateness of this agency relationship has been questioned [7]. However, an ideal agency relationship assumes that the clinician enables the patient, or their representative, to participate in the process of decision making to the extent they are able and willing and is able to communicate meaningfully the consequences of available options. In this context, clinical freedom is transparently a misnomer; good medicine is about clinical responsibility. Today, this realisation is more pertinent than ever before: although the asymmetry remains we increasingly refer to patients as consumers. The evidence-based culture is permeating through society and patients increasingly expect information and participation. Mishan's assertion that choosing healthcare is not like buying apples and oranges appears less true forty years on, and reflects medicine's ability to offer far more to the less severely ill [2].

Valuing healthcare

If we should start with a clean sheet of paper we might write out our aspirations for our work. I like the words: robust, valid, informative, transparent, participatory, comprehensive, relevant. Naturally, each word needs careful definition and you may have some additions.

To present clinicians and patients with treatment recommendations based on the aggregation of good and bad consequences of a constrained selection of alternatives is neither helpful nor clinically appropriate. It is akin to telling shoppers what their overall happiness will be once they have accepted a their basket of commodities. Instead patients, with clinicians as their agents, need to have described the separate attributes of treatments so that they can perform a personal overall valuation. For example the care provided for patients with raised blood pressure must take in evidence on half a dozen drug classes, an array of lifestyle considerations as well as measurement, cardiovascular risk assessment, drug sequencing, education, monitoring and adherence: all of these activities are currently using NHS resources. The path to a better use of resources appears to be to engage health service professionals and patients in valuing these activities rather than by attempting to impose external dos and don'ts.

Clinical guideline development processes provide an obvious forum for valuing health care options. The method allows that a representative group process identifies the important aspects of available treatments and uses evidence-based and health economic techniques to quantify important attributes of care [8]. On *process* grounds, for the domains 'informative, transparent, comprehensive and relevant' a guideline appears better placed than an appraisal to deliver where it counts. For 'robust and relevant' there may be little agreement between the 'lumpers' and the 'splitters' about how healthcare priorities should be informed.

Final thoughts

The different rationales apparent in the two processes for developing national guidance have been aired. For me the key point is whether we want our economic analyses to stay aloof, impinging only upon policy forums or do we want to get stuck into enfranchising clinicians, patients and the public in the valuation process for publicly provided healthcare. The latter approach appears a step toward public participation and understanding of the health service, while the former seems intended to keep the public at arms length. Of course any debate about impact may be academic: there is no guarantee that an overworked and guidance/audit/target overloaded NHS will take any notice of NICE guidance, how ever well conceived and written. Nonetheless we have a duty to define our discipline and its directions.

Protagonists often refer to their cost/QALY models as explicit decision-making with anything less being implicit. The vast majority in society who have to take these models on trust may feel excluded. Whether the move from a cost/QALY approach to a cost and consequence approach involves a

welfare loss or gain must depend partly upon the credibility of the respective valuation processes and the recommendations they generate. It is not possible to say that the widespread use of national guidance (however produced) will only ever improve the benefits received by patients. Very few new products have been denied funding. As the process matures, it may be that some patients will be denied expensive treatments of marginal health benefit that they personally value highly and would have received had guidance not been produced. It is however the aim that decision-making may become more consistently directed towards socially defined 'worthwhile' healthcare and thus make progressively better use of scarce resources. Alan Williams proffered that ' the immediate task of health economists is to get our notions of efficiency and fairness in the distribution of the benefits of healthcare so deeply embedded in the clinical consciousness that they come to be thought of as wholly within the realm of clinical autonomy'. This is realistic if we are willing to understand the realities of clinical care and thoughtfully engage our natural propensity to be economic.

References

1 Davies LM, Coyle D, Drummond MF and the EC Network. Current status of economic appraisal of health technology in the European Community: report of the Network. Social Science and Medicine 1994; 38(12) 1601-1608.

Drummond M, Cooke J and Walley T. Economic evaluation under managed competition: evidence from the UK. Social Science and Medicine 1997; 45: 583-595.

Ross J. The use of economic evaluation in health care: Australian decision makers' perceptions. Health Policy, 1995;31: 103-110.

2 Mishan EJ. Welfare economics: ten introductory essays. New York: Random House, 1969

3 Arrow, K.J. Social choice and individual values. John Wiley & Sons, New York, 1963 (2nd ed.)

4 Maynard A. Developing the healthcare market. The Economic Journal 1991; 101:1277-86

Mason JM, Drummond MF, Torrance GW. Some guidelines on the use of cost-effectiveness league tables. British Medical Journal, 1993, 306: 570-572

5 Loomes G, McKenzie L. The use of QALYs in health care decision-making. Social Science in Medicine. 1989; 28: 299-308

Rawles J. Castigating QALYs. Journal of Medial Ethics. 1989; 15: 143-147.

Smith A. Qualms about QALYs. Lancet 1987, May 16, 1134-1136.

Mason JM, Drummond MF. The DH register of cost-effectiveness studies: content and quality. Health Trends, 1995, 27(2): 50-56.

6 Arrow KJ. Uncertainty and the welfare economics of medical care. The American Economic Review. 1963; 5:941-97

7 Williams A. Health Economics: The End of Clinical Freedom? BMJ 1988; 297: 1183-6

8 Field, M.J., Lohr, K.N. (eds) (1992) Guidelines for clinical practice: from development to use, Washington DC: National Academy Press.

Eccles M, Mason J. How to develop cost-conscious guidelines. Health Technol Assess 2001; 5 (16)