Could the PPB be nicer than NICE? - an investigation of 'societal values' concerning costs per QALY

Neill Booth, Ph.D. student at the School of Public Health, University of Tampere, Finland.

ABSTRACT:

Aims
This paper describes key features of the roles that cost-effectiveness information and cost-effectiveness thresholds play in 'decision-making for society' (DMS) in England and Finland. It aims to investigate some of the grounds for DMS in each country – in particular, the decisions of the National Institute for Health and Clinical Excellence (NICE) and the Finnish Pharmaceuticals Pricing Board (PPB).

Methods
A limited review of the NICE approach is accompanied by details available from the literature on the Finnish setting. Some of the findings from a survey of a relatively large group of key opinion-leaders in Finland, undertaken via a handheld electronic voting system, are also reported. An attempt is then made to use a framework suggested by Williams to assess whether the PPB approach could be 'nicer' than that of NICE.

Results
The stated objectives of the publicly provided health care system are broadly similar in the two settings. However, this preliminary investigation shows that legislation, attitudes to DMS, and guidance in conducting technology appraisals currently differ with each country and demonstrate also that incentive structures show meaningful variation. This variation seems to result in differences in societal values with respect to costs per quality-adjusted life-year (QALY).

Conclusions
On the basis of this preliminary investigation, the structure of DMS is found to be context-dependent, as is the part health economics plays in related processes. There seems to be a need not only to clarify the bases for differing approaches to DMS but also to modify the use of health economics to better fit each context.

Introduction

Need is a difficult concept to define [1], especially for economists considering the topic of health care [2]. Williams offers a potential resolution to unravel the tangled mess of prioritisation: he suggests 'societal judgement', the only caveat being that 'societal judgement' is a complex game *per se* because the incentives of the players are both opaque and dynamic [2]. The National Institute for Health and Clinical Excellence (NICE) appraisal process is heralded as exemplary in its use of cost-effectiveness information and cost-effectiveness thresholds [3] as well as in its use of deliberation [4]. The use of cost-effectiveness information in what has been termed evidence-informed decision-making has even been labelled 'social' decision-making or 'societal' decision-making (SDM) (see, e.g., [5, 6]). One problem with this is that the meaning of decision rules in 'theoretical' health economics may be far removed from that of decision rules in choice problems concerning alternative health care interventions outside academia.

Fortunately, numerous publications have together begun to dispel the myth that there is a single 'societal value' of a quality-adjusted life year (QALY) (see, e.g., [7-9]). However, a mythical beast (somewhere between 18,000 and 50,000 pounds sterling in size) still seems to lie close to the heart of some of the decisions of NICE's appraisal committees [10, 11]. In Finland, on the other hand, the only beasts that currently seem to surface are the friendly-looking storybook characters the Moomins and a partial rejection of cost-effectiveness analysis as a source of useful information for decisions concerning reimbursement and wholesale pricing of prescription medicines [12].

There has been a broad degree of similarity between the Finnish and English health care systems, especially since the end of World War II, with the organisation and financing of health care being, largely, a collective responsibility of society. This similarity has led to the idea of comparing the current situation in England and Finland. By this I mean comparison in terms of both the respective societal objectives and the manifestations of potential objectives in the choice processes devised, between NICE and the Finnish Pharmaceuticals Pricing Board (PPB).

Various propositions for consistency in economic evaluation have been put forward, often grounded in welfarism or economics (see, e.g., [13]), but other texts have pointed to some of the difficulties this might involve, such as the need for specificity as to context [14].

As an addition to the quasi-theoretical bases for extra-welfarism in health care set out in the recent work of Brouwer et al. [15], this kind of extra-welfarism is based on cost–benefit thinking (CBT) (see, e.g., [16]), which pre-dates the Paretian tradition [17] and 'new' welfarist economics. If the foundations of the decision-making approach to extra-welfarism [18] are not, necessarily, within utilitarianism, neither need they be confined by its utilitarian maximisation principle [15] or the QALY egalitarianism that usually its accompanies it.

The monetary valuation of mean changes in, say, health-related quality of life is invariably linked with prioritisation. Williams [19] notes that "rationing and the use of thresholds has always been with us" and goes on to outline three broad approaches to rationing of health care both prior to and following the publishing of the Beveridge report [20]. At the

beginning of this period, a widespread approach was rationing on the basis of price – i.e., by individuals' willingness and ability to pay. Next came rationing by medical opinion under a publicly provided health care system, often based on individual clinicians' assessments of how much society is willing and able to pay for publicly provided health care. Finally, in recent years, there has been a move toward wider consensus being sought with respect to rationing in a publicly provided health care system. Just how this has played out in Finland and England is still unclear, but it may have manifested itself as rationing through pharmaceutical pricing initiatives and other meso-level decision-making entities, often with their own viewpoints concerning the amount that society is willing and able to pay for publicly provided health care [19].
Under price rationing and from the individual's perspective, need for health care is mainly restricted by the individual's budget, and this may be a forceful constraint. Under non-price rationing, decisions to withhold health care can be in direct opposition to any single individual's perceived, potential, or proven need for health care. Such competing objectives are an integral part of publicly provided health care, as is the general conflict between the value of treatment for the individual patient and considerations of the value to society of that treatment for all such patients.

Societies tend to differ in the ways they handle such conflict. The objectives of this paper are to clarify the current 'state of play' in Finland and England and to explore what health economics might have to offer each setting. This paper will then go on to give a tentative answer to the question of which is 'nicer' from the perspectives of both cost–benefit thinking and societal objectives.

Methods

Using some of the available literature, policy documents, and legislation, the paper will offer a description of objectives of health policy in each setting, followed by the corresponding methods of implementing these objectives of health policy. The roles and use of cost-effectiveness information and cost-effectiveness thresholds, and other forms of economic evaluation play in each context, will then be investigated.
To some extent here, we attempt to follow the approach of Williams and, for each country in turn, make suggestions as to "who shall play what according to which rules" [2]. To investigate such 'social judgements', herein referred to as 'decision-making for society' (DMS), an attempt is made to outline the DMS players in each setting. Likewise, the roles and rules governing the main players in DMS will be outlined. In addition to a preliminary note on the objectives of economics and economists, a sketch is provided of the loosely defined headings of country objectives, organisational objectives, and the current state of play in DMS.
In addition, we present a potential source of useful information in the form of a survey of a large group of key opinion-leaders that was undertaken in Finland by means of a handheld electronic voting system. This survey was undertaken as part of the 17th symposium of the Finnish Society for Health Economics, in February 2008. The society's annual symposia are attended by delegates from the Ministry of Social Affairs and Health as well as other leading figures in the Finnish health care sector, from fields such as research, policy, industry, and management. The participants numbered around 300, and most of the 250 handheld devices available were in use.

Results

This paper is still very much a work in progress, partly because judgements concerning decision-making are difficult to observe from outside the choice processes themselves or with economic tools [21].

English health care objectives

The Green Paper 'Our Healthier Nation' stated that the broad, though competing, objectives of the then government were to 1) improve the health of the population as a whole by increasing the length of people's lives and the number of years people spend free from illness and 2) to improve the health of the worst off in society and narrow the health gap [22]. On a more pragmatic level, the National Institute for Health and Clinical Excellence is charged with appraising the clinical benefits and the cost-effectiveness of forthcoming English National Health Service (NHS) treatments and to make recommendations concerning the provision of these treatments within the NHS [23].

NICE's objectives

In undertaking the task outlined in the previous section of the paper, when undertaking appraisals, NICE should also take into account the broad clinical priorities of the Secretary of State for Health, the degree of clinical need of the patients, the broad balance of benefits and costs, any guidance from the Secretary of State on the resources likely to be available, concern for effective use of available resources, and the need to be sympathetic to the longer-term interest of the NHS in encouraging innovation of good value for patients [24].

NICE in practice

Given the objectives of the decision-maker (in this case, the government), a decision-making body has at least two key distinguishing features: its methods of implementing the objectives and the roles played by cost-effectiveness information and cost-effectiveness thresholds, and other forms of economic evaluation. In the case of NICE, in general, the methods are becoming increasingly clear [25-27]. Possible exceptions to this state of clarity are the guidance on cost-effectiveness thresholds (see pp. 58-9), broad rejection of budget impact analysis (BIA) as a tool (p. 57), and partial rejection of cost–consequences analysis (CCA) within NICE's assessment process in consequence of focus on the reference-case perspective (p. 32) [25].

Finnish health care objectives

There is a somewhat understandable dearth of English-language sources addressing the objectives of health care in Finland. Both the Finnish constitution [28] and the Primary Health Care Act [29] can be interpreted as underpinnings for the Finnish government's objectives concerning health care. Much of health care in Finland is provided by tax-based funding for all within the catchment areas of the various municipal authorities [30]; a major exception to this is the Finnish Social Insurance Institution, which oversees reimbursement provided under the National Health Insurance scheme [31].

PPB objectives

At a national level, and in accordance with the Health Insurance Act [31] and the Government Decree on the Pharmaceuticals Pricing Board (PPB), the PPB is subordinate

to the Ministry of Social Affairs and Health and assesses what constitutes reasonable wholesale prices and reimbursement for medicinal products. The PPB must take into account the therapeutic value of the product. As part of the assessment, the PPB is charged with investigating the costs and benefits for the patient and for health care and social services, the costs and benefits of other available alternatives, the prices of comparable products in Finland and other European Economic Area countries, and the manufacture and research and development costs of the product, as well as taking into account the funds available for reimbursements [32].

The PPB in practice

The methods of implementing objectives of health policy via the PPB are, as is potentially the case with NICE, current interpretations of the statutes and decrees that govern its role. Applications concerning pharmaceuticals containing any new chemical entity must include a health economic evaluation [32], but there is no obligation on the part of the PPB to take submissions of cost-effectiveness information into account in their decisions. On the other hand, the reality seems to be, at the very least, a partial rejection of cost-effectiveness analysis (as recently applied) as a source of useful information for decisions concerning reimbursement and wholesale pricing of prescription medicines [12].

A DMS survey

Below are presented aggregate-level results concerning two questions (in figures 1 and 2) related to the maximum 'cost per QALY' in Finland and the use of a 'cost per QALY' threshold in Finland. However, post-survey technical problems rendered impossible further analysis of the background variables collected and the voting preferences of each voter at any other level than the aggregated.

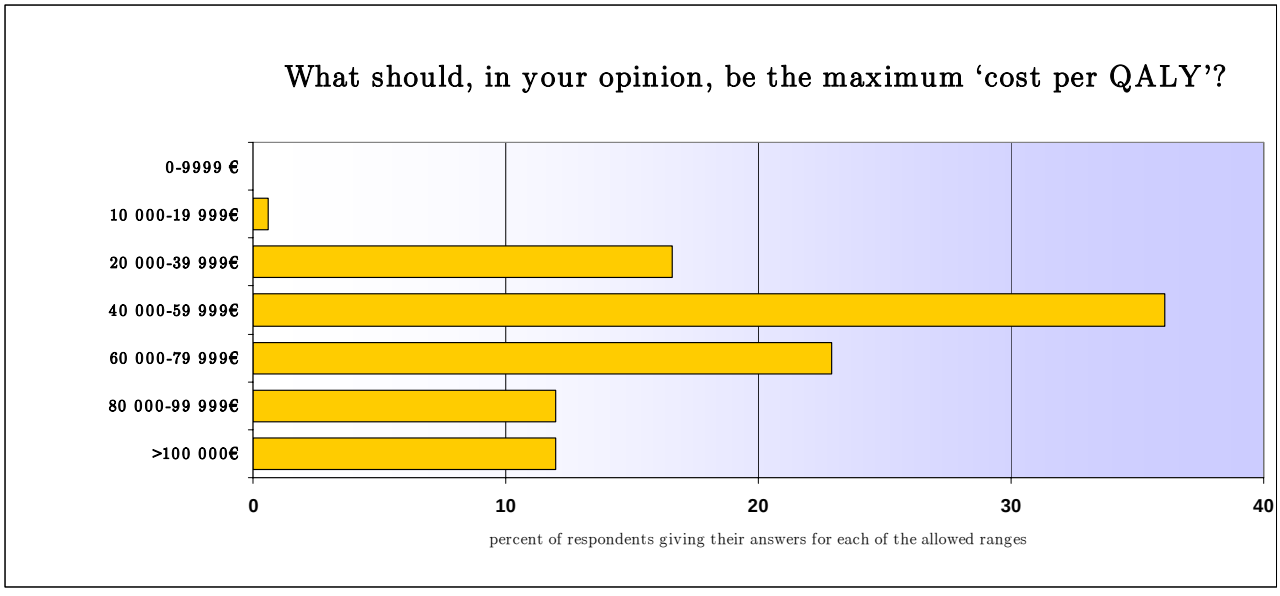

Figure 1: Survey results on opinions concerning the maximum 'cost per QALY' in Finland

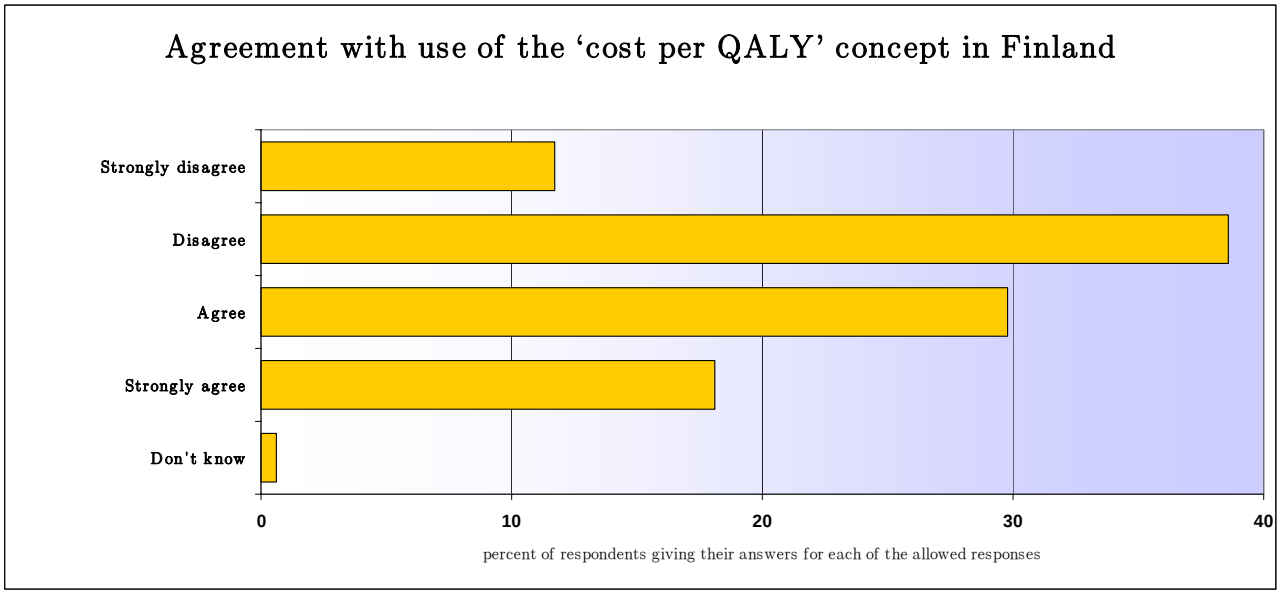

Figure 2: Respondents' opinions concerning the use of a 'cost per QALY' threshold in Finland

A note on the objectives of economics and economists

How well do the current use of health economics and the role of health economists mesh with the principles of practical cost–benefit analysis [18]? Sugden & Williams offer the following advice: "Because the analyst has some responsibility to principles over and above those held by the decision-maker, he may have to ask questions that the decision-maker would prefer not to answer, and which expose to debate conflicts of judgement and of interest that might otherwise comfortably have been concealed" (p. 241). There is potential conflict between the rhetoric of health maximisation and the practical maximisation of a proxy for health, in the form of a QALY estimate. The formation of a super-consistent proxy metric for health is unlikely, but it seems that consistency – i.e., some semblance of a scientific approach – is sought [26]. Practical extra-welfarism appears to need to be highly context-specific, and, therefore, generalised statements of cost-effectiveness, with little regard for local or national opportunity costs or CBT, can at best be erroneous and at worst disingenuous [33].

Given the dominant focus on monist cost-effectiveness analysis, one suggested (partial) solution [34, 35] is to present other calculations of the opportunity costs involved in the choice by supplementing the use of CEA with cost–consequences analysis [36] or budget impact analyses [37], or substituting the latter methods for it.

The information that follows, in **Table 1**, is a crude and stylised summary of the above results.

	PPB	NICE
CEA	✗	✓
BIA	✓	✗
CCA	✓	✗
industry lobby	✗	✓

medical benefit	✓	✓
health economists	×	✓
social cohesion	✓	×
economic evaluation guidelines	×	✓
medical ethics education	✓	×

Table 1: Factors in the study question

Discussion

Whatever form cost–benefit thinking takes, economic evaluation cannot hope to avoid all tensions between the approach chosen and other forms of moral or ethical thinking, especially given the multiple ethical principles often found within the health care sector. Some of these tensions are likely to have bearing on the suitability of particular applications of cost–benefit thinking in any publicly funded health care system. The Beveridge report [20] states that factors deemed 'irrelevant to real need' have no place in restricting health care. However, when something akin to cost-containment becomes a relevant factor for societally (and/or medically) defining some notion of need, the costs and consequences of treatment may have plenty of impact.

One added difficulty for an investigation such as this is the dynamic nature of 'societal values' concerning costs per QALY. When thinking about the art–science divide [38], some health economists might benefit from heeding a recent press release from NICE [9], as it could spell the end of dominance of the QALY-egalitarian, all-consistent, cost-per- -individual-outcome-measure paradigm in the UK [39]. For groups near the end of life, opportunity costs change; when this happens, the QALY metric used in CEA seems to collapse and art in decision-making (i.e., DMS rather than SDM) seems the only plausible option for saving people deemed to be in 'societal' need [40].

One piece of information that health economics can provide as to the value of health-related improvements is the incremental cost-effectiveness ratio. However, in the absence of suitable thresholds for investment and disinvestment [41], as may well be the case in both the settings described here, other information seems to be warranted. Offering an incremental cost-effectiveness ratio and simply stating or assuming a single threshold can be increasingly regarded as only providing a dark alley for passing drunks [42]. Recognition that a comprehensive health economic assessment should include supplementary elements of budget impact analysis and cost–consequences analysis is my current interpretation, in part, of Culyer's deliberative processes in 'societal' decision-making [43].

Limitations of the study

The PPB undertakes national-level decision-making processes to select those outpatient prescription medications for which reimbursement is to be granted, as well as the level of reimbursement [44]. By contrast, NICE's focus is on the use of medical interventions in the NHS and, to an increasing degree, the assessment of public health interventions [45]. However, these two examples of DMS can be considered to be related by way of a general

aim in the prioritisation of what should be covered by the public health care system in each country, in turn. …

Preliminary answers to the study questions:

On the basis of the survey, some of the available literature, and CBT, it seems there are no clear reasons for which a NICE approach would be nicer than the PPB, in Finland.
In England, however, if the community and its representatives, including health economic analysts, have chosen the NICE reference case and the A4R, which may underpin it, then NICE could be nicer than the PPB. For example, NICE's remit includes a remit to take into account the need to be sympathetic to the more long-term interest of the NHS in encouraging innovation of good value to patients. Amongst other things, this objective in the list could be interpreted as an institutionalisation of pharmaceutical interests [46, 47].

Conclusions

In summary, it seems reasonable to suggest that in health economic evaluation, in many cases, supplementing the multiple calculation of cost-effectiveness ratios by undertaking both budget impact analyses and cost–consequences analysis could produce a clearer three-or-more-dimensional health economic picture of the choice in question and be thoroughly informative for the evidence-informed decision-making process that health economics purports to serve.

Potential HESG discussion points

This paper is still in need of revision; hence, I have more than a few potential questions to the potential HESG audience:

- I remain somewhat puzzled by the NICE guidelines' apparent rejection of the use of CCA and BIA in the assessment process. How can it be explained? Is there really a a trade-off between practicality/consistency and a form of economic evaluation including CCA and BIA?
- The largely liberalist and American concept of A4R [48] is on display in at least two NICE documents [27, 49], but is this central (or even on display) in the well-rehearsed objectives of English health care? Is it just that "the decision-maker has not thought consciously about all the objectives" [18]?
- Which is more important in each setting, to ration public spending on health care or ration the profits of the pharmaceutical industry?
- Why do many health economists advertise the utilitarian efficiency argument part(s) of NICE's 'decisions', i.e., eulogize the use of CEA under a reference case? – *Can it be stopped?* (as NICE, at the very least, overtly employs deliberative processes?)
- Is there any reason to suppose that a partial application of a partially scientific approach would be better than a reasonable application of an approach based on CBT?
- If what we are doing here is 'just' 'evaluation' [45], why is NICE clinging to the 'scientific' remnants of orthodox neo-classical economics instead of looking to a broader notion of CBT for help?

References

1. Culyer AJ: **Need - an instrumental view**. In: *Principles of Health Care Ethics, 2nd Edition.* Edited by Ashcroft R, Dawson A, Draper H, McMillan J. Chichester: Wiley; 2007: 231-238.
2. Williams A: **Need - an economic exegesis**. In: *Economic aspects of health services London: Martin Robertson, 1978.* Edited by Culyer A, Wright K; 1978.
3. Walker S, Palmer S, Sculpher M: **The role of NICE technology appraisal in NHS rationing**. *British medical bulletin* 2007, **81-82**:51-64.
4. Culyer AJ: **NICE's use of cost effectiveness as an exemplar of a deliberative process**. *Health Economics, Policy and Law* 2006, **1**:299–318.
5. Griffin S, Claxton K, Sculpher M: **Decision analysis for resource allocation in health care**. *Journal of health services research & policy* 2008, **13**(Suppl. 3):23-30.
6. Smith P, Ginnelly L, Sculpher M (eds.): **Health Policy and Economics: Opportunities and Challenges**. Maidenhead: Open University Press; 2005.
7. Brouwer W, van Exel J, Baker R, Donaldson C: **The New Myth: The Social Value of the QALY.** In: *PharmacoEconomics.* vol. 26: ADIS International Limited; 2008: 1-4.
8. Culyer A, McCabe C, Briggs A, Claxton K, Buxton M, Akehurst R, Sculpher M, Brazier J: **Searching for a threshold, not setting one: the role of the National Institute for Health and Clinical Excellence**. *Journal of health services research & policy* 2007, **12**(1):56-58.
9. **NICE announces measures on end of life medicines** [http://www.nice.org.uk/media/6C4/6C/2008066MeasuresOnLifeMedicines.pdf]
10. Williams A: **What Could Be Nicer than NICE?** In. London: Office of Health Economics; 2004.
11. Devlin N, Parkin D: **Does NICE have a cost-effectiveness threshold and what other factors influence its decisions? A binary choice analysis**. *Health Econ* 2004, **13**(5):437-452.
12. Kivioja A: **[The role of pharmaceconomics in decisions concerning reimbursement and wholesale prices]**. *Dosis* 2006, **22**(3):301-307.
13. Claxton K: **The irrelevance of inference: a decision-making approach to the stochastic evaluation of health care technologies**. *J Health Econ* 1999, **18**(3):341-364.
14. Lehoux P, Williams-Jones B: **Mapping the integration of social and ethical issues in health technology assessment**. *International Journal of Technology Assessment in Health Care* 2007, **23**(01):9-16.
15. Brouwer WBF, Culyer AJ, van Exel NJA, Rutten FFH: **Welfarism vs. extra-welfarism**. *Journal of Health Economics* 2008, **27**(2):325-338.
16. Ruskin J: **Munera pulveris**. New York: Lovell, Coryell & Co.; 1862.
17. Pareto V: **The New Theories of Economics**. *The Journal of Political Economy* 1897, **5**(4):485-502.
18. Sugden R, Williams A: **The principles of practical cost-benefit analysis**. New York: Oxford University Press; 1978.
19. Towse A, Pritchard C, Devlin N (eds.): **Cost effectiveness thresholds: economic and ethical issues**. London: King's Fund/Office of Health Economics; 2002.
20. Beveridge SW: **Social insurance and allied services report**. *HMSO, London,* 1942.
21. Eddama O, Coast J: **A systematic review of the use of economic evaluation in local decision-making**. *Health Policy* 2008, **86**(2-3):129-141.
22. Department of Health: **Our healthier nation: a contract for health**. In. London: Stationery Office; 1998.
23. McCabe C, Claxton K, Culyer AJ: **The NICE cost-effectiveness threshold: what it is and what that means**. *PharmacoEconomics* 2008, **26**(9):733-744.
24. **Framework Document** [http://www.nice.org.uk/niceMedia/pdf/appendixB_framework.pdf]
25. National Institute for Health and Clinical Excellence: **Guide to the methods of technology appraisal**. In. London: NICE; 2008.
26. National Institute for Health and Clinical Excellence: **Guide to the methods of technology appraisal: Appendix B**. In. London: NICE; 2008.
27. National Institute for Health and Clinical Excellence: **Social value judgements: Principles for the development of NICE guidance, Second edition**. In. London: NICE; 2008.
28. **Constitution Act of Finland** [http://www.finlex.fi/pdf/saadkaan/E9990731.PDF]
29. **Primary Health Care Act** [www.finlex.fi/fi/laki/kaannokset/1972/en19720066.pdf]
30. **Health care in Finland** [http://www.stm.fi/Resource.phx/eng/subjt/healt/hcare/index.htx]
31. **Amendment of the Health Insurance Act** [http://www.stm.fi/Resource.phx/eng/orgis/board/pharmaboard/legislation.htx.i209.pdf]
32. **Government Decree on the Pharmaceuticals Pricing Board** [http://www.stm.fi/Resource.phx/eng/orgis/board/pharmaboard/legislation.htx.i221.pdf]
33. Dowie J: **Clinical trials and economic evaluations? No, there are only evaluations**. *Health Econ* 1997, **6**(1):87-89.
34. Donaldson C, Currie G, Mitton C: **Cost effectiveness analysis in health care: contraindications**. *BMJ* 2002, **325**(7369):891-894.

35. Bate A, Mitton C: **Application of economic principles in healthcare priority setting**. *Expert Review of Pharmacoeconomics & Outcomes Research* 2006, **6**(3):274-284.
36. Mauskopf JA, Paul JE, Grant DM, Stergachis A: **The role of cost-consequence analysis in healthcare decision-making**. *PharmacoEconomics* 1998, **13**(3):277-288.
37. Mauskopf JA, Earnshaw S, Mullins CD: **Budget impact analysis: review of the state of the art**. *Expert Review of Pharmacoeconomics & Outcomes Research* 2005, **5**(1):65-79.
38. Eklund K, Grip L, Israelsson B, Levin LA, Lindahl B, Wallentin L: **[2008 National Cardiology Guidelines: Prioritization requires consideration to other factors than scientific studies alone]**. *Lakartidningen* 2008, **105**(19):1408-1409.
39. Drummond M, Donaldson C: **In the land of blind decision making, is one-eyed economics the king?** *Applied health economics and health policy* 2003, **2**(2):73-76.
40. Hadorn DC: **Setting health care priorities in Oregon. Cost-effectiveness meets the rule of rescue**. *JAMA* 1991, **265**(17):2218-2225.
41. Dowie J: **No room for kinkiness in a public healthcare system**. *Pharmacoeconomics* 2005, **23**(12):1203-1205.
42. Birch S: **Making the problem fit the solution: evidence-based decision making and "Dolly" economics**. In: *Evidence-based health economics: from effectiveness to efficiency in systematic review.* Edited by Donaldson C, Mugford M, Vale L. London: BMJ books; 2002.
43. Culyer AJ: **The bogus conflict between efficiency and vertical equity**. *Health Economics* 2006, **15**(11):1155-1158.
44. Vuorenkoski L, Toiviainen H, Hemminki E: **Decision-making in priority setting for medicines--A review of empirical studies**. *Health Policy* 2008, **86**(1):1-9.
45. Sculpher M, Claxton K, Akehurst R: **It's just evaluation for decision-making: recent developments in, and challenges for, cost-effectiveness research**. In: *Health policy and economics: opportunities and challenges.* Edited by Smith P, Ginnelly L, Sculpher M. Maidenhead: Open University Press; 2005.
46. Morgan S, Barer M, Evans R: **Health economists meet the fourth tempter: drug dependency and scientific discourse**. *Health Econ* 2000, **9**(8):659-667.
47. Birch S, Gafni A: **On being NICE in the UK: guidelines for technology appraisal for the NHS in England and Wales**. *Health Econ* 2002, **11**(3):185-191.
48. Daniels N, Sabin J: **Setting limits fairly: can we learn to share medical resources?** Oxford: Oxford University Press; 2002.
49. National Institute for Health and Clinical Excellence: **Social value judgements: Principles for the development of NICE guidance**. In. London: NICE; 2005.

Acknowledgments

I would like to thank three members of the Finnish Society for Health Economics for allowing me to place the question concerning 'the use of a cost per QALY threshold in Finland' in the survey and Pfizer Ltd. for kindly providing the enabling technology to the Finnish Society for Health Economics. I also wish to thank my supervisor, Pekka Rissanen, for giving me the space to investigate this topic.