Essays on

healthcare priority setting

for population health

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Declaration

I certify that the thesis I have presented for examination for the MPhil/PhD degree of the London School of Economics and Political Science is solely my own work other than where I have clearly indicated that it is the work of others (in which case the extent of any work carried out in jointly by me and any other person is clearly identified in it).

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Statement of joint work

Chapter 3 is a joint work with Prof Alec Morton, who is one of my supervisors. I built on a preliminary conference paper prepared by Prof Morton. In this paper, Prof Morton argued for the axiomatic equivalence of two particular metrics that measure health. I contributed the work on the identification of the fundamental flaw in one of the two measures and I suggested a methodological correction to restore its normative validity (see my single-authored working paper, Airoldi 2007). I confirm that my contribution to this chapter is 50%.

Chapter 5 is also joint work with Prof Alec Morton. I led the field work, conducted the analysis and drafted the paper. I confirm that my contribution to this chapter is 90%.

Chapter 4 and 6 are joint work with the QQuIP/SyMPOSE research team of the LSE. I have been a key member of this research team since 2005 and significantly contributed to the development of the analytical framework used in these papers. For both chapters, I led the work, conducted the analysis of results and wrote the paper. The QQuIP/SyMPOSE team commented on the developing work and commented on draft version of the paper. I confirm that my contribution to these chapters is 90% each.

I confirm that I am the sole author of Chapter 1, 2, 7 and 8.

To my family

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Essays on healthcare priority setting for population health

Abstract

Healthcare priority setting is a major concern in most countries because healthcare represents a large and increasing public expenditure. Yet, there is not well established procedure that is consistently used to support those responsible for priority setting decisions.

This dissertation consists of a review of the literature and five independent essays on healthcare priority setting, focusing on the value of formal analysis to support local healthcare planners in allocating a fixed budget.

This dissertation makes both an intellectual and a practical contribution. The intellectual contribution is a synthesis of both economics and decision analysis insights. The review of the literature shows that tools grounded in health economics currently fail to contribute to local healthcare priority setting decisions because they are not practical. At the same time, tools grounded in (multi-criteria) decision analysis fail to incorporate the methodological advances of health economics and are hence theoretically weak. My thesis contributes to closing this gap.

The practical contribution is that I design, and test the value of, a process and of particular value functions that can be used by local healthcare planners within their limited resources.

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List of abbreviations

ASL   Azienda Sanitaria Locale (Local Healthcare Agency)

BoD   Burden of Disease

B-S SWF   Bergson-Samuelson Social Welfare Function

CCG   Clinical Commissioning Group

CEA   Cost Effectiveness Analysis

DALY   Disability Adjusted Life Year

DCCT   Diabetes Control and Complication Trial

GBoD   Global Burden of Disease

GCEA   Generalised Cost Effectiveness Analysis

GDP   Gross Domestic Product

LHIN   Local Health Integration Network

MAUT   Multi Attribute Utility Theory

MAVT   Multi Attribute Value Theory

MCDA   Multi Criteria Decision Analysis

NICE   National Institute for Health and Clinical Excellence

NHS   National Health System

OECD   Organisation for Economic Co-operation and Development

PBMA   Program Budgeting and Marginal Analysis

PCT   Primary Care Trust

QALY   Quality Adjusted Life Year

RCT   Randomised Controlled Trial

SWF   Social Welfare Function

YLDs   Years Lived with Disability

YLL   Years of Life Lost

WESDR   Wisconsin Epidemiologic Study of Diabetic Retinopathy

WHO   World Health Organization

1 Introduction

In 2010 OECD countries spent on average 9.5% of GDP on healthcare. Public expenditure in healthcare for these countries has continued to grow since the institution of national health services or health insurance schemes, with an annual growth rate of 4.5% in real terms in the decade 2000-2010 (OECD 2012). The financial crisis that has affected most of the OECD economies since 2008 is putting further pressures on public expenditures. Healthcare priority setting is hence a current and pressing concern for most countries.

In this dissertation I take the view that decisions around healthcare priority setting are complex (Calabresi and Bobbitt 1978, Fuchs 2011) and that hence formal analysis should play a central role in supporting such decisions (Rosenhead and Mingers 2001, Pidd 2003). In this dissertation I focus on a particular type of formal analysis, i.e. quantitative models to assess the value of alternative healthcare interventions to improve population health or models of option appraisal. These evaluations are of course just one element of a decision making process. A useful framework to locate my contribution is Mark Moore's distinction of three key questions that public managers should address (Moore 1995): (i) does this decision lead to adding public value; (ii) is there sufficient support and legitimacy for the decision? And (iii) is it operationally feasible within the organisational structure? With respect to Moore's framework, I focus on the first question, i.e. on the 'substantive value', as assessed by formal models of option appraisal and offer insights and suggestions on the other two questions when our research dovetails naturally in these domains.

The literature on the definition and measurement of substantive value for decisions in the public sector is extensive and usually referred to as 'policy analysis' or

'programme evaluation' (House 1982, Boardman, Greenberg et al. 1996, Weimer and Vining 2011). The most prominent theoretical framework for conducting these evaluations is economic theory and in particular welfare economics.

As I discuss in chapter 2, in the context of healthcare priority setting, cost-effectiveness analysis (CEA) is the recommended approach to operationalise welfare economics. The use of CEA, however, is problematic because it makes overwhelming information demands. These demands are evident in two aspects. First, CEA assumes that it is possible to describe and asses all alternative allocation of resources - so that they can be ranked according to their cost-effectiveness and they could be funded in this order until resources allow. Second, CEA should rely on good evidence to assess the health impact of particular interventions and the golden standard of good evidence is the randomised controlled trial. Evidence-based medicine plays a prominent role in the provision of the knowledge base, yet it requires interpretation to extrapolate results from laboratory settings to the general population (Morris 1997, Kelly, Morgan et al. 2010, Cartwright and Hardie 2012). Furthermore, there is an ever changing knowledge base on the benefit of particular healthcare interventions, which requires frequent revisions of past decisions.

In practice, in the face of these difficulties, CEA is generally applied to a limited subset of all possible interventions using a threshold value to identify interventions that are deemed cost-effective, as I describe in Chapter 2. This practice is exemplified by the work of the National Institute for Health and Clinical Excellence (NICE, http://www.nice.org.uk/).

The availability of CEA reports is certainly helpful in informing priority setting decisions in any healthcare system. Decisions on priorities, however, need to be made (and are routinely made) by insurers or local healthcare planners in the absence of Randomised Controlled Trials (RCTs), systematic literature reviews or CEA reports. In this dissertation I focus in particular on local planners, i.e. agencies responsible for setting priorities and allocating a fixed annual budget to meet the healthcare need of a particular populations. Examples of these agencies are Primary Care Trusts (PCTs) now replaced by Clinical Commissioning Groups (CCGs) in the English National Health Service (NHS), or Health boards in Wales, Aziende Sanitarie Locali (ASL) in Italy, Local Health

Integration Networks (LHINs) in Ontario, Canada, or Health Boards in New Zealand. They make the fundamental decisions that operationalize high level policy aims through contracts for healthcare provision.

Local healthcare planners do not have the financial resources or time to commission ad-hoc cost-effectiveness analysis studies, which in the UK per intervention cost around £150,000 and take between three and six months (Department of Health 2009). They also have limited ability to interpret available CEA reports, evidence from randomised controlled trials and to adapt them to the local context (Bryan, Williams et al. 2007, Williams and Bryan 2007, Eddama and Coast 2008). There is currently little evidence on how these local bodies arrive at their recommendations (Robinson, Dickinson et al. 2011).

In this dissertation I focus in particular on the problem of the local healthcare planners, who have to allocate a budget despite limited financial and analytical resources to conduct rigorous economic analyses and lacking good evidence.

1.1   Overview of chapters

Chapter 2 reviews the literature on policy analysis or programme evaluation in the specific case of healthcare priority setting, starting with the normative framework of welfare economics and its limitations. I focus in particular on the theoretical difficulty of aggregating benefits over people, i.e. in making interpersonal comparison of utilities and on the rejection of cost-benefit analysis in healthcare. I review two programme evaluation techniques which draw on welfare economics and which are prominent in the health economic literature: cost-effectiveness analysis (Gold, Siegel et al. 1996, Drummond, Sculpher et al. 2005) and generalised cost-effectiveness analysis (GCEA; Hutubessy, Baltussen et al. 2002, Tan-Torres Edejer, Baltussen et al. 2003). I then introduce multi-criteria decision analysis as an alternative normative framework and I present the technique of programme budgeting and marginal analysis (PBMA; Mitton and Donaldson 2001, Mitton and Donaldson 2004, Mitton and Donaldson 2004). I will argue that these techniques are either theoretically robust and impracticable, or practicable but theoretically wanting, providing a motivation for my research.

This dissertation contributes to this literature through five independent essays. In the first essay (Chapter 3) I explore how measures of health available in the literature could be used to aggregate benefits over people. In particular, I focus on the difference between taking a health or a disability perspective in assessing the impact on healthcare interventions, i.e. by assessing the value of an intervention in terms of its impact in increasing health, versus reducing ill-health or disability. I find that the method currently recommended to measure reductions in ill-health (Hutubessy, Baltussen et al. 2003) is problematic. I also prove how the problem could be overcome, turning the choice of a health or disability perspective into a stylistic rather than substantive choice.

In the second essay (Chapter 4) I argue that the standard practice of discounting costs and benefits over time in CEA reports may exacerbate myopic decision making practices. This practice assumes that current costs could be offset by future benefits or savings. Healthcare planners, however, need to demonstrate financial balance in the short term and they hence have few incentives to invest in interventions with delayed benefits. To overcome this bias, I suggest reporting information on the costs and benefits of interventions in the long run using a 'steady state' model that assumes away the delay. I illustrate the value of this procedure with the practical example of glycaemic control in patients with diabetes type 1 in England and Wales.

The last three essays (chapter 5, 6 and 7) contribute to the development of a theoretically robust, pragmatic approach to healthcare priority setting at the local level through action research with local healthcare planners of the English NHS. In these chapters I propose a socio-technical approach, which on the technical dimension employs a value function that is drawn from health economics and decision analysis, and on the social dimension relies on the practice of decision conferencing to engage local stakeholders in defining alternatives for resource allocation and assessing them with the proposed value function. Taken together, these three papers add to the literature in two ways. First, I discuss the theoretical robustness of value functions used in PBMA exercises, drawing from CEA, generalised CEA and Multi-Criteria Decision Analysis (MCDA). Second, I engage local stakeholders in a decision making process to test the pragmatic value of the approach. Over the three papers I have worked in an increasingly complex context, moving from allocating additional resources within a specific budget category (i.e. reducing risk of cardiovascular diseases), to allocating additional resources

across budget categories (i.e. mental health, cardiovascular diseases, respiratory diseases, children's health and cancer), to disinvestments within a disease area (i.e. eating disorders).

Each of the last three papers also makes a distinctive methodological contribution on its own. In Chapter 5 I explore the systematic use of a portfolio approach drawing from GCEA in building a simple, multi-criteria model in collaboration with stakeholders. In Chapter 6 I focus on the accessibility and acceptability of GCEA or CEA models. These models are difficult to understand and use for people who do not have health economic training. I test whether these obstacles could be overcome by building a formal model through a participatory approach. In Chapter 6 I consider the difficult case of generating agreement around disinvestments and test if the participatory approach I developed could support such decisions.

In the conclusion I summarise the contribution of my research to the literature.

2   Current approaches to priority setting in healthcare

In this chapter, I review the normative and prescriptive approaches for priority setting and resource allocation in the specific case of healthcare. I identify welfare economics and decision analysis as two principal normative frameworks. The normative framework of welfare economics is the reference for the prescriptive approach of social cost benefit analysis, cost effectiveness analysis, and generalised cost effectiveness analysis. Decision analysis, and in particular, Multi-Criteria Decision Analysis (MCDA) is the reference framework for Programme Budgeting and Marginal Analysis (PBMA).

I argue that the prescriptive approaches that draw from welfare economics make a careful and systematic use of welfare economic principles, as demonstrated by the lively debates in the health economic literature. I hence consider these approaches theoretically robust. However, as I will discuss below, they are impractical and those responsible for allocating healthcare resources do not use them systematically. This is disconcerting as the evaluation of prescriptive approach revolves around their pragmatic value. On the other hand, PBMA is a pragmatic tool that appears accessible to healthcare planners, yet the application of MCDA principles is erratic, as I will demonstrate. I hence consider PBMA, as currently practiced, a pragmatic but theoretically weak approach.

For clarity in Box 1 I provide the definition of 'priority setting', 'resource allocation' and 'rationing' that I use in this dissertation. Box 2 summarises the distinction of normative, prescriptive and descriptive research paradigm in decision-making research.

Box 1 Definitions: Priority setting versus resource allocation

In the literature there is a distinction between these terms.

Priority setting is the process of ranking interventions from most to least preferred, given one's goals and constraints.

Resource allocation refers to the actual allocation of resources to options or programmes (Phillips and Bana e Costa 2007).

Box 2 Research paradigms in decision-making research

The normative paradigm in decision making research. *The normative paradigm* consists in the formal representation of rational choice. In particular, it describes the decision problem in an abstract and usually mathematical form and it identifies the optimal solution(s) under well-specified assumptions about the preferences of a single decision maker. From a methodological perspective, this paradigm uses the principles of logic and mathematics. Normative validity is tested in terms of theoretical adequacy and logical coherence.

The descriptive paradigm in decision making research. *The descriptive paradigm* aims to represent observed behaviours and violations of normative principles. It develops methods from empirical research. Descriptive validity is tested through empirical observation.

The prescriptive paradigm in decision making research. The *prescriptive paradigm* is similar to, and yet distinct from, the normative one. The two paradigms are similar because they aim at informing the choice of a preferred course of action given an abstract representation of a problem. They are distinct in the modelling assumptions; normative approaches usually assume agents are "idealized, rational, super-intelligent people" (Bell, Raiffa et al. 1988, p 16) and that they can think and act coherently and rationally; coherence and rationality are defined in terms of formal axioms. Prescriptive approaches are concerned with the application of normative theories to support decisions by real, rather than idealized people. In the application of the theory, it is usually necessary to take into account that it is not possible to collect all the relevant information, that it takes time – which is also a limited resource – to analyze the information and that, if the decision maker uses the information without an explicit model, her cognitive capabilities will in general choose a sub-optimal option, i.e. one of the discarded or disregarded options would have been better able to meet her objective. Prescriptive validity is assessed in terms of pragmatic value, i.e. the ability to help people to make better decisions (Bell, Raiffa et al. 1988).

In this thesis I test both the normative validity of models to support priority setting in healthcare but also, through action research, their prescriptive validity. To test the normative validity I discuss the theoretical adequacy and logical coherence of models. To test the prescriptive validity I rely on field notes, observations and interviews with participants.

2.1 The normative benchmark of Welfare economics

2.1.1 Basic idea

Normative models of priority setting in the health economics literature draw substantially from the conceptualization of resource allocation in the economy in terms of a constrained optimisation problem. This problem can be at first seen in the abstract scenario of a central planner or a benevolent dictator who should decide how to use the available resources of the economy for production and consumption. This benevolent dictator is assumed to know the preference of individuals and the technology of firms. It is also assumed that this benevolent dictator will maximise consumers' utility and firms' profits (which are owned by consumers). The 'greatest possible satisfaction from consumption' is represented by the maximization of a *utility function* subject to the constraint of available resources. The concept of utility was introduced by Bernoulli (1954; English translation from original 1738 text) and considered to be a measure of well-being or satisfaction by economists in the 19th century which could be measured in 'utils'. This view admitted interpersonal comparison on utility, i.e. it was considered meaningful to state that 'Mark gains more utility (or utils) from one apple (or from £1) then Paula'. In the first half of the 20th century, however, some economists challenged as the idea of 'utils' as unscientific because it was not empirically testable (Robbins 1935). The key theoretical development that saved economists from this impasse was the new utility theory proposed by von-Neumann and Morgernstern (von Neumann and Morgenstern 1944). Their theory consists of a *representation theorem*, i.e. they prove that, given certain assumption on *individual* preferences, it is possible to represent them with a function that associated a higher number to preferred state of the worlds. They also prove that individual's preferences are represented by a unique function or its linear transformations. This new concept of utility, however, is insufficient to solve the

problem of the benevolent dictator, because it does not allow for aggregating utilities across different individuals.

2.1.2 Attempts to overcome the issue of interpersonal comparison of utilities

The incomparability of individual utilities may not be problematic if their aggregation is not necessary. In particular, quantity consumed and produced are usually not decided by a benevolent planner but they emerge in the market as the result of individual choices (Smith 1776). Indeed, under the institutional arrangement of perfect competition, and given some assumptions on consumers and producers behaviour, there exists an equilibrium that maximises the individual objective function of producers and consumers (Arrow and Debreu 1954). Perfect competition is an institutional arrangement to organize consumption and production which is characterized by many producers and many consumers (no individual consumer or producer can individually affect the price on the market), property rights to various assets (including labour), freedom to trade assets for other assets or goods at publicly known prices (e.g. Mas-Colell, Whinston et al. 1995). If the consumer behaves in order to maximize the satisfaction of their preferences; their preferences are *well-behaved* (complete, transitive, continuous and reflexive) and if producers behave in order to maximize profit under known technologies, the market can achieve an equilibrium in which quantity produced are equal to quantity consumed and producers and consumers both maximize their respective objective function. This outcome is Pareto optimal, which is a state of the world in which it is not possible to make at least one person better off without making at least another person worse off (Pareto 1906). The proof of the Pareto optimality of the competitive equilibrium is embodied in the two Fundamental theorems of Welfare Economics (Arrow 1951, Debreu 1951): a competitive equilibrium is a Pareto optimum; and any Pareto optimum solution could be achieved through a competitive mechanism by changing the initial allocation of resources.

The attractition of Pareto optimality is that this does not require interpersonal comparisons of utility. Let us call A and B two different ways to allocate a given quantity of consumption goods to different individuals. If allocation A is as least as preferred as B

by all individuals and at least one person prefers A to B, A is 'Pareto efficient'. The problem with Pareto optimality is that it is substantially conservative in the sense that departures from the status-quo are rarely Pareto efficient. For instance, if all the resources of a given community are owned by a single misanthropic individual and the rest of the community is doomed to starvation, most observers would argue for a redistribution of resources, but the redistribution would not be Pareto efficient because the rich person would be 'worse off'. Pareto optimality, hence, seems excessively limited. Two different techniques have been proposed to compare two allocations which are both Pareto optima. One is the compensation principle proposed by Kaldor, Hicks and Scitovsky and the other is the use of a social welfare function proposed by Bergson and Samuelson.

Kaldor, Hicks and Scitovsky proposal is an extension of the Pareto criterion (Kaldor 1939, Hicks 1940, Scitovsky 1941). Let us assume that A (status quo) and B are two Pareto optimal states. The move from state A to state B will leave some people better off (the gainers) and others worse off (the losers). B is preferred to A if the gainers could compensate the losers and still be better off and the losers would not be able to bribe the gainers not to undertake the change. It is controversial, however, to recommend an option based on a compensation that is only potential and may not be in fact be paid. The compensation is in fact measured in monetary terms, i.e. money that gainers are willing to pay to losers. Because of decreasing marginal utility of wealth, wealthier individuals will be willing to transfer relative more resources for the same change in utility compared to what they would do, would they be poorer. As a result, their preferred options are more likely to be favoured through CBA. This is not problematic if a monetary transfer actually takes place to compensate the losers. In fact, if the compensation takes place, the outcome is Pareto optimal and it would not be necessary to invoke the Kaldor, Hicks and Scitovsky principle. The value of their compensation principle lies precisely in the hypothetical nature of the compensation. It seems socially unfair, however, to judge a state of the world superior to another on the basis of hypothetical compensations.

The alternative solution of a social welfare function (B-S SWF) proposed by Bergson and Samuelson (Burk 1938, Samuelson 1947, chapter 8, Bergson 1954) consists in defining an explicit preference ordering for different distribution of utility among

individuals, with the individual utility representing individual's preferences over alternative allocations of resources. Several authors maintain that the approach requires an ethical observer to engage in interpersonal comparison of *cardinal* utilities (Kemp and Ng 1976, Parks 1976, Mueller 2003). This view is however debated by Samuelson, who confirms the need for an ethical observer to engage in interpersonal comparisons, but he proves that ordinal preferences are sufficient and that there is no need to invoke cardinal intensities (Samuelson 1977). A B-S SWF transfers the concept of utility and indifference curves from the individual preferences over goods to society's preferences over distribution of utility. The shape of the SWF 'indifference curves' requires preferences derived from ethical judgments on distribution to be explicit (Bergson 1954). The concept is powerful and attractive in the normative domain, but it is unclear how the analyst should specify such a function in supporting decisions on behalf of society (i.e. in a prescriptive approach).

2.2 Prescriptive approaches grounded in Welfare economics

In this section I review three approaches that operationalize Welfare economic principles in assessing policy options: Cost-Benefit Analysis (CBA; or Social Cost Benefit analysis, SCBA), Cost Effectiveness Analysis (CEA) and Generalised Cost Effectiveness Analysis (GCEA). I will focus in particular on the application of these prescriptive approaches in the context of healthcare priority setting.

2.2.1 Cost Benefit Analysis to priority setting in healthcare

Given the difficulties in making a B-S SWF operational, the dominant economic approach to inform resource allocations relies on the normative model of the Kaldor-Hicks-Scitovsky potential compensation principle. The operational technique to apply this model in practice (i.e. prescriptively) is Cost Benefit Analysis (or CBA; e.g. Layard and Gleister 1994), which is currently the recommended approach to resource allocation in the British Government (HM Treasury 2003). In CBA options are appraised by summing the monetary value of gains to gainers and losses to losers and the option with the highest value is recommended. As discussed in the previous section, the model does not require the compensation to take place. Although this has been criticised, one may argue that the role of the economist is to indicate the superiority of an option to its

alternatives (i.e. if the compensation would be implemented, the chosen option would be Pareto efficient) and let politicians decide if the compensation should be imposed (Scitovsky 1951).

The allocation of scarce resources to healthcare for a defined population inevitably determines some 'winners' and some 'losers': if the same resources are invested in portfolio of investments A compared to an alternative portfolio B, some people will be better off in A rather than B and some will be worse off. CBA is an approach to assess if the net benefit of choosing A over B is positive, by estimating if the gain to the 'winners' would be sufficiently great to compensate the 'losers'.

2.2.2   Limitations of Cost Benefit Analysis to priority setting in healthcare

Despite the existence of a clear normative framework, its operationalization through CBA has some limitations. In particular, the use of 'willingness-to-pay' to attribute a monetary value to policy outcomes is normatively weak because it can lead to intransitive preferences, it depends on the current distribution of wealth (hence raising equity concerns) and it is not clear whose preferences should be included or excluded (Boardman, Greenberg et al. 2011).

In the case of evaluating alternative healthcare interventions or policies, furthermore, it is necessary to attribute a monetary value to lives saved or health gain. Techniques to elicit the maximum willingness to pay to reduce *the risk of* death or injuries are available (for a theoretical discussion, see Schelling 1968, Jones-Lee 1976, for its application in Central Government appraisals and evaluations see HM Treasury 2003, p 61-62), although there is no agreement of what that value should be and different monetary values are used in different contexts (Tengs, Adams et al. 1995). In the case of healthcare interventions which may benefit a named individual, however, the monetisation of the benefits is morally objectionable because of the 'rule of rescue', i.e. the moral imperative which demands that everything possible should be attempted to rescue a life regardless of the costs (Hadorn 1991, Mooney and Wiseman 2000). Although it is possible to derive a mathematical equivalence between saving a life and reducing the risk of a fatality (Mason, Jones-Lee et al. 2009), the 'rule of rescue' claims

that the two contexts are substantially different and no monetary value can compensate 'losers', when the substance of their loss is their death.

The potential compensation of 'losers' may be particularly repulsive or plainly impossible in healthcare as 'losing' might lead to death. For instance, let us suppose that two people, one wealthy and one poor individual, have the same age and disease. One pill exists which can extend the life of people with this disease by two months on average, but there is only one dose available. The wealthy individual may provide a higher 'willingness to pay' for extending his life by two months than a poor individual. According to the Pareto principle, the wealthy individual should compensate the poor for dying so that they will be both better-off. Most people find this conclusion repulsive (Sandel 2012).

In the absence of an agreed monetary value of life and of health, the recommended prescriptive approach formulated by health economists is Cost Effectiveness Analysis (or CEA; e.g. Gold, Siegel et al. 1996), which I discuss in the next section.

2.2.3 Cost-effectiveness analysis for priority setting in healthcare

In CEA the problem of those responsible for allocating resources could be represented as follows (adapted from Torrance, Thomas et al. 1972):

Equation 1

$$\max_{\mathbf{X}} F(x_i)$$

$$F(x_i) = \sum_{i=1}^{n} e_i x_i;$$

$$s.t. \sum_{i=1}^{n} c_i x_i \leq B;$$

$$0 \leq x_i \leq 1; \forall i = 1, 2, ..., n$$

$$\sum_{i \in I_j} x_i \leq 1; \forall j = 1, 2, ..., p$$

where x_i is the quantity of intervention i provided and could range from 0 (not provided) to 1 (provided to the whole population who might benefit), e_i is the effectiveness or benefit of the intervention. The benefit is usually assumed to be the

individual's health *or* the utility derived from being in a particular health state, measured in terms of longer life expectancy adjusted for quality of life using metrics such as the Quality Adjusted Life Year or QALY (Williams 1985). The QALY metric results from the product of life duration expressed in years and quality of life represented on an interval scale ranging from 0 to 1, where 0 correspond to the quality of life equivalent to being dead and 1 to that of 'full health'. One QALY represents *the equivalent of* a year spent in full health. *B* represents the available resources and c_i the cost of providing the intervention if provided to the whole population who might benefit. To maximise *F*, the decision maker selects a portfolio of interventions *X*, which is a vector representing the different quantities *x* for each intervention *i.*

The interpretation of QALYs in terms of health or in terms of *utility derived from* being in a particular health state is fundamental from a theoretical standpoint. In the work that led to the original development of CEA, the objective function represented the objective of those responsible for allocating resources (e.g. a healthcare planning agency). Pioneers in the technique came from the operational research and engineering community. They argued that the objective of these agencies should be the maximisation of health and they proposed metrics to measure it (e.g. Fanshel and Bush 1970, Quade 1971). Later, economists provided a normative framework to CEA, arguing that the objective function should represent social welfare (usually called a Health-Related Social Welfare Function, e.g. Garber and Phelps 1997, Dolan 1998) and that welfare economics provides the ethical framework for ordering alternative states of the world. This fundamental theoretical distinction is currently referred to as 'welfarism' or 'non welfarism' as I summarise in Box 3.

Box 3 Welfarism, extra-welfarism

> **Welfarism**
>
> Welfarism entails "judging the goodness of states of affairs only by utility information" (Sen 1986; p 111). The objective function (e.g. a social welfare function) is hence a function of individual utilities.
>
> **Extra-welfarism**
>
> In extra-welfarism, the objective function may include elements beyond utility information. For instance, some goods may have a special status (e.g. 'merit goods' in Musgrave 1959) and increasing their availability is 'good' regardless of the utility they generate. Health and healthcare may be considered to have such characteristics. Amartya Sen's concept of 'capabilities', i.e. focusing on what a particular good or state of the world *enables* an individual to be or to do rather than focusing on the emotional response to that good or state as captured by utility (Sen 1980).

The use of metrics such as the QALY to express the objective function however is highly controversial. Some authors argue that the QALY tools fail to make the critical distinction between "X is *healthier* than Y" and "X's health state is *better* than Y's health state" and simply assumes that the two statements are equivalent (Sen 1979, Hausman 2006). Evidence on the choice between radiation and surgery to treat lung cancer, shows that people prefer surgery, even though they would be healthier, in terms of five-year survival rates, with radiation (McNeil, Wiechselbaum et al. 1978) and hence confirm Hausman's concern.

In the face of these critiques, some authors maintain that health is a valuable good per se and that an individual's level of health affects his welfare or utility. One may argue that more health, *ceteris paribus*, is to be preferred to less health and that the role of a healthcare system is simply to produce health, even though this is not a direct measure of individual utilities. This is the core idea of extra-welfarism (Culyer 1991, Brouwer, Culyer et al. 2008), which provides a potential justification for QALY maximisation, but places CEA outside mainstream normative economics.

An alternative route to prove the normative foundations of CEA in healthcare is to demonstrate its link with CBA, which derives its normative foundations in the Pareto principle through the Kaldor-Hicks-Scitovsky compensation mechanism. Johannesson proved that the two approaches are equivalent if the willingness to pay for one QALY is

constant for all individuals in society (Johannesson 1995). This assumption has been however challenged by Dolan and Edlin, who proved the impossibility of a link between Cost Effectiveness and CBA if we assume (i) expected utility theory; (ii) QALYs as a measure of individual utilities; (iii) illness affects the ability to enjoy consumption (Dolan and Edlin 2002). In response to the argument of Dolan and Edlin, Hansen and her colleagues claimed that the core issue is the aggregation of the benefit derived from healthcare programmes (Hansen, Hougaard et al. 2004). They argue that CEA is a constrained optimisation framework and its normative validity resides in how the benefits are expressed and aggregated in the objective function. Given the impossibility of making interpersonal comparison of utility directly, they advocate the use of CEA within a *Decision Making Approach*: the analysis should "assist the decision-maker in making choices that are consistent with his (that is, the decision-maker's) objective"(Sugden and Williams 1978; p235).

The Decision Making Approach can be attributed to Alan William's reflections on the essential core of microeconomics for supporting public decision making (Williams 1972, Sugden 2007). In William's view, this core is 'constrained maximisation', which requires clarity on what should be maximised and on the constraints in operation. In this view, the approach is value-free, in the sense that ethical judgments should not be assumed by it. The technique is simply a tool to show the logical implications of particular decisions given particular, stated objectives. In the Decision Making Approach the ethical judgments are still necessary, but they are those of people responsible of making a decision on the basis of the analysis (Williams 1972).

Reducing microeconomics (and hence welfare economics) to the essential core of constrained maximisation as Williams' did may be criticised as naïve, because the theory of production is simplistic, especially in its application to the production of health (Jacobs, Smith et al. 2006). Already in 1963, Kenneth Arrow pointed out that in healthcare a market is unlikely to be efficient, in particular, for the information asymmetry between patients, physicians and payers (Arrow 1963).

In William's reflections, as well as those of other CEA proponents, the model should aid and inform rather than prescribe a particular course of action (Culyer 1991, Williams 1991, Johannesson and Weinstein 1993, Gold, Siegel et al. 1996, Drummond, Sculpher et

al. 2005) and the failed experiment of the Oregon Health Plan in the 1990s is often quoted to prove that a mechanistic use of CEA will face harsh rejection and lead to counterintuitive results (Hadorn 1991). The constrained maximisation framework can be an acceptable approximation. From a technical perspective it has been recognised that the provision of healthcare is characterized by (partial) decomposability, i.e. most inputs are predominantly used for a specific disease and type of patients, and it is hence possible to envisage separate 'product lines' (Harris 1977). It is hence maintained that the model could still offer useful insights to inform those responsible for allocating resources and that the use of the normative model of welfare economics, although imperfect, is a valuable guide to interpret results of CEA and to develop the tool further (Garber, Weinstein et al. 1996). Within the Decision Making Approach, hence, the role of the 'decision makers' is not simply that of defining the objectives and the constraints, but also to interpret the results of a model to arrive at recommendations for policies.

2.2.4 Limitations of CEA

2.2.4.1 The threshold problem

The formulation in Equation 1 is a conceptual framework which is used to structure rather than conduct the analysis at the level of the system as a whole, because it would be impracticable to specify all its parameters. In practice the analysis is conducted 'at the margin', i.e. by assuming that the current allocation of resources is efficient and by assessing the value of departing from the current allocation using 'incremental cost-effectiveness ratios' (ICER). The ICER is calculated by comparing a new candidate programme (e.g. a new pharmacological treatment or surgical procedure) with its alternatives through pair-wise comparisons. Alternatives which are dominated (i.e. cost more and produce less benefits compared to other options or their combination) are excluded from the analysis. Non dominated alternatives are ranked according to increasing costs (or, equivalently, benefits), and each alternative is evaluated compared to the next in the rank order. The evaluation consists of taking the ratio of the difference in costs and the difference in benefits of the two options. When benefits are measured in QALYs, the ratio is simply referred to as "cost per QALY" (or "cost/QALY"). A 'low' ratio indicates that each additional £1 invested to provide the more expensive interventions, provides a relatively 'high' additional benefit and it is hence 'good value for money'. In this approach, it is clearly necessary to specify a critical value under which the ICER signals good value for money.

The critical value of the ICER, in terms of problem represented in Equation 1, is the Lagrangean multiplier associated with the budget constraint, i.e. programmes could be ranked according to their ICER, from lowest to highest, and funded according to this order. The ICER of the last affordable project would hence be the critical value of the ICER or, simply, the 'critical ratio' (Johannesson and Weinstein 1993, Stinnett and Paltiel 1996).

It is not feasible, however, to specify Equation 1 fully in practice, because it is beyond the capacities of analysts to identify all current and possible interventions (or portfolios of interventions), their costs and health consequences. The representation of the problem as in Equation 1 is indeed an instance of the rationalistic approach of

'rational comprehensive planning', which cannot be implemented in decision-making (Lindblom 1959, Etzioni 1967, Ackoff 1979).

The inability to specify Equation 1 exhaustively implies that the critical ratio cannot be derived mathematically. This is particularly problematic for bodies such as the National Institute for Health and Clinical Excellence (NICE) in England, which routinely uses the tool of CEA and cost/QALY estimates to issue national recommendations prescribing the provision of new intervention. NICE tends to recommend the provision of most interventions below a critical ratio of about £30,000 (Devlin and Parkin 2004), but the rationale of this ratio is unclear.

It is argued that the ratio could represent the willingness to pay of the English public for a QALY, but this has not been proved (although recent attempts to assess its value made progress in this direction and produced preliminary estimates between £20,000 and £70,000 pounds; Mason, Jones-Lee et al. 2009). Furthermore, it is not clear if the current funding of the National Health Service is adequate to finance all interventions recommended by NICE or if its recommendations are crowding out more cost-effective interventions currently funded (Iqbal, Price et al. 2006, Martin, Rice et al. 2008, Appleby, Devlin et al. 2009).

2.2.4.2   Accessibility and acceptability concerns

The National Institute for Health and Clinical Excellence (NICE) in England is a notable example of the success of CEA to inform resource allocation in practice. NICE was set up in 1999 to ensure homogeneity of healthcare provision for equal need across the country. Bryan and colleagues recently investigated the ability of NICE to inform and influence policy and found that CEA is used systematically to support the Institute's recommendation and that, over time, members of the appraisal committee have developed technical skills to interpret the analysis with more confidence (Bryan, Williams et al. 2007). The QALY metric is praised for offering a common unit of measurement across different interventions and for combining both length and quality of life.

At the same time, however, members of the committee highlighted issues with the accessibility and the acceptability of CEA (Bryan, Williams et al. 2007, Williams and Bryan

2007). In terms of accessibility, the details of CEA models are difficult to understand even for NICE committee members who are national clinical experts. Committee members lament that results are presented in a very technical format, making the interpretation difficult. In terms of acceptability, the QALY metric, although valuable, fails to capture some relevant criteria in practical applications (e.g. the irreversibility of a condition). Also, perceived problems with CEA are its failure to consider the opportunity cost explicitly (i.e. the health benefits which are forgone by funding the intervention under investigation rather than an alternative intervention and could hence be crowding out more cost effective, yet not assessed, interventions), and that other criteria, such as equity, are taken into account informally and hence it is not clear if this is done in a consistent way for all recommendations. Based on the analysis, Bryan and colleagues recommend to CEA analysts to provide information which (i) is seen as relevant by end-users; (ii) is appropriate for the decision at hand, taking into account contextual factors; (iii) can inform implementation in a complex decision making environment.

The problem of ignoring opportunity cost and of crowding out has also been highlighted with respect to the work of NICE by health economists (Birch and Gafni 1992, Donaldson, Currie et al. 2002, Gafni and Birch 2006, Birch and Gafni 2007). By using a critical value of £30,000 per QALY (although it is acknowledged that this is not done mechanistically), NICE is in fact assuming that the NHS will finance the mandatory recommendations by disinvesting from interventions with a higher cost/QALY. This assumption has however no empirical validation (Iqbal, Price et al. 2006, Martin, Rice et al. 2008, Appleby, Devlin et al. 2009) and it is hence not clear if NICE is forcing the NHS to disinvest from interventions which are in fact more cost effective.

The analysis of the use of CEA to inform resource allocation at local level shows an even more disappointing picture (Ross 1995, Drummond, Cooke et al. 1997, Sloan, Whetten-Goldstein et al. 1997, Bryan and Brown 1998, Duthie, Trueman et al. 1999, Drummond and Weatherly 2000, Kernick 2000, von der Schulenburg 2000, Eddama and Coast 2008). This evidence indicates that CEA is not used locally because efficiency is not the only relevant criterion, there is a lack of expertise to interpret and understand the results, that analyses are based on poor data and are not timely, the conclusions from the analysis are often not actionable because models take a long term perspective but

the organisations tasked to implement CEA recommendations have short term financial constraints and are not able to free up the necessary financial or managerial resources.

2.2.5   Generalised Cost-Effectiveness Analysis

Generalised Cost Effectiveness Analysis (GCEA) is an approach proposed by the World Health Organisation (WHO; Hutubessy, Chisholm et al. 2003, Hutubessy, Baltussen et al. 2003). The approach aims to overcome economic criticism to the WHO and World Bank work on the Global Burden of Disease (GBoD). GBoD reports assess the extent of ill health for world regions (World Health Organization 1990, Murray, Vos et al. 2012) measured in Disability Adjusted Life Years (DALYs). DALYs and the Burden of Disease work attracted much criticism in the past because they neither consider the impact of intervention on reducing the Burden of Disease, nor the costs of these interventions (Anand and Hanson 1997, Williams 1999, Bevan and Hollinghurst 2003). In order to set priorities for resource allocation, however, it is necessary to focus on interventions, their benefits and their costs. The fact that there is a massive Burden of Disease associated with a particular condition does not justify, per se, investing large amounts of resources in tackling the burden because there might not exist effective interventions or, if they exist, they may be too costly (Hollinghurst, Bevan et al. 2000). GCEA assesses both the costs and benefits from interventions (in 'averted DALYs) and hence tackles these criticisms as I explain in detail below.

GCEA is based on the general constrained optimisation problem outlined in Equation 1. In contrast to CEA, however, its proponents emphasise the need to show the scale of benefits and costs from implementing specific interventions. In GCEA the objective function and the budget constraint are modelled explicitly in order to confront those responsible for allocating resource with the opportunity cost of their recommendations (Hutubessy, Chisholm et al. 2003, Tan-Torres Edejer, Baltussen et al. 2003). In practice, GCEA is not conducted for the overall healthcare budget and the set of all possible interventions which the healthcare planner may consider. The base of the analyses conducted to date is the disease (results are available online at http://www.who.int/choice/interventions/en/ for nineteen different diseases and fourteen country areas): a disease model to simulate a prevalent and incident population produces estimates of the current Burden of Disease (BoD) measured in Disability Adjusted Life Years or DALY (World Bank 1993, Murray 1996, Murray, Vos et al.

2012), interventions to prevent or treat the disease are then modelled in isolation and in combination to estimate the avoidable DALY and the costs of providing the intervention at the population level (e.g. a country or a region). GCEA ranks interventions according to their ICER (the cost per DALY averted).

GCEA proponents highlight that the key difference of their approach compared to the standard cost per QALY is not the use of the DALY metric and indeed they admit QALY as a valid substitute (Tan-Torres Edejer, Baltussen et al. 2003, p 65). The value of the GCEA is that results are explicitly presented in terms of the benefits and costs at the population level as well as in terms of ICER and hence facilitate those responsible for allocating resources to integrate the analysis with other concerns. In particular, health planners will be interested in achieving other goals such as health equity and system responsiveness (Hutubessy, Chisholm et al. 2003), or may need to take into account other constraints, such as managerial and ministerial attention (Murray and Lopez 2000).

There seem to be opportunities for the GCEA model to contribute to the further development of the cost per QALY framework because of the recognised limitation in the normative foundation of CEA and the emphasis in the role of the analysis to provide a systematic framework and a language for an informed discussion. To facilitate the discussion, the analysis should be complemented with transparent information about the costs and consequences of alternative policies (Mauskopf, Paul et al. 1998, Kernick 2000, Cooper, Brailsford et al. 2006, Williams and Bryan 2007). An example of the readiness of CEA proponents to size up benefits and costs at the population level is the latest guidance to CEA for technology appraisals issued by NICE, which states that "an estimate of the resulting health impact (for example, QALYs or life-years gained) in a given population should ideally be attempted" (National Institute for Health and Clinical Excellence 2008, p 50) and the costing tools which routinely accompany all new NICE recommendation to estimate their costing implications for different geographical locations.

2.2.6 Limitations of GCEA

The limitations I identified for CEA also apply to GCEA. Contrary to CEA, the generalised approach admits openly that health benefits could be aggregated across

individuals. The results of the analysis are hence presented at the population level. This is done implicitly in CEA through the 'a QALY is a QALY is a QALY', that is the principle that a unit of benefit (one QALY) is worth the same regardless of who receives it, and the implicit assumption of constant returns of scale in health technologies. These two principles allow CEA analysts to present estimates for the 'average' patient and yet make recommendations for allocating budgets over a population because the benefit to the 'average' patient can hence be multiplied by the number of patients.

2.3   The normative framework of Multi-Criteria Decision Analysis

2.3.1   Basic idea

Multi criteria Decision Analysis (MCDA) has been an active area of study among operational researchers since the 1960s. MCDA considers the problem of assessing alternative states of the world (e.g. the consequences of alternative courses of action) in terms of conflicting objectives or criteria.

The term covers an umbrella of techniques such as data envelopment analysis (DEA), outranking, goal programming, multi attribute utility theory (MAUT) and multi attribute value theory (MAVT). In this section I will focus in particular on MAUT and MAVT, which are reference frameworks for Programme Budgeting and Marginal Analysis, a widely used prescriptive approach to support healthcare priority setting.

The fundamental text which defined the standards of MAUT and MAVT is Keeney and Raiffa's book on 'Decisions with Multiple Objectives' (Keeney and Raiffa 1976). These standards consist of logically coherent procedures for representing preferences and value trade-offs over conflicting objectives under conditions of certainty (in the case of MAVT) or uncertainty (in the case of MAUT).

The theoretical basis of MAVT and MAUT is that of measurement theories of value (Suppes and Zinnes 1963, Krantz, Luce et al. 1971) and utility (von Neumann and

Morgenstern 1944, Savage 1954). A particular contribution to the normative foundations of MAVT is Dyer and Sarin's theory of *measurable multi-attribute value function* (Dyer and Sarin 1979), which provides the conditions for ordering the differences in strength of preferences among pairs of alternatives.

Decision analysis is 'value-free' in the sense that the objectives and constraints represented in a model should reflect the objectives and constraints of a 'decision maker'. The underpinning philosophy of decision analysis is very similar to that of the Decision Making Approach in health economics.

2.3.2 Additive and multiplicative utility and value models

MAUT and MAVT models represent the preferences and value judgments of decision-makers through two primary components: preferences in terms of each individual criterion and an aggregation model. The most used aggregation models are additive or multiplicative. Their typical formulation for MAUT is provided in Equation 2 and Equation 3 below, respectively. The formulation for MAVT is similar, but preferences are represented by utility functions *v* in place of value functions *u*.

Equation 2

$$u(a) = \sum_{i=1}^{m} w_i u_i(a)$$

Equation 3

$$u(a) = \prod_{i=1}^{m} [u_i(a)]^{w_i}$$

In these equations, *a* is an alternative and *u(a)* a number that represents the 'utility' of such alternative, such that *u(a)>u(b)* if and only if *a* is preferred to *b* - and *u(a)=u(b)* if and only if there is indifference between *a* and *b*; preferences are expressed in terms of *m* criteria indexed by $i \in [1,m]$; $u_i(a)$ is the partial utility function that represents preferences between alternatives in terms of criterion *i*; and w_i is the weight that captures the relative importance of criterion *i* and hence the value trade-off.

The choice between an additive and a multiplicative model depends on the characteristics of the multiple attributes. For the purpose of this dissertation, I emphasise the need for assuming *preferential (or utility) independence of the criteria*. In MAUT this assumption requires indifference between acts with identical marginal (single-attribute) probability distributions (Keeney and Raiffa 1976, von Winterfeldt and Edwards 1986). Keeney and Raiffa (p 226) propose a simple test to verify preferential independence in the case of two attributes *Y* and *Z*. Let us denote a point in the bi-attribute space as (*y*,*z*), where $y^0 \leq y \leq y*$ and $z^0 \leq z \leq z$. Let us now fix *z* to z^0 and consider a 50-50 gamble between (y^1, z^0) and (y^2,z^0). Let us suppose that the certain equivalent for that gamble is *ŷ*. Will the certain equivalent change, if we fix *z* to a different level, say *z'*? If not, and if this condition holds for any fixed y^1 and y^2, then the attribute *Y* is utility independent of attribute *Z*.

Utility independence is a necessary assumption for representing preferences with an additive multi-attribute utility function (Fishburn 1965). Similarly, preferential independence is a necessary condition for representing preferences with an additive value function (Dyer and Sarin 1979). If utility or preferential independence does not hold, it is necessary to use a different functional form to aggregate utilities or values over attributes, for instance a multiplicative function (or more complex functions).

2.4 Prescriptive frameworks drawing on MCDA

There are several ad-hoc priority setting decision-aids which appear to draw on MCDA, and in particular MAUT or MAVT. The most notable example is Programme Budgeting and Marginal Analysis (PBMA), which I will discuss in more detail below. Many of these tools have been developed by consulting firms. An unpublished review conducted by Nigel Edwards in 2011 for the Health Foundation identified several 'multi-criteria' tools in use in England, such as the HELP tool by Matrix knowledge (http://help.matrixknowledge.com), Prioritise with Care by PriceWaterhouseCoopers, as well as other proprietary tools developed by McKinsey & Co and United Healthcare (Nigel Edwards, personal communication). Edwards' review points out that most of these tools are very limited in uptake, with the exception of PBMA.

2.4.1 Programme Budgeting and marginal Analysis (PBMA)

PBMA is a pragmatic approach to aid local decision makers (Mitton and Donaldson 2004). PBMA has been used over the past thirty years (Mitton and Donaldson 2001). In practice PBMA covers a variety of different practices, with a similar process but different formulae to evaluate alternatives quantitatively.

PBMA follows the Decision Making Approach philosophy in the sense that the process is deliberative and engages local key stakeholders in a systematic process to formulate their objectives, their options and to explore the value of alternative policies. This is accomplished through several steps: to determine the aim and scope of the analysis, to identify where resources are currently spent, to form a panel of decision makers including local stakeholders, to determine locally relevant criteria for decision-making, to identify options for investment and disinvestment, to assess options against the set criteria, to validate results and recommend resource re-allocation (Mitton, Patten et al. 2003).

The concepts of 'marginal analysis' and 'opportunity cost' are central to PBMA. 'Marginal analysis' consists in focusing on decisions 'at the margin', i.e. in considering initiatives for funding additional interventions, it is necessary to identify disinvestments from current activities in order to release the necessary financial resources. It is hence an 'incremental' approach, rather than a 'rational comprehensive approach'. The concept of 'opportunity cost' is related to that of 'marginal analysis' in that participants in a PBMA exercise compare the costs and benefits of introducing a new interventions from a 'wish list' to the forgone costs and benefits of discontinuing interventions from a 'disinvestment list' (Kemp, Fordham et al. 2008). This is in stark contrast to the mainstream approach of CEA, in which, as we discussed above, the opportunity cost is not explicitly considered (Donaldson, Currie et al. 2002).

The quantitative assessment of the benefits of the proposed options consists in generating a multi-attribute benefit score. Those responsible for allocating resources generate a list of criteria that are locally relevant. To combine the different criteria in an overall benefit score, PBMA proponents recommend to use multi-attribute utility functions (Peacock, Richardson et al. 2007, Peacock, Mitton et al. 2010).

The deliberative nature of the approach and the systematic consideration of additional criteria have potential for tackling the acceptability challenges of CEA. A recent systematic assessment of PBMA in England confirms this (Kemp, Fordham et al. 2008). The evaluation by Kemp and colleagues highlights that participants appreciated the ability to incorporate multidisciplinary inputs in a transparent, objective and systematic process; it enabled them to focus on health gain, and it was possible to use available information, although imperfect, to generate an overall benefit score which participants could use as a basis for the discussion.

2.4.2   Limitations of PBMA

The PBMA literature provides a clear description of the process in terms of facilitating a structured discussion and in terms of particular steps that should be accomplished. Contrary to CEA, however, there is relatively little guidance and discussion on choosing a particular form for the quantitative multi-attribute utility or value function (Wilson, Rees et al. 2006, Peacock, Richardson et al. 2007).

Many papers reporting applications of PBMA do not provide details of the quantitative assessment. In part this is justifiable because these value functions are defined ad-hoc by local stakeholders and they are hence not generalisable. By reviewing the literature, I identified eight papers or reports which indicate the set of criteria used and provide some details on the form of the value function. These are summarised in Table 1. As expected, the set of criteria and value function vary in each case.

The ad-hoc definition of the set of criteria and the value function poses a challenge to the application of PBMA, because the normative validity of the chosen value function ought to be verified every time.

In reviewing the criteria and value function reported in Table 1, it is possible to identify violation of normative principles of (multi-criteria) decision analysis. For instance, Mitton, Patten et al. (2003) and Tony, Wagner et al (2011) suggest scoring proposed interventions against multiple criteria, taking a weighted sum of the scores (with higher weights for more important criteria), and ranking interventions from the highest to the lowest overall score to determine priorities for funding. There are two violations of recommended MCDA practice. First, the weighting procedure of asking

direct importance weights has been discredited in favour of 'swing weights'(Edwards and Barron 1994). Questions of direct importance weights are poorly defined and open to different interpretation. On the contrary, 'swing weights' are scaling factors to discriminate the extent to which scores assigned to a particular criterion discriminate between alternatives (Belton and Stewart 2002). Second, in the context of resource allocation the MCDA literature clearly recommends ranking alternatives according to their cost-effectiveness, defined as the ratio between a (multi-criteria) benefit score and costs (Goodwin and Wright 2004, Kleinmuntz 2007). Instead, Mitton, Patten et al. (2003) and Tony, Wagner et al (2011) include cost-effectiveness as one of the five criteria of an additive value function.

Peacock, Richardson et al (2007) and Wilson, Peacock et al. (2008) challenge the PBMA community to be more open in discussing the choice of particular value function in order to build academic and professional consensus on issues of normative validity on one hand, and to offer clearer practical tools to practitioners. I did a bibliographic search of all the publications which cited Peacock, Richardson et al (2007) and Wilson, Peacock et al (2008). Out of the 15 referencing papers I found and reviewed, only one discusses the normative validity of multiattribute value functions in models to support healthcare priority setting decisions (Thokala and Duenas 2012). This paper concludes that the technique is insufficiently developed to grant its wider adoption. Other authors have attacked PBMA-like approaches contending that the apparent simplicity of a scoring and weighting procedure gives an illusion of transparency, but in facts obfuscates the decision at hand (Mullen 2004).

Table 1 Examples of criteria and value functions published in the PBMA literature

Source	Criteria	Value function
Mitton, Patten et al. (2003)	Access/capacity Appropriateness Sustainability/cost-effectiveness System integration Clinical/population health effectiveness	Additive scores. Interventions ranked by aggregate benefit scores, funding allocated in this order until constraint met
Wilson, Rees et al. (2006)	Access and equity Effectiveness Local and National priorities Need Prevention Process Quality of life	Additive, prioritization based on benefit/cost ratio
Peacock, Richardson et al. (2007)	Individual health Community health (i.e. community ownership and control of the programme; and long term sustainability) Equity (i.e. accessible and addressing the need of most disadvantaged groups)	Part multiplicative, part additive value function (the score for individual health is multiplied by a weighted sum of the other two criteria). The paper is focused on the use of Multi-Criteria decision analysis and does not explore how the priority ordering for resource allocation should be calculated

Source	Criteria	Value function
Robson, Bate et al. (2008) Similar lists in Kemp and Fordham (2008) and Baughan and Ferguson (2008)	Better outcomes (sub-criteria: contributes to local action plan; meeting outcomes for 'Every Child Matters'; impact on current and future need) Increased participation (sub-criteria: user centered; user involvement; feedback; community consultation) Improved working together (sub-criteria: mental health service delivery; Appropriate service partners; Common assessment framework; Appropriate workforce; recruitment; knowledge and expertise; supervision and support; local children services workforce strategy) Improved quality of services (sub-criteria: Experience; Risk management; Location of service; Professional standards; Social marketing)	Additive (using swing weights). prioritization based on benefit/cost ratio
Tony, Wagner et al. (2011)	Disease severity Size of population affected by disease Clinical guidelines Comparative interventions limitations Improvement of efficacy/effectiveness Improvement of safety & tolerability Improvement of patient reported outcomes Public health interest Type of medical service Budget impact on health plan Cost-effectiveness of intervention Impact on other spending Completeness and consistency of reporting evidence Relevance and validity of evidence	Additive scores. Interventions ranked by aggregate benefit scores
Thokala and Duenas (2012)	Cost effectiveness Equity Innovation Patient compliance Quality of evidence	Additive scores. Interventions ranked by aggregate benefit scores

2.5 Summary

In this dissertation I adopt the distinction between normative, prescriptive and descriptive approaches in decision making research. I identify two normative approaches for informing healthcare priority setting decisions, which are welfare economics and multi-criteria decision analysis.

Welfare economics is the dominant framework for health economists. The prescriptive approaches which attempt to operationalise the welfare economic framework in healthcare priority setting are cost benefit analysis (CBA), cost effectiveness analysis (CEA), generalised cost effectiveness analysis (GCEA).

Among the techniques which draw from welfare economics, CEA has been particularly influential through the work of the National Institute for Health and Clinical Excellence (NICE). NICE's work has stimulated a rich academic literature to develop CEA techniques which adhere as much as possible to the underlying theoretical framework of welfare economics. To maintain the theoretical robustness, it is however necessary to use a threshold value of cost-effectiveness, over which there is no consensus and no sound basis for choosing. CEA is also of little practical value for local planners who do not have the necessary resources. Furthermore, even available reports also have limited use because they are difficult to understand for non health-economists.

Some health economists have proposed a more pragmatic approach which uses the economic principles of marginal analysis and opportunity cost, i.e. Programme Budgeting and Marginal Analysis (PBMA). PBMA helps to assess the value of alternative uses of resources against multiple criteria by engaging some of the local stakeholders. Multi-criteria decision analysis is the normative reference framework for PBMA.

A particular advantage of PBMA is that its use encourages those responsible for allocating healthcare resources to discuss openly the opportunity cost of their recommendations. The assessment is also based on locally relevant criteria through MCDA. The application of normative MCDA principles is however under-developed. Practitioners use ad-hoc evaluation procedures that are not consistent with theory and there has been little discussion of the theoretical validity of alternative value functions.

2.6   My contribution: closing the gap

For the purpose of clarifying the contribution of this dissertation, in Table 2, I classify the four prescriptive approaches I reviewed according to their theoretical robustness and pragmatic value. On one hand, PBMA is described as high in pragmatic value but theoretically weaker (at least in its application). On the other hand, CEA, GCEA and CBA are theoretically stronger, but resource intensive and impractical for local healthcare planners.

Table 2 Four pragmatic approaches assessed with respect to theoretical robustness and pragmatic value

Theoretical robustness

		Weaker	Stronger
Pragmatic value	Higher	PBMA	
	Lower		CEA, GCEA, CBA

The aim of this dissertation is to develop an approach that is theoretically stronger, by drawing systematically from the health economics and decision analysis literature, yet usable within the time, skills and resources of local healthcare planners.

3   Adjusting life for quality or disability: stylistic difference or substantial dispute?[1]

Abstract

This paper focuses on the contrast between describing health benefits from the point of view of health gains (QALY-type ideas) and disability reduction (DALY-type ideas). The background is an apparent convergence in practice of the work conducted under both traditions: DALY-based approaches have evolved by focussing on cost-effectiveness of interventions and by considering age weighting a discretionary feature; at the same time, recent developments have seen mainstream economic approaches increasingly used in conjunction with population-based models of disease. In the light of these methodological developments, we contrast a health planner who wants to maximize health and one who wants to minimize disability. Assuming consistent health and disability weights we find that interventions will be ranked in a systematically different way. The difference, however, is not determined by the use of a health or a disability perspective but by the use of life expectancy tables to determine years of life lost. We show that this feature of the DALY method is problematic and

we suggest its dismissal in favour of a fixed reference age rendering the use of a health or a disability perspective merely stylistic.

3.1 Introduction

One of the most obtrusive and least discussed differences between the QALY (Weinstein and Stason, 1977, Williams, 1985, Drummond *et al.*, 2005) and the DALY approach (World Bank, 1993, Tan-Torres Edejer *et al.*, 2003) is the description of health status using years of life *lived* adjusted for 'health' versus years of life *lost* adjusted for 'disability'. It is not clear whether this difference is essentially presentational or whether it reflects some fundamental dispute about what is at stake.

In their early development the difference was presentational. Fanshel and Bush (1970), in particular, proposed an operational definition for health to measure changes in the population health status over time that could capture both mortality and morbidity to inform health planners. They define being 'healthy' as being in a *functional* state or, equivalently, in a *dysfunction-free* one and they proposed the amount of time spent in that state to measure health.

Within the health economics community, the health perspective has been the major focus of attention, with much applied work based on Cost-Utility analysis using the QALY concept and approaches to prioritisation such as cost-per-QALY league tables.

On the contrary, a disability perspective has been favoured in the Global Burden of Disease approach (World Bank 1993) and its successor, Generalised Cost-Effectiveness (GCEA) promulgated by the World Health Organisation (Tan-Torres Edejer, Baltussen et al. 2003). The original Global Burden of Disease (GBoD) project attempted to map out the extent of disability attributable to disease throughout the world using DALY. These studies attracted critical commentary in the health economic literature (Anand and Hanson 1997, Williams 1999, Mooney and Wiseman 2000, Bevan and Hollinghurst 2003) which has in turn drawn robust responses from the framers of the Global Burden of Disease studies (Murray and Acharya 1997, Murray and Lopez 2000).

One of the primary criticisms of the Global Burden of Disease (GBoD) project from an economic point of view was that it encouraged decision makers to focus on diseases rather than interventions, and to decide on priorities without reference to cost. This interpretation has been disputed by Murray and Lopez (1997). However, the most recent incarnation of the WHO approach, GCEA (Tan-Torres Edejer, Baltussen et al. 2003), does address both these points, recommending an approach to prioritisation based on cost-effectiveness league tables, where costs are financial inputs, and benefits are reductions in disability, measured in DALYs. Age-weighting, another controversial feature of the method, has been soft-pedalled in subsequent implementations, and is currently presented as a discretionary, rather than a core, feature.

At the same time, a central motivation for the GBoD programme was a perceived lack of interest within the economic paradigm in the assessment of population need and epidemiological modelling generally (Hollinghurst, Bevan et al. 2000). How far this was ever true is contestable, but it certainly seems to be less true now than ever. For example, a high profile policy document, the Depression Report (Centre for Economic Performance's Mental Health Policy Group 2006), has recently argued for a large and innovative expansion in the provision of mental health services based on a combination of economic modelling and epidemiological evidence (including the GBoD studies); and renewed emphasis on the finiteness of the NHS budget (Maynard, Bloor et al. 2004) has led NICE to develop population-based costing tools to support NHS organisations to quantify the impact of NICE guidelines on their finances (National Institute for Health and Clinical Excellence 2006). Moreover, current policy developments such as the increasing focus on NHS productivity (Dawson, Gravelle et al. 2005, Department of Health 2005) suggest sizing up gains in health at the population level will become more, rather than less necessary in years to come.

There may, then, be greater commonality between what may be summarily referred to as the DALY and QALY approaches than may have been the case in previous years. This is not to say that either approach is uncontroversial. Philosophically, both approaches are open to objections on the grounds that they are not consistent with ethical intuitions such as the rule of rescue (Mooney and Wiseman 2000). Nor are such approaches in general compatible with conventional welfare economics (Garber, Weinstein et al. 1996). This is not so much a weakness of the approaches, but simply a

reminder of the difficulty of achieving a consensus on the principles which should guide policy when life and death are at stake.

Such controversies notwithstanding, it is now clear that the approaches of the WHO have fallen on fertile ground, both in Ministries of Health around the world, and in the global health community (e.g. Melse, Essink-Bot et al. 2000, Fox-Rushby 2002, Hutubessy, Chisholm et al. 2003, Andrews, Issakidis et al. 2004, Chisholm 2005). At the same time, utilisation of the more familiar (in the health economics context) tools of QALY-based cost-effectiveness analysis (Gold, Siegel et al. 1996, Drummond, Sculpher et al. 2005) has also grown apace, particularly in Health Technology Assessment centres, like NICE in the UK. This leaves government planners in an awkward situation, with similar-yet-different approaches being applied to similar-yet-different problems in different countries (and sometimes in the same country).

Gold, Stephenson and Fryback (2002) and Bevan and Hollinghurst (2003), among others, have surveyed the differences between the QALY and DALY approaches, and the reader is referred to their papers for alternative discussions of the differences between these two traditions. As discussed above, however, as of 2007, many of these distinctions do not seem as sharp as they may have done a few years ago. In this paper we discuss whether in the light of these developments the use of a health or a disability perspective to inform healthcare planners is merely of a stylistic nature.

3.2   Health versus Disability

Disability is defined against a normative benchmark, typically a life of a given duration in full health, although conceptually more complex benchmarks could be envisaged (e.g. a lifecourse which involves a particular pattern of progressive degradation in health over time).

This notion of disability seems to have considerable appeal for those trained in public health and epidemiology, disciplines for which the natural unit of analysis is the disease. As propounded by the WHO, the DALY concept originates in an attempt to supplement a commonly used measure of this health deficit attributable to diseases,

Years of Life Lost (YLLs), with a second component, Years of Life with Disability (YLDs), which captures morbidity.

Economics, on the other hand, suggests a different frame, oriented around agents capable of making economic decisions. Considering life years at different levels of health as a good held by such an agent is natural way of thinking within this frame.

We discuss whether viewing the allocation of healthcare resources from a health quality adjustment perspective leads one to different conclusions from those which one would reach if one views the same problem from a disability adjustment perspective. To make this problem concrete, we will contrast a government planner who takes the view that her role is maximise health (a "health utilitarian") with another who considers that minimising disability is a more appropriate objective (a "disability utilitarian").

3.2.1 Formal framework

Let us think of a particular individual as progressing through a series of discrete health states from birth until death. We describe his lifetime health profile with a function $w(t)$ that associates any point in time t from birth, $t=0$, to death, $t=\check{S}$, with a health state $a \in A$, where A is the set of health states (including a state representing death). Interventions which affect his health, for instance performing a surgical procedure or curing an illness, can be thought of as replacing $w(t)$ with an alternative profile $\gamma(t)$.

A planner is responsible for providing healthcare interventions within a limited budget, which she wishes to spend either to maximize health or minimize disability.

The planner who wishes to take a health-utilitarian point of view will have to have some way of rendering health states commensurable so that she can aggregate health over time and over persons. To do this, she might assume that *health measurements exist*, in the sense that there exists a real valued function over health states $h(a)$, $0 \leq h(a) \leq 1$, and her preferences for life profile $w(t)$ can be represented by a function

$$H(f) = \int_0^{\check{S}} h(w(t))dt$$, in the sense that she (non-strictly) prefers profile $w(t)$ to profile

g(t) iff *H (W)≥H (X).*In keeping with convention, we will assume that the health weight for full health is 1 and for death is 0, and states worse than death or better than full health cannot be valued. In order for such measurements to exist, the health-utilitarian's preferences have to exhibit certain sets of conditions which have been well-explored in the theoretic literature (e.g. Pliskin, Shepard et al. 1980, Johannesson, Pliskin et al. 1994, Østerdahl 2005). An example of such a *H*(·) is depicted in Figure 1. This individual experiences three transitions, from full health to some degraded condition *a'* at age t_1, to some further degraded condition *a''* at age t_2, to death at age Š*;* which represent health profile W*(t)*. The existence of health measurement entails that the individual's total (time-integrated) lifetime health, *H(W)*, can be measured by finding the area to the left of the staggered line in Figure 1.

If, on the other hand, the planner takes a disability-utilitarian viewpoint, she might wish to make the corresponding assumption that *disability measurements exist.* The measurement of disability, as opposed to health, poses a particular conceptual challenge, that of determining the baseline age for measurement. Fanshel and Bush suggest that this baseline age is an 'ideal' (1970, p. 1036) and Murray and Lopez that it is a 'target' (2000, p. 73). This sort of usage has some precedent in economic measurement: in particular, it resonates with the use of reference income levels as thresholds for the measurement of poverty (Atkinson 1987).

However, the reference age in the GBoD studies is operationally determined based on life tables: the YLL component of the DALY is computed based on residual life expectancy at time of death. These could be life expectancies relative to the local population, to a specific cohort within the population or to an ideal, standard population (Murray 1996). We will call the reference ages identified by such methods as *death-dependent* reference ages, since the reference age depends on the age at which the individual dies.

The assumption that disability measurements exist, here is taken to mean that there exists a real valued function over health states *d(a)*, $0 \leq d(a) \leq 1$, and a real-valued *reference age T(Š) ,* such that her preferences for life profiles W*(t)* can be represented by a function $D(f) = \int_0^{T(Š)} d(W(t))dt$, in the sense that she (non-strictly) prefers profile W*(t)*

to profile x*(t)* iff *D(w)≤D(x)*.Again, in keeping with convention, we will assume that the disability weight for full health is 0 and for death is 1, and states worse than death or better than full health cannot be valued. An example of such *D(ĩ)* is depicted in Figure 1. The total (time-integrated) lifetime disability, *D(w)*, can be found by finding the total area to the right of the staggered line, with the vertical dimension measured by the scale on the right axis.
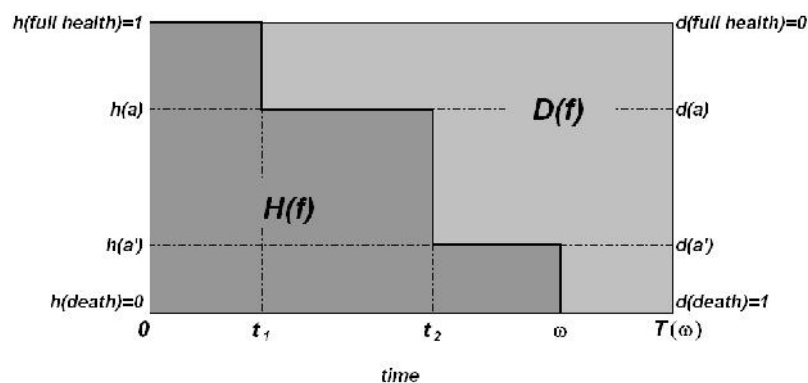
Figure 1 Health *H*(ĩ) and disability measurement *D*(ĩ) on a life profile

The reader will note that in the DALY literature, the underlying model which is typically presented as a basis for disability measurement is a model of a population, rather than a model of the individual: we take the view, however, that any model of a population must implicitly contain a model of the individual as a special case.

3.2.2   Health gain versus reduction in disability

Consider an intervention whereby a life profile w*(t)* is replaced by another x*(t)*. A key question is how the resulting gain in health, Δ*H(w,x)=H(x) - H(w)*, relates to the corresponding change in disability, Δ*D(w,x)=D(x) - D(w)*.

Demographers pointed out that an intervention which saves a life at age Š, does not usually add $T(Š)-Š$ years of life and that a full demographic model of the population with and without the intervention is the best tool to inform health planners (Preston 1993). In response, DALY proponents discussed a population based model comparing the difference between YLLs averted and healthy life years gained by a life saving intervention (Murray 1996) and recommended the use of local period life expectancy

tables for single-year interventions 'as long as the changes caused by the intervention do not change age-specific and overall life expectancies substantially' (Tan-Torres Edejer *et al.*, 2003; p. 55). A reduction in infant mortality by 50%, for instance, would imply a substantial change in life expectancy at birth, $T(0)$, and the use of period local life table would be inadequate. In those cases, a full population model should be built to directly estimate healthy years of life lived under the intervention scenario, abandoning the disability perspective.

We compare UH(w,x) and UD(w,x) on an individual basis in trying to confirm these findings from models at the population level. We will then expand the analysis including changes in quality of life. The intervention used in carrying out the comparison is a single year intervention that is not expected to affect mortality rates and life expectancies at the population level, a context in which the use of measures such as DALY with local period life tables is recommended. For simplicity the reader can imagine an intervention that affects the health of a single individual in a large population. We assume consistency between health and disability weights by setting *d(a)=1-h(a)*. Sassi (2006) used a similar approach but maintained age weighting in the DALY calculation as he focussed on its empirical on estimates of QALY gained and corresponding DALY averted.

Let us first consider a *health improving* intervention in the sense that x is identical to w, except that the time between t_1 and t_2 is spent in a more desirable health state *a'* instead of a less desirable state *a*. This situation is depicted in Figure 2, and it is easy to see that:

$$\Delta H(w,x) = (t_2 - t_1)[h(a') - h(a)] = -(t_2 - t_1)[d(a') - d(a)] = -\Delta D(w,x)$$
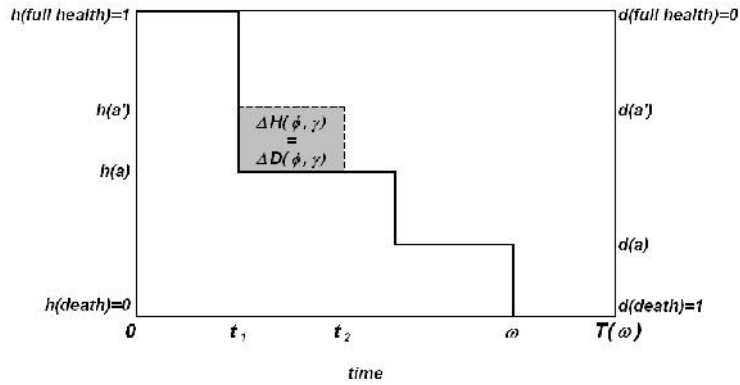
Figure 2 Gain in health ∪*H*(ȋ) and reduction in disability ∪*D*(ȋ) from *health improving* intervention replacing health profile ᴡ (bold line) with health profile x (dashed line between *t₁* and *t₂*, bold line elsewhere).

On the other hand, consider a *life extending* intervention. For simplicity, let us imagine the health profile ᴡ of an individual living from birth to death at age Š in health state *a* and assume that there exists an intervention capable of extending her life by *k* years, until age Š'=Š+k, in health state *a'* as in Figure 3. We estimate ∪*H(ȋ)* and ∪*D(ȋ)* varying parameters Š, *k* and *h(a')* assuming consistent quality weights and, for illustrative purposes, death-dependent reference age *T(Š)* based on recent, English life tables (GAD 2006).
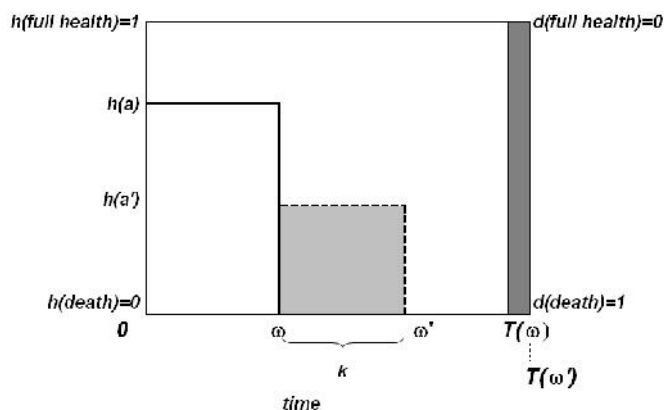
Figure 3 Gain in health and reduction in disability from *life extending* intervention replacing health profile ᴡ (bold line) with health profile x (dashed line between Š and Š', bold line elsewhere).

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We present our results in the form of a ratio ⌐ of the reduction in disability relative to the gain in health, denoting *h(a')* by *h** for notational simplicity. Clearly, the more comparable are the QALY and DALY measures, the closer this ratio is to 1.

$$\Gamma(\check{S}, k, h^*) = -\frac{\Delta D(\text{W,X})}{\Delta H(\text{W,X})} = \frac{D(\text{W}) - D(\text{X})}{H(\text{X}) - H(\text{W})} =$$

$$= \frac{T(\check{S}) - T(\check{S} + k) + k \cdot h^*}{kh^*} = 1 - \frac{T(\check{S} + k) - T(\check{S})}{kh^*}$$

The denominator is the health gain, which is simply *k* years adjusted for their quality, *k⍳h**; the numerator is the reduction in disability and is affected by the change in reference age from *T*(Š) to *T*(Š') because a death at age Š carries *T(*Š*)*-Š YLLs, whereas a death at age Š*', T(*Š*+k)* – Š *-k* YLLs. The total reduction in DALY is therefore U*D(*W,X*)*=[*T(*Š*')*-Š*'+(1-h**)⍳*k+C*]- [*T(*Š*)*-Š*+C*]=*T(*Š*+k)- kh*- T(*Š*),* where *C* denotes YLDs not affected by the intervention (e.g. years lived with disability before age Š in Figure 3).

We begin by holding *h** at 1, that is by comparing years of life gained to the corresponding reduction in years of life lost, without adjusting for quality. In this case, U*H(*W,X*)* reduces to *k,* and the difference in years of life lost to *T(*Š*+k)- T(*Š*)-k*. As we discuss in the Appendix, typically 0 ≤ *T(*Š*+k)-T(*Š*)* ½ *k* which implies that - Δ*D(*W,X*)*½*k=*Δ*H(*W,X*)* and that the α ratio of DALY averted over QALY gained is between 0 and 1. We show in Figure 4 the behaviour of α varying Š and *k.* As expected 0 ≤ α ≤ 1 and *α* decreases both with Š and *k* for most values of Š. For the very young, however, *α* is briefly increasingly, reflecting that fact that life expectancy itself can increase with age, after one has survived the dangerous days and months immediately following birth. The DALY calculations can be then very different from QALY calculations, particularly for the very old: for such people, an increase in life from Š to Š' but is also likely to have a significant effect on residual life expectancy as measured by *T(*Š*')- T(*Š*)*. The DALY averted from an intervention which extends their life will be negligible because the benefits of a longer life are for the most part offset by a higher normative benchmark *T(*Š*')*.
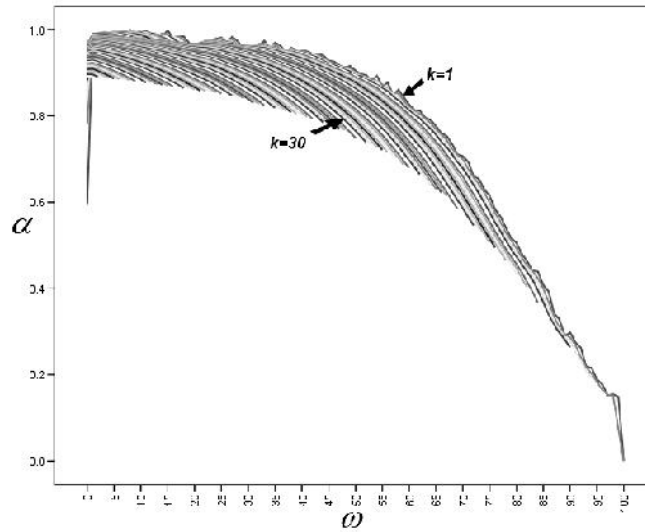
Figure 4 Ratio of reduction in disability to gain in health (Γ) – empirical estimates using recent, English life tables. The *x*-axis reports the age at which death is prevented (Š). Γ values are reported for intervention extending life by *k* years, where *k* is varied from 1 to 99. The graphs for *k*=1 and *k*=30 are indicated to guide the reader.

The results are consistent with previous findings with models at the population level, but offer new insights on their nature. In particular, Murray find a similar relationship between gains in *healthy* life years and reduction in YLLs from an intervention which saves a life at any age, using different life expectancy tables to define YLLs (Murray, 1996) and interprets them as 'meaningful implied equity weights' which assign less value to the benefits of older individual compared to younger ones (Murray and Lopez, 2000; p. 78). Our analysis, by comparing QALY gained and DALY averted at the individual level, shows that the shift in reference age from $T(Š)$ to $T(Š')$, which is particularly marked at older ages, determines the lower value of YLLs gained in elderly beneficiaries of health interventions. This is also easy to see from Figure 4, in which the curve associated with a health gain of *k*=1 years of life represents the difference between $T(Š)$ and $T(Š+1)$.

If we now allow *h** to assume values smaller than 1, we can explore the relationship between years of life lived adjusted for health and years of life lost adjusted for disability. We estimated the ratio *α* of -U*D*(·)/U*H*(·) using once again recent English life tables but varying *h** over the interval [0,1].

We find that a health extending intervention might produce *more* rather than less DALY. This happens when the years of life added adjusted for their quality, *kh**, are more than compensated by the shift in reference age from *T(Š)* to *T(Š+k)*. Figure 5 reports results for *k*=1 and *k*=30. Note that these graphs are different from the previous ones and each curve now corresponds to different values of *h**.
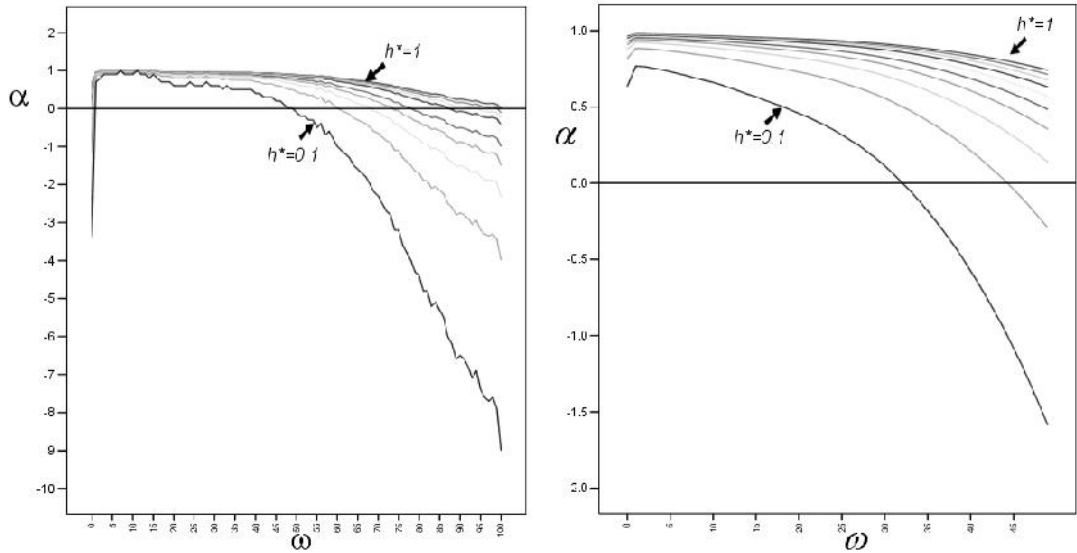
Figure 5 Ratio of reduction in disability to gain in health (Γ) – empirical estimates using recent, English life tables. The *x*-axis reports the age at which death is prevented (Š). Γ values are reported for intervention extending life by1 year in the left-hand side graph and by 30 years in the right-hand side graph with a quality of life *h**. The curves for *h**=1 and *h*=0.1 are indicated to guide the reader.

3.3 Discussion

A health-utilitarian and a disability utilitarian health planner would rank interventions in a systematically different way, even if both made the same assumptions about costs and effectiveness, and assumed consistent health and disability weights, so long as the form of the DALY used embodies a death-dependent concept of the reference age.

Consider for example a decision maker who can fund treatment for one and only one of the following people: *(i)* a 65 year old man who, untreated, will die today; *(ii)* a 45

year old man who, untreated, will die today. The person receiving the treatment will live for one other year, with quality of life valued at 0.1 on a 0 to 1 scale where, as usual, the quality of life associated with being dead is 0 and that associated with perfect health is 1. Let us assume that the decision maker wants to maximise the health benefit, measured as gains in QALYs or reduction in DALYs.

If the decision maker measures the health benefit with a QALY metric, funding any of the two interventions would lead to a gain of 0.1 QALY. This endorses an egalitarian judgment that QALY are equal, no matter who receives them and the decision maker might set up a lottery to determine who will receive the intervention or invoke further decision criteria, e.g. to favour younger over older patients on a *fair-innings* argument (Williams 1997).

On the other hand, if the decision maker measures the health benefit using a DALY metric, funding the first intervention would lead to an *increase* in the burden of disease of 0.16 DALYs, but funding the second intervention would lead to a slight reduction in the burden of disease of 0.02 DALYs, and would then offer the treatment to the 45 year old man. In fact, she would not provide the intervention to the 65 year old man even if resources were available to fund it, because his death today is associated with a lower burden of disease than his death in a year's time, which is at variance with the original assumption that a quality of life of 0.1 is better than death.

We find the DALY valuation of a health benefit quite problematic when computed in this way and that it is difficult to see how an intervention which increases an old person's life (even at a level of health only marginally better than being dead) can represent an *increase* in the burden of disease.

This difference between QALY- and DALY-based rankings of interventions, however, is not determined by the use of a disability perspective per se. In particular, if the government planner's preferences meet the condition for health measurements to exist, and there is an upper bound on all possible lifetimes, then disability measurements also exist, as $D(\cdot)$ can be found by using a consistent set of health and disability weight, $d(a) = 1 - h(a)$, and setting the reference age to a constant value of $T \geq Š$. Under these conditions, $H(w) + D(x)$, is equal to T. However, since $H(w) + D(w) = H(x) + D(x) = T$, by simple

algebra, any increase in health must be matched with an identical decrease in disability, i.e. ΔH(w,x)= - ΔD(w,x).

To extend this result to measures of DALY averted at the population level, the selected constant reference age *T* should be identical for all individuals. For the equivalence of the two approaches to hold, operationally, the reference age simply needs to be higher than any admissible age for a human life.

We think that the language of disability measurement is a useful one, particularly when introducing health measurement concepts to professionals trained in public health or epidemiology, to whom years of life lost represent a natural intellectual starting point.

We are not suggesting the use of a constant reference age for the DALY approach in general. If one is interested in describing the health status of a population in terms of the current, total burden of disease, the use of life expectancy from a standard, ideal or local population should be used as recommended in the DALY framework (Murray 1996, Murray and Acharya 1997). If one is interested in the benefits from an intervention, however, one should use a health perspective with a QALY-type measure or a disability perspective with a constant reference age. This is however simply an algebraic fix to avoid erroneous estimates and misleading recommendations of using DALY-type measures to assess benefits from health interventions.

3.4   Appendix

In this appendix we discuss when $0 \leq T(\check{S}+k)-T(\check{S}) \leq k$. We do this in two steps. First, we define and discuss the shape of the residual life expectancy function *L(x),* identifying the conditions under which its first derivative lies between -1 and 0. Then, we show that $T(\check{S}+k)-T(\check{S})$ is always positive and discuss when $T(\check{S}+k)-T(\check{S}) \leq k$.

Let us define the following three functions (Keyfitz 1968, Lindsey 2004):

(1) the *survivor function*, *S(x)*, that is the probability of living until age *x:*

$$(1) \quad S(x) = \Pr[T > x] = 1 - F(x) = \int_{x}^{\infty} f(t)dt \; ;$$

where $F(x)$ is the cumulative distribution and $f(x)$ is the corresponding density function;

(2) the *mortality rate*, $\}(x)$, that is the instantaneous probability that death will occur at age x:

$$(2) \quad \}(x) = \frac{f(x)}{S(x)};$$

(3) the *residual life expectancy, L(x),* that is the average prospective lifetime of an individual aged x:

$$(3) \quad L(x) = \frac{\int\limits_{x}^{\infty} S(t)dt}{S(x)};$$

Differentiating *L(x)* with respect to *x*:

$$(4) \quad \frac{dL}{dx} = \frac{-S^2 - (-f(x))\int\limits_{x}^{\infty} S(t)dt}{S^2} = -1 + \}(x)\cdot L(x);$$

It can be easily seen that $dL/dx \geq -1$ always, because both $\}(x)$ and $L(x)$ are non negative; and $dL/dx \leq 0$ if and only if $\}(x)\cdot L(x) \leq 1$, that is residual life expectancy is a decreasing function in correspondence of ages *x* where $L(x) \leq \dfrac{1}{\}(x)}$.

Empirical analysis of life tables shows that *L(x)* may increase during early years of life, when there is a high risk of infant mortality. In developed countries, where the life expectancy at birth is above 70 years, this usually happens only for the first year of life or even just for the first few months, and *L(x)* is a decreasing function of age *x* thereafter (Coale and Demeny 1983, Goldman and Lord 1986, Shrestha 2005).

Let us now discuss when $0 \leq T(\check{S}+k)-T(\check{S}) \leq k$. First note that $T(\check{S})=\check{S}+L(\check{S})$.

We can re-write $T(\check{S}+k)-T(\check{S})=k+L(\check{S}+k)-L(\check{S})$ as

$$T(\check{S}+k)\text{-}T(\check{S}) = k \cdot \left[1 + \frac{L(\check{S}+k)-L(\check{S})}{k} \right] =$$

$$=$$

$$k \cdot \left[1 + \frac{L(\check{S}+k) - L(\check{S}+k-1)}{k} + \frac{L(\check{S}+k-1) - L(\check{S}+k-2)}{k} + ... + \frac{L(\check{S}+1) - L(\check{S})}{k} \right]$$

.

The years gained with the intervention, k, are non-negative. As we discussed above, the first derivative of $L(x)$ is greater than -1 for any x, hence the term in square brackets is non negative, that is $T(\check{S}+k) - T(\check{S}) \geq 0$ always. Similarly, for values of x where the first derivative $dL/dx \leq 0$, that is when $\}(\check{S}) \cdot L(\check{S}) \leq 1$, the term in square brackets is less than one, hence $T(\check{S}+k) - T(\check{S}) \leq k$.

4 Requisite models for strategic commissioning: the example of type 1 diabetes

Abstract

A developing emphasis of health care reforms has been creating organisations with responsibilities for strategic commissioning of services for defined populations. Such organisations must set priorities in aiming to meet their populations' needs subject to a budget constraint. This requires estimates of the health benefits and costs of different interventions for their populations. This paper outlines a framework that does this and shows how this requires modelling to produce estimates in a way that is transparent to commissioners, of requisite complexity to produce sound estimates for priority setting using routinely available data. The example illustrated in this paper is an intervention that would improve glucose control in the English population with type 1 diabetes. It takes many years for a change in glucose management to deliver maximum benefits; hence the intervention is not good value-for-money in the short run. We aim to give a more strategic view of the costs and benefits modelling costs and benefits in a steady-state model which suggests that the intervention is good value-for-money in the long run.

4.1 Introduction

Cost-effectiveness analysis (CEA) and disease modelling have grown apace in the hope of informing policy formation, however many authors have questioned their actual contribution to the development and implementation of policies (Ross 1995, Drummond, Cooke et al. 1997, Duthie, Trueman et al. 1999, Drummond and Weatherly 2000, Bryan, Williams et al. 2007). This paper develops a framework for CEA and cost-

effectiveness analysis to provide information for organisations responsible for strategic commissioning of health services for defined populations and illustrates its use by modelling intensive glucose control in type 1 diabetes in England. Strategic commissioners (or purchasers) have emerged in reforms of health care, which are required to assess needs of populations, determine the optimal way of meeting these needs, and accordingly contract with providers of different services. This is currently the task of Primary Care Trusts (PCTs) in the National Health Service (NHS) in England (Department of Health 2006) and Local Health Integration Networks (LHINs) in Ontario (Ontario 2006).   The second section of this paper outlines the framework we have developed to help strategic commissioners set priorities. The third section illustrates how this framework was used in modelling type 1 diabetes. The final section discusses the results and implications of our framework for disease modelling.

4.2   Framework of analysis

The mainstream evaluation framework in economic evaluation for priority setting is that of Quality-Adjusted Life Years ((Weinstein and Stason 1977, Williams 1985); see (Gold, Siegel et al. 1996, Drummond, Sculpher et al. 2005) for a review of proposed, albeit less widespread alternatives). A Quality-Adjusted Life Year (QALY) is a year weighted for quality of life, with a weight of one for perfect health and zero for death. QALYs are used to compare alternative interventions and to prioritize cost-effective interventions for funding. The cost-effectiveness of an intervention is measured by the ratio between its added value in terms of health benefits and its incremental cost compared to an alternative, the "incremental cost-effectiveness ratio" or simply "cost/QALY". Interventions with a lower cost/QALY represent better value for money because a smaller investment is needed to produce a unit of benefit or, alternatively, more QALYs can be achieved per unit spent. A different measurement tool that raised a heated debate is the concept of Disability-Adjusted Life Years (DALYs) to estimate the Burden of Disease (BoD) in a population (Anand and Hanson 1997, Murray and Acharya 1997, Williams 1999, Mooney and Wiseman 2000, Murray and Lopez 2000, Bevan and Hollinghurst 2003). DALYs are a form of summary measures of population health and combine information on mortality and morbidity (for a review of alternative measures see Lopez (2002)) and consist of the sum of Years of Life Lost (YLLs) from premature mortality and Years Lived with a Disability (YLDs), in which each year of life is weighted

for disability with a weight of zero for perfect health and one for death.   These different approaches have subsequently been developed to converge to produce information on costs and benefits of interventions in the population in terms of reductions in BoD measured in DALYs (Hutubessy, Chisholm et al. 2003, Andrews, Issakidis et al. 2004, Evans, Edejer et al. 2005), or gains in health, measured in QALYs (Dawson, Gravelle et al. 2005, Department of Health 2005, Martin and Smith 2006, UK Centre for the Measurement of Government Activity 2006).

Beside common serious methodological, ethical and empirical problems (Gold, Siegel et al. 1996, Lopez, Mathers et al. 2002), each approach, as originally developed, was subject to different limitations as bases for setting priorities. The methodology of Cost/QALY was designed for marginal analysis: it does not distinguish interventions of low cost and low benefit from those of high cost and high benefit; does not tell us whether the bulk of resources are being currently used effectively (Hutubessy, Chrisholm et al. 2003, Evans, Adam et al. 2005); nor the number of people affected by an intervention. The value of reporting on the scale of the intervention has been highlighted by Murray and Lopez (Murray and Lopez 2000): "If there are fixed assets, other than disposable dollars, limiting the feasible combinations of interventions that can be delivered – real-world examples include the attention of senior Ministry of Health decision-makers or the political commitment of government leaders –, then these should be devoted not just to the most cost-effective interventions but to those cost-effective interventions with the potential to effect substantial improvements in population health status'. The standard approach to estimating BoD in DALYs, however, gives estimates of that which exists given the current delivery of health care, and hence is best described as the 'current' BoD.   Estimates of the current BoD in DALYs are of no value in themselves, nor a good guide on the potential benefit from an intervention. Hollinghurst *et al.* (2000) estimate the current BoD and the potential benefits from interventions in the South West of England. Estimates varied greatly across different diseases and showed that, although the current BoD of heart diseases was higher than that of depression, the DALYs that are potentially avoidable by improving treatment of depression were much more than those of improving treatment of heart diseases. To set priorities using DALYs, we require information on benefits and costs, but to interpret the relationship between DALYs and costs, we need to distinguish between estimates of three different components of BoD (Bevan, Hollinghurst et al. 1998, Hollinghurst, Bevan

et al. 2000, Hollinghurst and Bevan 2003): (i) DALYs 'avoided' from the current delivery of health care which with their costs indicate cost-effectiveness of current practice; (ii) DALYs 'avoidable' through improving treatment (coverage, appropriateness or compliance) which need to be put alongside estimates of their costs to indicate potential cost-effectiveness of changing practices; and (iii) DALYs that are 'unavoidable' and cannot be reduced given current evidence, and are hence irrelevant to assessments of setting priorities among available interventions.

To set priorities for populations we require methods that draw on both cost/QALY and DALYs by applying the framework of cost-effectiveness to populations in order to estimate the 'avoidable' burden of disease (Hollinghurst and Bevan 2003). The concept of 'avoidable' burden of disease builds on the idea of using 'avoidable mortality' to assess the use of resources among different health care services (Rutstein, Berengerg et al. 1976, Charlton, Hartley et al. 1983, Holland 1991) and combines it with DALYs to estimate both mortality and morbidity avoidable through an intervention. This has been the common basis for three different recent sets of studies: cost-effectiveness of treating mental illness in Australia (Andrews, Issakidis et al. 2004); WHO's project for *Choosing Interventions that are Cost Effective* (Hutubessy, Chrisholm et al. 2003, Tan-Torres Edejer, Baltussen et al. 2003, Evans, Edejer et al. 2005); and estimates of NHS productivity that sought to estimate gains in QALYs for the population of England (Dawson, Gravelle et al. 2005, Department of Health 2005, Martin and Smith 2006, UK Centre for the Measurement of Government Activity 2006).

To deal with costs and health benefits occurring at different points in time, manuals of cost-effectiveness recommend the use of a common discount rate, but acknowledge that theory and empirical evidence on the relationship between interest rates and rates of time preference is unsettled. For strategic commissioners, the cost-effectiveness of a health intervention based on its derived present value is difficult to interpret and use: they are allocated annual budgets and cannot easily translate results from economic evaluations to the financial impacts in the short and in the long term. This is nicely illustrated by intensive glucose control for type 1 diabetes. This is because, although some evidence suggests that over the patient's lifetime this is more cost-effective than conventional care (Diabetes Control and Complication Trial 1996, Herman, Dasbach et al. 1997, Tomar, Lee et al. 1998, Palmer, Weiss et al. 2000), its funding will cause an

immediate increase in costs and delayed benefits. This paper proposes a different approach by measuring impacts on population health and on the commissioner's budget in the short- and long-run.

4.3 Modelling type 1 diabetes

4.3.1 The Disease and Interventions

Diabetes mellitus is one of the most common chronic diseases and the diabetic population in England is estimated to be about 2.2 million (Forouhi, Merrick et al. 2006). Of these, 2 million have type 2 diabetes, which is characterised by insulin resistance and usually diagnosed in the middle aged or elderly; and about 170,000 have type 1 diabetes, which is characterised by an absolute deficiency of insulin and is usually of rapid onset.

The evidence is that only a minority of people with type 1 diabetes have blood-glucose concentrations below the recommended levels (Figure 1) (National Clinical Audit Support Programme 2005); there is a well-known association between poor glucose control and the development of microvascular complications, i.e. eye, kidney and nerve damages that could lead to blindness, dialysis and amputation (Diabetes Control and Complication Trial 1990, Diabetes Control and Complication Trial 1993, Diabetes Control and Complication Trial 1996) hence, these people are expected to develop complications. A large longitudinal study has shown, however, that it is possible to reduce the levels of glucose concentration by providing intensive and personalized advice on insulin doses, diet and exercise and that, over time, this leads to a significant reduction in microvascular complications (Diabetes Control and Complication Trial 1990, Diabetes Control and Complication Trial 1993, Diabetes Control and Complication Trial 1996). There is also some evidence that the intervention is cost-effective according to standard economic evaluation both in type 1 (Diabetes Control and Complication Trial 1996, Herman, Dasbach et al. 1997, Tomar, Lee et al. 1998, Palmer, Weiss et al. 2000) and in type 2 diabetes (e.g. Eastman, Javitt et al. 1997, Gray, Raikou et al. 2000). However, microvascular complications are progressive, appear after several years after the onset of diabetes and tend to degenerate over time. The typically degenerative nature of these complications poses a particular challenge in designing policies for these patients: those who already have moderate complications will have limited benefits

from intensive glucose therapy, as the damage is already present and cannot usually be reversed; the full benefits are for those who receive intensive glucose control from the early stages of their diabetes only, but there are long time lags between the start of the therapy and its benefits in terms of reduced complications.

Diabetes Type 1 population with glucose under control
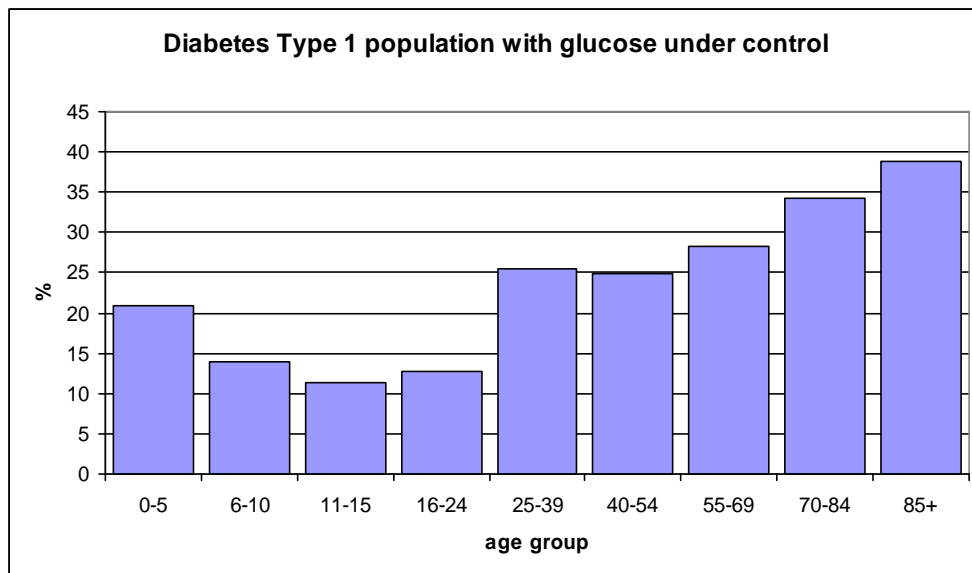
Figure 6 Proportion of type 1 diabetes population with glucose levels within the recommended level, by age group (National Clinical Audit Support Programme 2005; data breakdown provided upon request by NHS – Health and Social Care Information Centre)

4.3.2 Modelling requirements of our framework

Our framework required estimates of the BoD from type 1 diabetes that is 'avoidable' through intensive glucose control by modelling the relationships between better glycaemic control and: reduced risks of developing renal, eye or neural complications; and slower progression from mild to severe stages after the onset of the complication; and lower mortality rates. We required estimates of the current BoD and that which is 'avoidable' from in terms of:

- Deaths;

- Years of Life Lost (YLLs) – the residual life expectancy at the age of the 'avoidable' death according to local life tables; and

- Years Lived with a Disability (YLDs) using disability weights developed by the Dutch Disability Weight study (Stouthard, Essink-Bot et al. 1997);

- DALYs (the sum of YLLs and YLDs), with and without discounting, using a 3.5% discount rate (National Institute for Health and Clinical Excellence 2007).

We also required estimates of average annual net costs of:

- expenditure each year, for the whole of the diabetic population, drugs, equipment; monthly specialist visits and measurement of HbA_{1c}, less

- savings due to intensive glucose control from reductions in the costs of treating the sequelae of diabetes, renal disease (including dialysis), eye disease, and diabetic foot (including amputation).

We also required estimates of the short- and long-run impacts of intensive glucose control:

- over the next five years, assuming a policy in which intensive glucose control was introduced for all patients regardless of the stage of their disease, in which we modelled changes in the current population from aging and death, but omitted births (this is known as a 'closed population model'); and

- in the long run, in a future 'steady state', in which all patients would have intensive glucose control at the onset of the disease, in which we modelled a population cohort of new cases of different ages and simulated changes over time by assuming that the total size and age distribution of the population was stable.

Although five years was an arbitrary choice, it reflects a period between the immediate and long run and corresponds to the time horizon recommended for strategic planning in the English NHS (supplemented by yearly reviews) and is similar to the Ontarian 4-year typical time horizon with yearly reviews. The steady state scenario gives indications of the expected annual health benefits and costs for a stable

intervention and has been used in the past to evaluate services with long time lags as diabetes (Wood, Mallick et al. 1987, Bagust, Hopkinson et al. 2002).

To compare the health benefits with the net cost of the intervention, we attached a monetary value to life. We assumed a theoretical equivalence between a year of life in full health and a year of life free of disability (Fanshel and Bush 1970) and used the putative threshold of the National Institute for health and Clinical Excellence, which on average judges cost-effective a health intervention that costs less than £30k per QALY. We ran a sensitivity analysis on the value of health benefits.

In this paper we investigate the adequacy of a simple disease model within our framework of analysis. To be useful for informing strategic commissioning, we required a transparent, simple model, using routinely-available data, that would produce approximate estimates that would indicate orders of magnitude for comparison with other interventions within and across different diseases at the population level. Most of the diabetes models that have been developed understandably focus on type 2 diabetes (based on the pioneering work by Eastman and colleagues (Eastman, Javitt et al. 1997, Eastman, Javitt et al. 1997)), but some like the Archimedes, the CORE or the EAGLE model are designed for both type 1 and 2 (Eddy and Schlessinger 2003, Palmer, Roze et al. 2004, Mueller, Maxion-Bergemann et al. 2006). We tested the adequacy of our model through validation, sensitivity analysis and comparing results with those from more sophisticated models. The model we developed is requisite for our purpose and parsimonious (Phillips 1984, Pidd 2003).

We modelled diabetes as a Markov chain, which makes the simplifying assumption that the probability of transition from disease state A to disease state B does not depend on the patient's history before arriving in state A. However, the incidence of microvascular complications correlates significantly with diabetes duration (Morgan, Currie et al. 2000); we divided the population in 5-year age groups to allow the use of a different set of transition probabilities for each one. The probability of death is dependent both on age and degree of severity of complication. The incidence of complications and their progression rates vary with age, but as there are no routinely available data on these, we assumed no incidence of microvascular complications before the age of 15 and lower incidence rates in young adults compared to older ones. The

specifications for the two models are outlined in Figure 7. A description of the key assumptions and an evaluation of the data are given in Table 3 and Table 4. We estimated the BoD: from higher mortality (deaths and YLLs) from all causes; and disability (YLDs) associated with microvascular complications, diabetic nephropathy, retinopathy and diabetic foot; but not from acute diabetic events (ketoacidosis), non-fatal myocardial infarctions, non-fatal strokes and coronary revascularisations. Although we did not model patients with cerebrovascular complications explicitly, deaths caused by these complications are accounted in the YLLs from all causes.

The model can be run for any local population and we have used it for England, ten different PCTs in the South-East of England and two PCTs in central London. However, the demographic differences across these PCTs did not have a significant impact on the relative magnitude of results. In this paper we discuss estimates for the population of England.
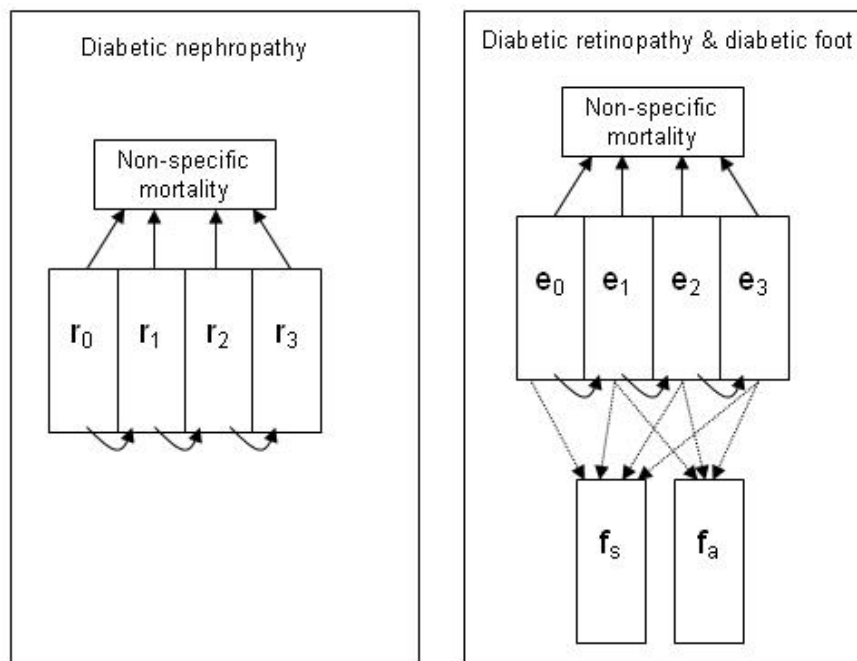
Diabetic nephropathy model	
r_0	Normo-albuminuria
r_1	Microalbuminuria (urinary albumin excretion $\geq$40 mg/24 hr)
r_2	Macroalbuminuria or overt-proteinuria (urinary albumin excretion $\geq$300 mg/24 hr)
r_3	End-Stage-Renal-Disease (ESRD)
Progression	Diabetic patients move through disease states according to annual transition probabilities. See table A3 in Appendix 2.
Mortality	All-cause mortality.
Diabetic retinopathy	
e_0	No retinopathy
e_1	Background diabetic retinopathy (BDR)
e_2	Proliferative diabetic retinopathy (PDR)
e_3	Severe visual loss
Progression	Diabetic patients move through disease states according to annual transition probabilities. See table A4 in Appendix 2.
Mortality	All-cause mortality.
Diabetic foot	
f_s	Sores /Ulcers
f_a	Amputation

DALYs	**YLDs +**	Years lived in each state *s* weighted for the disability associated with the state.
	YLLs	Years of Life Lost to premature (excess) mortality attributable to diabetes**

Figure 7 Base structure of the model for diabetic nephropathy (left) and diabetic retinopathy & diabetic foot (right). **Deaths in the diabetic population are caused by 'normal' mortality, i.e. mortality rate as in the non-diabetic population, and 'excess' mortality due to diabetes. Only 'excess' mortality generates Years of Life Lost (YLLs) for the Burden of diabetes estimate.

Table 3 Key modelling assumptions

Assumption	Justification
The transition probabilities from state *i* depend only on being in state *i* and not on the history before arriving in state *i*.	This is the standard simplifying assumption in modelling stochastic processes and is the basis of Markov chain models that are widely used in modelling progression of disease and is common practice for modelling diabetes. To relax this assumption we divide the population in 5-year age groups and use a different set of transition probabilities for each one if data was available.
Same rates apply to men and women.	With the exception of myocardial infarction complication rates are similar in men and women (National Clinical Audit Support Programme 2005).
Under the intervention scenario, all the diabetic population is subject to intensive treatment.	This reflects NICE recommendations to maintain HbA$_{1c}$≤7.5% in all diabetic patients, but will overstate the benefit of the intervention. We used sensitivity analysis on compliance rates to test this assumption.

4.3.2.1 First five years

The model of the first five years tracked 100 birth cohorts, i.e. the population from ages 0 to 99 over five consecutive years. The distribution by age of the initial population was that in England in 2003. Estimates of BoD in DALYs were calculated by Equation 4:

Equation 4

$$DALYs = YLLs + YLDs =$$

$$= \sum_{i=1}^{5} e^{-ri} \sum_{j=0}^{99} \sum_{s=0}^{k} A(i,j,s) * \sim'(i+j,s) \int_{1}^{L(i+j+1)} e^{-rt} dt +$$

$$+ \sum_{i=1}^{5} e^{-ri} \sum_{j=0}^{99} \sum_{s=0}^{k} A(i,j,s) * w(s) * e^{-r}$$

where:

- *i* is the index for the years over which the model is run;

- *j* is the index for the cohorts (*j* is the initial cohort age);

- *s* is the index for the degree of severity of the condition;

- *r* is a discount rate. The model was run with *r*=0 (which corresponds to no discounting) and with *r*=3.5% (giving discounted values);

- $A(i, j, s)$ is the number of the population with diabetes at stage *s* in year *i* of cohort *j;*

- ~'(*i+j, s*) is the *excess* mortality rate from type 1 diabetes with degree of severity *s* for the *j*th cohort in year *i* (by which time the members of this cohort will be [*i+j*] years old);

- *L* (*i+j*) is the residual life expectancy of the *j*th cohort in year *i.* As discussed in Chapter 3 (Airoldi and Morton 2009) we use a constant reference age of 100 years to assess residual life expectancy;

- *w(s)* is the disability weight associated with degree of severity *s*.

At the core of the model, was the system of difference equations that model the evolution of two populations, *A* and *N*. *A(i, j, s)* was the population with type 1 diabetes in degree of severity *s, N(i, j)* was the population without type 1 diabetes (both constituted the *j*th cohort in the *i*th year of modelling).

The population with type 1 diabetes in the *j*th cohort in the (*i+1)*th year of modelling [*A(i+1, j, s)*] was derived from populations with type 1 diabetes [*A(i, j, s)* and *A(i, j, s-1)*] and without type 1 diabetes [*N(i, j)*], in the *j*th cohort in the *i*th year of modelling, and estimated by Equation 5:

Equation 5

$$A(i+1, j, s) = A(i, j, s)\big[1 - x_s(i+j, s+1) - \sim(i+j, s)\big] +$$

$$+ A(i, j, s-1)x_s(i+j, s-1) + N(i, j)\big[\Gamma(i+j, s)\big]$$

for all *j* (0 to 99) and for all *i* (1 to 5) where:

- $x_s(i+j, s+1)$ is the transition probability from stage *s* to *s+1*;

- $\sim(i+j, s)$ is the death rate from type 1 diabetes in stage *s* for the *j*th cohort in year *i* (and is equal to age-specific mortality rate for the population without the condition, $\}(i+j)$, plus the excess mortality rate from type 1 diabetes with degree of severity *s* in year *i* of the cohort *j*th, ~'(*i+j, s*));

- $x_s(i+j, s-1)$ is the transition probability from stage *s-1* to stage *s*;

- $r(i+j, s)$ is the incidence rate of new cases of type 1 diabetes at stage *s* from population *N*, where $\sum_s r(i+j, s) = r(i+j)$.

The population without type 1 diabetes in the *j*th cohort in the (*i+1*)th year of modelling [*N(i+1, j)*], was derived from the population without type 1 diabetes in the *j*th cohort in the *i*th year of modelling [*N(i, j)*], were and estimated by Equation 6:

Equation 6

$$N(i+1, j) = N(i, j)\left[1 - r(i+j) - \}(i+j)\right]$$

for all *j* (0 to 99) and for all *i* (1 to 5) where:

- $r(i+j)$ is incidence rate of new cases with type 1 diabetes for the jth cohort in year i;

- $\}(i+j)$ is death rate for of the population without type 1 diabetes in year *i*.

The model required estimates of the initial populations without and with type 1 diabetes: *N(0,j)* and *A(0,j)*. These were derived using data on the 2003 population in England (Department of Health 2004) and prevalence estimates published by Harvey *et al.* (Harvey, Craney et al. 2002). We did not find data on the distribution of the population with type 1 diabetes (*A(0,j)*) in terms of degrees of severity by age of renal and eye complication. We estimated these distributions by generating a hypothetical birth cohort of 100,000 persons and simulating their aging, deaths and progression to and through diabetes over 100 years. The dynamic of the hypothetical cohort was

modelled with a Markov-chain model that used the same transition probabilities of the main model presented in this paper. We assumed that the proportion of diabetic patients with degree of severity s at period t of the hypothetical cohort simulation was representative of the proportion of diabetic patients aged t in the current English diabetic population. We subject the resulting initial condition to validation.

Figure 2 outlines the progression of type 1 diabetes in the stages of nephropathy (left panel) and retinopathy (right panel). The stages of nephropathy are:

- microalbuminuria, i.e. an increased concentration of the protein 'albumina' in the urine;

- macroalbuminuria, i.e. overt proteinuria or 'clinical nephropathy', and

- end stage renal disease (ESRD).

Each of these stages is also associated with increased mortality rates, mainly due to cardiovascular disease (Laing, Swerdlow et al. 1999, Laing, Swerdlow et al. 1999, Soedamah-Muthu, Fuller et al. 2006, Soedamah-Muthu, Fuller et al. 2006); and these are particularly high for macroalbuminuria (Borch-Johnsen, Andersen et al. 1985, Rossing, Hougaard et al. 1996). The progression of retinopathy to blindness is also associated with a higher mortality rate compared to the non-diabetic population. The effect of glycaemic control was modelled through transition probabilities x, which are lower for diabetic patients under intensive glucose control compared to conventional care, which means there is a slower progression of the disease to and through microvascular complications (see Appendix).

The retinopathy model also estimated the BoD from ulcers, sores and amputation using the incidence rates of these complications associated with different degrees of retinopathy (Moss, Klein et al. 1992) (see Appendix). The Diabetes Control and Complications Trial (DCCT study) does not report the reduction in lower extremity amputation rates. We built on the association between degrees of severity in retinopathy and lower extremity amputation (Moss, Klein et al. 1992). We made two assumptions: first, poor glucose control is an underlying cause of both diabetic retinopathy and diabetic foot; second, the association between degree of severity of

retinopathy and diabetic foot is the same in the intensive glucose control and in the conventional treatment group (keeping constant the provision of other treatments, e.g. laser treatment). For instance, the 4-year incidence of lower extremity amputation is 7.8% in patients with proliferative diabetic retinopathy (PDR). However, fewer people have PDR with intensive glucose monitoring and control than with conventional therapy. The model we built did not model neuropathy and diabetic foot explicitly and would be unsuitable to measure the impact of other specific interventions (e.g. changes in laser therapy).

There are interdependencies among all complications that cannot be represented in a simple spreadsheet model like ours (to represent them, the CORE model builds on fourteen sub-models and the Archimedes model generates the biology of a virtual patient directly rather than modelling distinct health states). We combined the nephropathy and retinopathy/diabetic foot models to estimate YLLs and YLDs from type 1 diabetes as follows:

- YLLs based on deaths from the nephropathy model, because albuminuria is the best predictor of all-cause mortality in type 1 diabetes (Rossing, Hougaard et al. 1996). These deaths includes those from macrovascular complications such as myocardial infarctions and strokes;

- YLDs from the nephropathy model (for macroalbuminuria and ESRD);

- YLDs from the retinopathy-diabetic foot model (for uncomplicated type 1 diabetes, moderate and severe visual impairments, sores, ulcers and lower extremity amputation.

The current BoD and health gains from reduced non-fatal macrovascular complications have not been estimated here.

4.3.2.2 The steady-state

The model of the steady-state estimated the BoD of type 1 diabetes for one year with a set of initial conditions *A(j,s)* based on the age specific profile of a hypothetical birth cohort modelled over 100 years using again equations (2) and (3) for modelling transitions in the population with and without type 1 diabetes. The differences from the

model for the first five years are the assumptions that: the size of the population does not change (as those who die are replaced with individuals of the same age); and that the hypothetical cohort has received intensive treatment from the onset of type 1 diabetes, and hence has also been subject to lower transition probabilities from the onset of the disease. In this model, the number of diabetic patients in each age group is the same as in the initial population of the model for the first five years, but they all have blood glucose under the recommended level and fewer of them have developed complications. The 'steady state' model reflects the delay between the intervention and its full benefits, estimating the reduction in burden of disease *as if* the current diabetic population was subject to treatment from the onset of diabetes and does not take into account recent predictions of increasing future incidence rates (Forouhi, Merrick et al. 2006). It therefore underestimates the likely future burden of disease. The initial population of the steady state model is a stable population, where everybody has blood glucose below the recommended level. At the end of the year the population progresses according to transition probabilities characteristic of diabetic patients with good glycaemic control.

Estimates of BoD in DALYs were estimated by Equation 7 (using the same notation as Equation 4):

Equation 7

$$DALYs = YLLs + YLDs =$$

$$= \sum_{j=0}^{99} \sum_{s=0}^{k} A(j,s) * \sim'(j,s) \int_{1}^{L(j+1)} e^{-rt} dt + \sum_{j=0}^{99} \sum_{s=0}^{k} A(j,s) * w(s) * e^{-r}$$

4.3.2.3 Data

As most death certificates of diabetic patients do not report diabetes as a cause of death, official statistics that report causes of mortality are unreliable for diabetes. So we estimated mortality from diabetes using mortality rates from longitudinal studies (Rossing, Hougaard et al. 1996, Soedamah-Muthu, Fuller et al. 2006) and prevalence data from Harvey et al (Harvey, Craney et al. 2002). We estimated the presence and degree of severity of complications using the best evidence we could find, including

studies conducted in the US or the Netherlands. A systematic review of the evidence, although needed and valuable, was beyond the scope of this paper. Details on the assumptions needed to deal with missing data are given in the last column of Table 4.

Table 4 Data sources and assumptions on missing data

Information	Source	Description/Evaluation	Assumptions on missing data
Incidence α	National Clinical audit Support Programme (2005)	This is an overview of diabetes and diabetes care in England. Coverage is partial: about 22% of eligible PCTs, GP practices and Hospitals registered; about 34% of paediatric units.	It gives data for 0-16 years old. We assumed diabetes onset is before age 35 using the incidence rate for 0-16 also for people 17-35 years old. This assumption implies a slight overestimate of the burden of diabetes in the model for the first five years. We made the standard assumption that all Type 1diabetic patients are diagnosed.

Information	Source	Description/Evaluation	Assumptions on missing data
Current population with Type 1 Diabetes by age group *A(0,j)*	Harvey, Craney et al. (2002); Diabetes UK (2004); Diabetes UK (2004); Health and Social Care Information Centre (2004). Details for Health and Social Care Information Centre (2004) were provided upon request by NHS Health and Social Care Information Centre	Diabetes UK (2004) gives estimates for the 17,000 children with Type 1 Diabetes which are based on audited data of about 10,000 children. Diabetes UK (2004) estimates the diabetic population but in wide age bands. Health and Social Care Information Centre (2004) is the QOF data at GP level (carefully audited as the basis for the new GMS contract) and reports the total number of diabetic patients in the surgery (but lacks details on type of diabetes and age). Harvey, Craney et al. (2002) reports age-specific prevalence estimates of Type 1 Diabetes for the County of Clwyd in North Wales.	There is no single definitive source of audited prevalence data of Type 1 Diabetes for all age groups. We used Harvey, Craney et al. (2002). This assumes that the estimates are representative for England.

Information	Source	Description/Evaluation	Assumptions on missing data
Number of people by degree of severity s	Various authors (Klein, Klein et al. 1989, Klein, Klein et al. 1989, Klein, Moss et al. 1989, Diabetes Control and Complication Trial 1990, Diabetes Control and Complication Trial 1993, Klein, Klein et al. 1994, Diabetes Control and Complication Trial 1996, Rossing, Hougaard et al. 1996, Brailsford, Davies et al. 1998, Klein, Klein et al. 1998, Davies, Roderick et al. 2001, Niessen 2002, Soedamah-Muthu, Fuller et al. 2006)	These data do not refer to the English population and some are ten years old. Most of these sources report transition probabilities based on longitudinal studies but the original dataset of the study is not available. Data are usually reported for the whole population in the study or for wide age groups.	We needed to make some heroic assumptions to generate the initial distribution of diabetic population across degrees of severity of renal and eye disease complications. We used our model to generate a sample population of 100,000 susceptible and projected it over 100 years. We assumed that the proportion of people in each degree of severity for each age was representative of the current population of that age. We applied these proportions to the $A(0,j)$ as estimated above.
Transition probabilities in nephropathy (excluding mortality rates)	Diabetes Control and Complication Trial (1990, 1993, 1996); Niessen (2002)	Niessen (2002) developed Markov chain models of diabetes complications, also on the DCCT study . The DCCT study was a major, multi-centre study of 1,441 diabetic patients in the US, lasted nine years. The study quantifies the effect of intense treatment on progression in microvascular sequelae. These data do not refer to the English population and some are ten years old. They report transition probabilities based on longitudinal studies but the original dataset of the study is not available. Data are usually reported for the whole population in the study or for wide age groups.	We assumed that the transition probabilities apply to the current diabetic population in England.

Information	Source	Description/Evaluation	Assumptions on missing data
Mortality rates in nephropathy model	Diabetes Control and Complication Trial (DCCT; 1996) ; Rossing, Hougaard et al. (1996); Soedamah-Muthu, Fuller et al. (2006)	Rossing Hougaard et al. (1996) is a cohort study of a 10-year observational follow up of 939 adult patients with insulin dependent diabetes in Denmark. Soedamah-Muthu, Fuller et al. (2006) gives all cause mortality rates from the General Practice Research Database. This is a reliable source of data for England, based on a 7-year longitudinal study of 7,713 patients with Type 1 Diabetes .	We used an average between the DCCT study (1996) and Rossing, Hougaard et al. (1996). The aggregate mortality rate is similar to that in Soedamah-Muthu, Fuller et al. (2006), which could not be used directly because it does not specify complications severity.
Transition probabilities in retinopathy (including mortality rates)	Various authors (Klein, Klein et al. 1989, Klein, Klein et al. 1989, Klein, Moss et al. 1989, Klein, Klein et al. 1994, Klein, Klein et al. 1998, Davies, Brailsford et al. 2000, Davies, Roderick et al. 2001)	The DCCT study had a high degree of uncertainty on its incidence estimate for retinopathy because only a small group of participants who did not have retinopathy at baseline stayed in the study for 9 years (Mount Hood 4 Modeling Group 2007). We used another study on the progression of retinopathy in our model, the Wisconsin Epidemiologic Study of Diabetic Retinopathy (WESDR) following Davies *et al.*(2000). WESDR data do not refer to the English population and are fifteen years old. They report transition probabilities based on longitudinal studies but the original dataset of the study is not available. Data are usually reported for the whole population in the study or for wide age groups.	We assumed that the transition probabilities apply to the current diabetic population in England.

Information	Source	Description/Evaluation	Assumptions on missing data
Incidence rates of amputations, sores or ulcers	Moss, Klein et al. (1992)	Moss, Klein et al. (1992) provide 4-year incidence rate of amputation and sores or ulcers by characteristics of the population, including the presence and degree of severity of diabetic retinopathy ($p<.0001$)	We assumed that the incidence rates from each degree of retinopathy apply to the current diabetic population in England.
Mortality rates non diabetic population	Soedamah-Muthu, Fuller et al. (2006)	Data about the non-diabetic population refers to a control group matching the diabetic population under study and is not representative of the general non-diabetic population.	We used the age-specific mortality rates for the population with Type 1 Diabetes to generate the expected deaths in one year. We subtracted this data from the total number of deaths from all causes per age group (Office of National Statistics 2003) and derived mortality rates for the non-diabetic population.
Disability weights	Stouthard, Essink-Bot et al. (1997)	The disability weights were estimated by the Dutch study that developed disability weights applicable to developed countries.	In the absence of disability weights in the presence of co-morbid conditions we assumed that the weights are additive.

The benefits of intensive glucose control are the difference between estimates of BoD with and without the intervention. In the absence of evidence on the level of disability from co-morbid conditions (e.g. retinopathy and nephropathy affecting the same person), we assumed that the disability from renal complications could be meaningfully added to the disability from eye and foot complications, that is, for instance, the disability of a patient with both nephropathy and severe retinopathy contributes 0.29+0.43 YLDs (0.72 YLDs). The Dutch study for disability weights provided weights for these separate complications (Stouthard, Essink-Bot et al. 1997). For comparison, this means that a year spent with diabetic nephropathy and severe visual impairments would have the same disability weight as, e.g., schizophrenia with several psychotic episodes and some permanent impairments, or a year of a child/adolescent in permanent stage with complex not curatively operable congenital heart disease. Patients with all three complications at the highest degree of severity would contribute 0.91 YLDs (0.29+0.43+0.19).

Our estimates of the potential net gain in output from intensive glucose control are based on estimated unit costs as outlined in Table 5 and Table 6.

Table 5 Cost of monitoring glucose levels and prescribing insulin

Item	unitary cost	Conventional treatment		Intensive treatment[*]	
		items per year	annual cost per diabetic patient	items per year	annual cost per diabetic patient
lancets	£0.07	730	£51	1,278	£89
glucose test stripes	£0.87	730	£633	1,278	£1,107
glucometer	£40.00	0	£11	0	£11
insulin	£0.26	730	£190	1,278	£332
insulin syringes	£0.15	730	£110	1,278	£192
insulin pen	£15.00	0	£4	0	£4
diabetes clinic visits	£106.00	1	£106	12	£1,272
nursing staff	£34.00	-	£-	9	£295
total			£1,105		£3,303

*When we run the model replacing monthly visit with specialist nurses on the phone, we change the intensive treatment assuming one annual visit at the clinic and three telephone conversations per week of 10 minutes each with the specialist nurse, for a total cost of intensive treatment of £2,726 per patient per year; when we tested the cost implications of using insulin pumps, we used the average annual cost of the pump and consumables (including savings from reduced use of insulin) from a recent Health Technology Assessment study (Colquitt, Green et al. 2004)

assuming monitoring was provided through telephone conversation with a specialist nurse, for a total annual cost of £4,333 per patient per year.

These costs assume the definition of intensive glucose control as it occurred in the original longitudinal studies (Rossing, Hougaard et al. 1996, Soedamah-Muthu, Fuller et al. 2006) and consisted of administration of insulin at least three times a day (or with an insulin pump); insulin dosage, dietary intake and exercise adjustment according to results of self-monitoring of blood glucose; self-monitoring of blood glucose at least four times per day; monthly measurement of HbA_{1c}; monthly visit at the diabetic centre; and specialist calls during the month to review regimens. We ran three sensitivity analyses of our estimates of costs. First, we replaced monthly clinic visits with telephone calls from a specialist nurse, which is a more realistic assumption of what might happen outside research conditions and does not appear to reduce health benefits (Thompson, Kozak et al. 1999). Second, we assumed the use of insulin pumps rather than multiple daily injections (although there is some evidence that insulin pumps are clinically more effective than multiple daily injections, most of the benefit is in terms of hypoglycaemic events or practical convenience and would not significantly affect microvascular complications). Third, we allowed for the cost of treating a diabetic patient to be about 30% higher than a non-diabetic one and about 27% above the average cost for the general population (Currie, Kraus et al. 1997).

Table 6 Cost of treating microvascular complications

Degree of severity	Data source	conventional care		intensive care[#]	
		cost 1st year	cost following years	cost 1st year	cost following years
microalbuminuria	(Gordois, Scuffham et al. 2004)	£44[a]	£44[a]	£44[a]	£44[a]
macroalbuminuria	(Gordois, Scuffham et al. 2004)	£4,215[a,b,c]	£4,215[a,b,c]	£3,791[a,b]	£3,791[a,b]
End Stage Renal Disease - dialysis	(MacLeod, Grant et al. 1998, Mowatt, Vale et al. 2003, Department of Health 2004, Gordois, Scuffham et al. 2004)	£21,152[d]	£21,152[d]	£21,152[d]	£21,152[d]
End Stage Renal Disease - transplant	(Department of Health 2004)	£18,727[e]	£240[e]	£18,727[e]	£240[e]
Background Diabetic Retinopathy	(Department of Health 2004)	£89	£55	£-	£-
Proliferative Diabetic Retinopathy visits	(Department of Health 2004)	£89	£55	£-	£-
Laser treatment	(Department of Health 2004)	£602	£-	£602	£-
PDR cost	visit + laser treat at onset	£691	£55	£602	£-
Severe vision loss (blind one eye)	(Clarke, Gray et al. 2003)	£872	£281	£872	£281
Sores/ulcers	(Department of Health 2004)	£162[f]	£45[f]	£162[f]	£-
Amputation	(Department of Health 2004)	£6,248[g]	£73[g]	£6,248[g]	£73[g]

4.3.3 Results

4.3.3.1 Health gains

Table 7 and the following Figures report annualised estimates for various measures of reductions in BoD and gains in DALYs.

The yearly estimates of the current BoD from type 1 diabetes in England was about 2,000 deaths; 66,000 YLLs and 34,000 YLDs; 100,000 undiscounted and 63,000 discounted DALYs. In the first five years and the steady state the estimated benefits from intensive glucose control are reductions in the BoD of about: 10 and 400 deaths; 300 and 11,000 YLLs; 1,200 and 11,000 YLDs; and 1,500 and 24,000 undiscounted DALYs; and 1,200 and 18,000 discounted DALYs. These are underestimates of the benefits as they do not include reductions in BoD from acute diabetes events (ketoacidosis), non-fatal myocardial infarctions, non-fatal strokes and coronary revascularisations, and this qualification also applies to our estimates of the monetary valuation of these benefits.

Table 7 Burden of Disease and its reduction through intensive glucose control in the first five years and in the steady-state

	Burden of disease with current care (current BoD)		Short term burden reduction from intensive glucose control (100% compliance)		Steady state: burden reduction from intensive glucose control (100% compliance)
	First 5 years (annualized)	First year only (sensitivity analysis)	First 5 years (annualized)	First year only (sensitivity analysis)	
Deaths ('000s)	2	2	0.01	0	0.4
Monetary value of deaths (£m)	2,300	2,300	9	0	440
YLLs ('000s)	66	65	0.3	0	11
YLDs from renal complications ('000s)	8*	7	0.2	0	3**
YLDs from eye complications ('000s)	23	23	0.9	0.3	8
YLDs from diabetic foot ('000)	3	1#	0	0	0.4
YLDs total ('000)	34	31	1.2	0.3	11
DALYs ('000s) (undiscounted)	100	96	1.5	0.3	23.5
DALYs ('000s) (discounted)	63	64	1.2	0.3	17.8
Monetary value of DALYs averted (discounted, £m)	1,900	1,900	35	9	535

Figure 8 shows the BoD in undiscounted DALYs from type 1 diabetes and the estimated reductions in the first five years and in the steady state from intensive glucose control. This shows that much of the current BoD from type 1 diabetes is unavoidable even with 100% compliance with intensive glucose control. Figure 9 to Figure 12 show the distribution by age group of deaths, renal and eye diseases and amputations for the

first five years and in the steady state. All these Figures bring out the common message that the benefits of intensive control appear to be much greater in the long run than the short run.
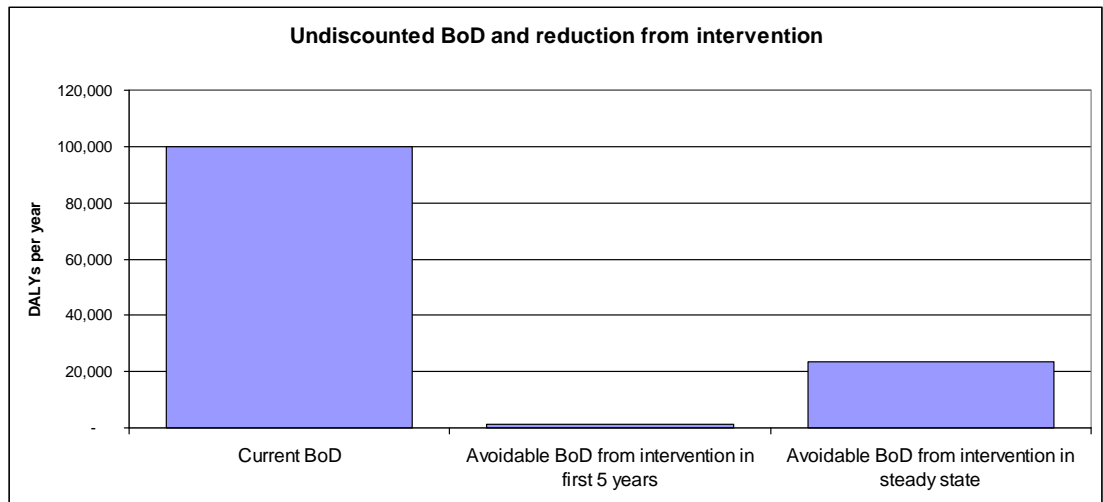
Figure 8 Estimates of BoD (undiscounted DALYs) from type 1 diabetes and reductions in the first five years and steady state from intensive glucose control

Figure 9 'Avoidable' deaths through intensive glucose control in the first five years and in the steady state by age at the beginning of the intervention

Renal complications - annual reduction from intervention
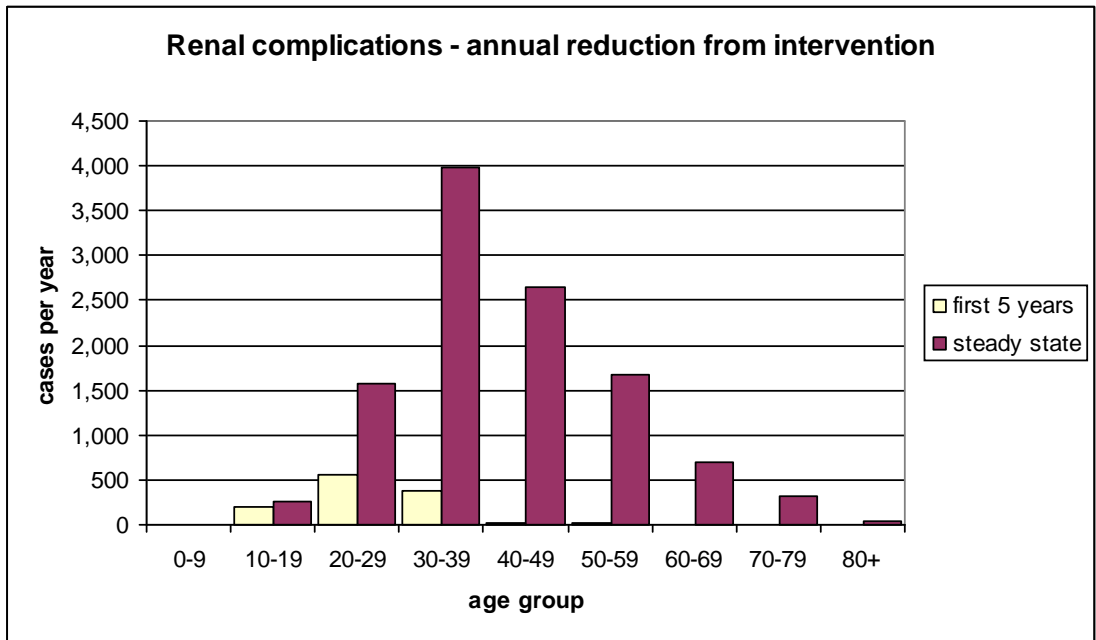
Figure 10 'Avoidable' cases of overt proteinuria and end-stage renal disease through intensive glucose control in the first five years and in the steady state by age at the beginning of the intervention
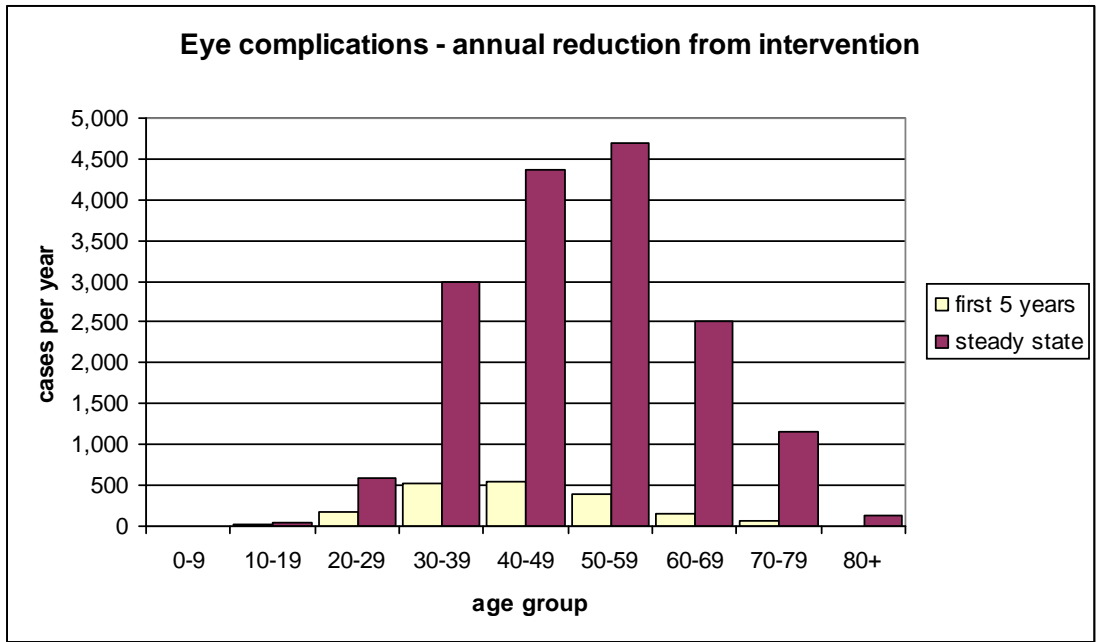
Figure 11 'Avoidable' cases of severe visual disorders through intensive glucose control in the first five years and in the steady state by age at the beginning of the intervention
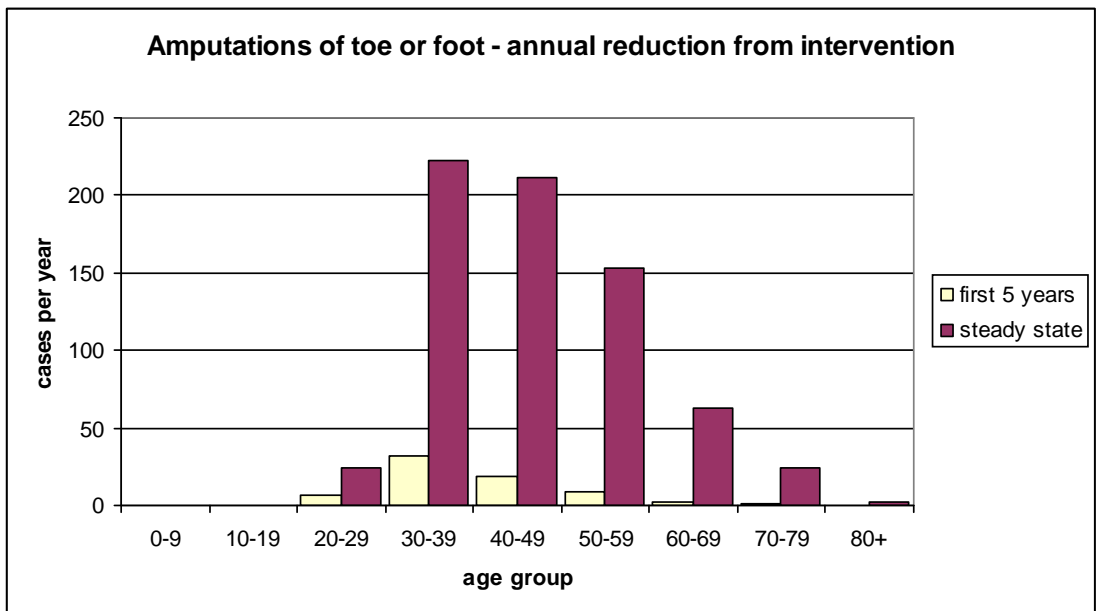
Figure 12 *Avoidable' cases of amputation through intensive glucose control in the first five years and in the steady state by age at the beginning of the intervention*

4.3.3.2 Net costs and net gains in output

We estimated that:

- the annual cost to prescribe, monitor and treat microvascular complications of diabetes type 1 in England is currently about £380m (most of which is spent on monitoring the disease, prescribing insulin and treating renal complications as summarised in Table 8);

- the introduction of intensive monitoring increases the cost of insulin prescribing and monitoring by £350m and reduces the annual costs of complications by £20m in the first five years; and by £370m and £100m respectively in the steady state;

- reductions in costs for eye diseases are mainly realized in the short run (£8m compared with long-run savings of £12m);

- reductions in costs for renal complications are mainly realized in the long run (£84m compared with short-run savings of £13m).

Table 8 Annual costs and savings (negative figures) from intensive glucose control in the first five years and the steady state

	Conventional care (current spend) in £ m		Intensive glucose control assuming monthly visit at diabetic clinic as in original DCCT study		Intensive glucose control replacing monthly visits with more frequent telephone supervision by specialist nurse	
	In first year	In first five years (annualized)	First five years: change in expenditure (annualized) in £ m	Steady state: change in expenditure in £ m	First five years: change in expenditure (annualized) in £ m	Steady state: change in expenditure in £ m
Insulin prescription and glucose monitoring	187	175	+ 349	+ 373	+257	+275
Treatment of nephropathy	175	169	- 13	- 84	-6	-79
Treatment of retinopathy	14	14	- 8	- 12	-2	-9
Treatment of diabetic foot	8	8	- 0.5	- 4	-0.5	-4
Expenditure	383	366	+ 328	+ 272	+249	+182

The estimates of costs and savings of intensive glucose control in the long run are of what these would be in a year: i.e. we have not examined these using discounting. If the savings were discounted, these would be negligible because of the long time lags between the start of incurring the costs of intensive glucose control and making these savings from reduced use of health services. In our estimates, the expected savings from reduced complication do not offset the increased cost for monitoring and prescribing. There is, however, evidence that these costs can be reduced. It is not necessary to have monthly visits to the diabetic clinic: a telephone discussion with a specialist nurse three times a week to adjust insulin dose and diet to the observed glucose levels was successful in reducing HbA_{1c} below the recommended level at six months (Thompson, Kozak et al. 1999). This practice would reduce the extra costs to about £270m and hence extra net costs to about £180m in the steady state.

We used these costs in Table 9, which gives results from comparing costs and benefits in the short and in the long run. This shows that the net cost of intensive glucose control in the short run are about six times larger than the monetary value of the health benefits. If the intervention were to be introduced and sustained over its run-in period, however, the monetary value of health benefits would be three times the net cost of the intervention.

Table 9 Net gain in output in the first five years and in the steady state

	Intervention in first five years in £m	Intervention in the steady state in £m
Monetary value of DALYs averted (at £30k per DALY, discounting YLLs)	30	530
Extra costs	250	180
Gain (loss) in output	**(220)**	**350**

4.3.3.3   Model validity

Assessing the validity of our model is difficult, because routinely available data usually refers to type 1 and type 2 diabetes combined (even when these labels are used, most patients belong to an 'unspecified' type of diabetes). The available combined figures are likely to be a reflection of prevalence and incidence rates of diabetes type 2, which is about 90% of the diabetic population and is not representative of the population with type 1. In fact, type 1 typically has a much younger onset compared to diabetes type 2 and the duration of diabetes is one of the main risk factors of complications. Where data on type 1 diabetes exist, usually either there is no breakdown by age, or data are not for England, or they are not routinely available and hence could not be used as input for our initial condition. We now discuss how we compared the prevalence of complications resulting from our initial condition with data from the literature.

Diabetic nephropathy

Table 10 compares prevalence rates of renal complications by degree of severity in our model and in the literature. Our estimates are generally consistent with data from empirical analysis, although we might overestimate the prevalence of end stage renal disease. The Renal Registry in England estimates that 30,000 people are receiving renal replacement therapy (including those who received a kidney transplant) and 5,000 started renal replacement therapy in 2002 (Ansell, Feest et al. 2003). Our model estimates that there are about 6,000 people with End Stage Renal disease and 1,000 new cases per year among patients with type 1 diabetes which would correspond to about 16% and 20% respectively of all patients receiving renal replacement therapy. This might be an overestimate and we will indicate the health benefits and cost component separately for ESRD in the result section for transparency.

Table 10 Prevalence rates of renal complications

	Normo-albuminuria prevalence	Micro-albuminuria prevalence	Macro-albuminuria prevalence	End Stage Renal Disease prevalence
Model estimates (conventional care)	57%	28%	11%	4%
Harvey, Rizvi et al. (2001) N=1,297; Wales, UK	61.4%	At 15-29 years duration: 27.2%; Below 5 years duration: 14%	11%	1.8%
DARTS (2001)	n/a	n/a	n/a	1%
Finne *et al.* (2005) n=20,005; Finland	n/a	n/a	n/a	Cumulative incidence at 20 years from onset = 2.2%; at 30 years from onset = 7.8%

Diabetic retinopathy

Estimates of diabetic retinopathy for the population with type 1 diabetes vary greatly. A recent literature review on prevalence reports rates between 0 and 84% for diabetic retinopathy in general; and between 1.1% and 25% for Proliferative Diabetic

Retinopathy (Williams, Airey et al. 2004). We report in Table 9 the prevalence of diabetic retinopathy in the WESDR study (which we used as a basis of our model) and the estimated prevalence based on the model by Davies *et al.* (2000) who used the same dataset. Table 11 shows that our estimates are reasonable, once we assume the WESDR data can be used for England. Furthermore, the 9-year cumulative incidence of background diabetic retinopathy in our model is 81%, which is similar to estimates from the EAGLE model (77%), which also uses the WESDR study (Mount Hood 4 Modeling Group 2007).

Table 11 Prevalence rates of eye complications

	No retinopathy	Background Diabetic Retinopathy	Proliferative Diabetic Retinopathy	Severe visual loss (including blindness)
Model estimates (conventional care)	26%	40%	23%	8%
Klein et al. (1984; US)	30%	46% (of which 17% severe non-proliferative diabetic retinopathy)	14%	9%
(Davies, Roderick et al. 2001)	20%	49%	30% (25% PDR and 5% untreatable)	

Diabetic foot

Health Episode Statistics (HES) report a total of 10,700 finished consultant episodes (FCEs) of amputation, including traumatic amputations and procedures associated with diabetic foot such as amputation of stumps. Our model predicts about 1,300 cases of amputation a year in the population with type 1 diabetes (toe and foot amputation) which would correspond to about 12% of all amputation procedures conducted in England (including diabetes type 2 and non-diabetic patients). From the publicly available HES data we could not identify what proportion of the total FCEs referred to

people with type 1 diabetes. Results for diabetic foot are reported separately from those of renal and eye complications for transparency.

We compared our results with 4-year incidence rates of amputation and sores/ulcers in Moss et al. (1992) and show results in Table 10. Our prevalence estimates are based on the work by Moss and, as one should expect, the incidence rates correspond. It is reassuring, however, to observe consistency in the overall incidence rate (last column in Table 12), which is an output of our model and our assumptions on those with different severities of retinopathy.

We did not find data on prevalence or incidence of diabetic foot for the population with type 1 diabetes in England to validate the diabetic foot model externally. Our model, however, estimates an annual incidence of 2.8% for sores/ulcers and 0.7% for amputation, which is similar to 2.1% and 0.6% mean national incidence rates for type 1 diabetes in the Netherlands (Ortegon, Redekop et al. 2004).

Table 12 4-year incidence rates of sores/ulcers and foot/toe amputations

4-year incidence of sores/ulcers				
	In patients with no retinopathy	In patients with mild or moderate retinopathy	In patients with PDR	All patients
Model estimate (conventional care)	5.6%	9%	18.7%	11.5%
Moss *et al.* (1992)	5.8% (n=273)	9% (n=440)	18.3% (n=166)	9.5% (n=879)
4-year incidence of amputation				
	In patients with no retinopathy	In patients with mild or moderate retinopathy	In patients with PDR	All patients
Model estimate (conventional care)	0%	1.4%	8%	3%
Moss *et al.* (1992)	0% (n=273)	1.4% (n=440)	7.8% (n=166)	2.2% (n=879)

Intensive glucose control

We compare our model estimates on the relative risk of renal and eye complications with those in the DCCT study and in other diabetes models from the literature in Table 13. Our model is consistent with the other studies in estimating the reduction in retinopathy and might slightly overestimate the reduction in renal complications by 15%. This overestimate does not have a significant impact on the estimate of the 'avoidable' Burden of Disease, which is mainly determined by a reduction in eye complications. The cost of renal complications, however, is the principal component of

the savings in treating complications in the intensive care scenario in the steady state. Assuming a 15% lower savings from fewer renal complications, however, would not have an impact on the order of magnitude of our results: the net loss in the first five years would be unaffected and the net gain in the steady state would reduce from £350m to £330m.

Table 13 also reports estimates in the reduction of neuropathy, but our model does not model these complications explicitly. The relative risk in 9-year incidence of sores/ulcers and amputation in the intensive glucose control scenario is 0.95 and 0.91 which is much lower than the 0.47 relative risk of neuropathy at clinical examination in the DCCT study. A reduction in neuropathy does not imply an equivalent reduction in diabetic foot, however, the relatively small reduction in diabetic foot estimated in our model compared to the relatively high reduction in neuropathy indicates that we might have underestimated the 'avoidable' burden of disease.

Table 13 Estimates of the risk reduction in 9-year incidence from microvascular complications

	DCCT study	Our model	EAGLE model	CORE model	Archimedes model
Microalbuminuria	0.59	0.68	0.61	0.54	0.53
BDR	0.27	0.33	0.90	0.37	0.32
Neuropathy	0.47	n/a	0.29	0.39	n/a

Costing

The estimate of the current, annual cost of monitoring, prescribing and treating microvascular complications amounts to about £2,300 per patient. This is broadly consistent with a recent estimate of the total healthcare cost of treating people with type 1 diabetes in the UK by Currie *et al.* (2007). The annual healthcare cost of participants in their survey spent about £3,200 a year, including treatment and prevention of macrovascular complications such as stroke and myocardial infarction.

Our estimate for the current cost of treating renal complications and diabetic foot are also in line with other estimates of the cost for type 1 diabetes in the UK. The estimate of £175m for nephropathy is consistent with Gordois *et al.* (2004) estimate of £152m (range £125-230m); the estimate of £8m for *incident* cases of diabetic foot seems consistent with the £35m (range £16-61m) of *prevalent* cases of diabetic peripheral neuropathy (Gordois, Shearer et al. 2003).

4.3.3.4   Sensitivity analysis

Our estimates of health benefits assume that the transition probabilities and mortality rates observed in longitudinal studies, in which the participants were generally between adolescence and middle age (Klein, Klein et al. 1989, Klein, Klein et al. 1989, Klein, Moss et al. 1989, Diabetes Control and Complication Trial 1990, Diabetes Control and Complication Trial 1993, Klein, Klein et al. 1994, Diabetes Control and Complication Trial 1996, Klein, Klein et al. 1998), apply to the type 1 diabetes population in England, and the confidence interval estimates of mortality rates in older cohorts are particularly wide (Soedamah-Muthu, Fuller et al. 2006). To test the robustness of the model to these assumptions, we estimated the effects of excluding from the analysis all people older than 75 years. As this reduced these estimates by about one per cent, we concluded that they are robust to our assumptions of transition probabilities and mortality rates of older cohorts.

A crucial assumption in our estimates of the impacts of intensive glucose control is that there is compliance at levels comparable to those of the DCCT study. There is a linear relationship between the proportion complying and the reduction in BoD in DALYs. Figure 13 shows the estimated relationship for the steady-state model: a 1% increment in the proportion receiving intensive treatment and complying as in experimental conditions corresponds to a reduction of 240 DALYs (or 180 discounted DALYs).

Sensitivity analysis on compliance

(Chart: y-axis "Burden of disease ('000)" from - to 120; x-axis "%compliance" from - to 100)

Avoidable BoD with intensive glucose control

Unavoidable BoD
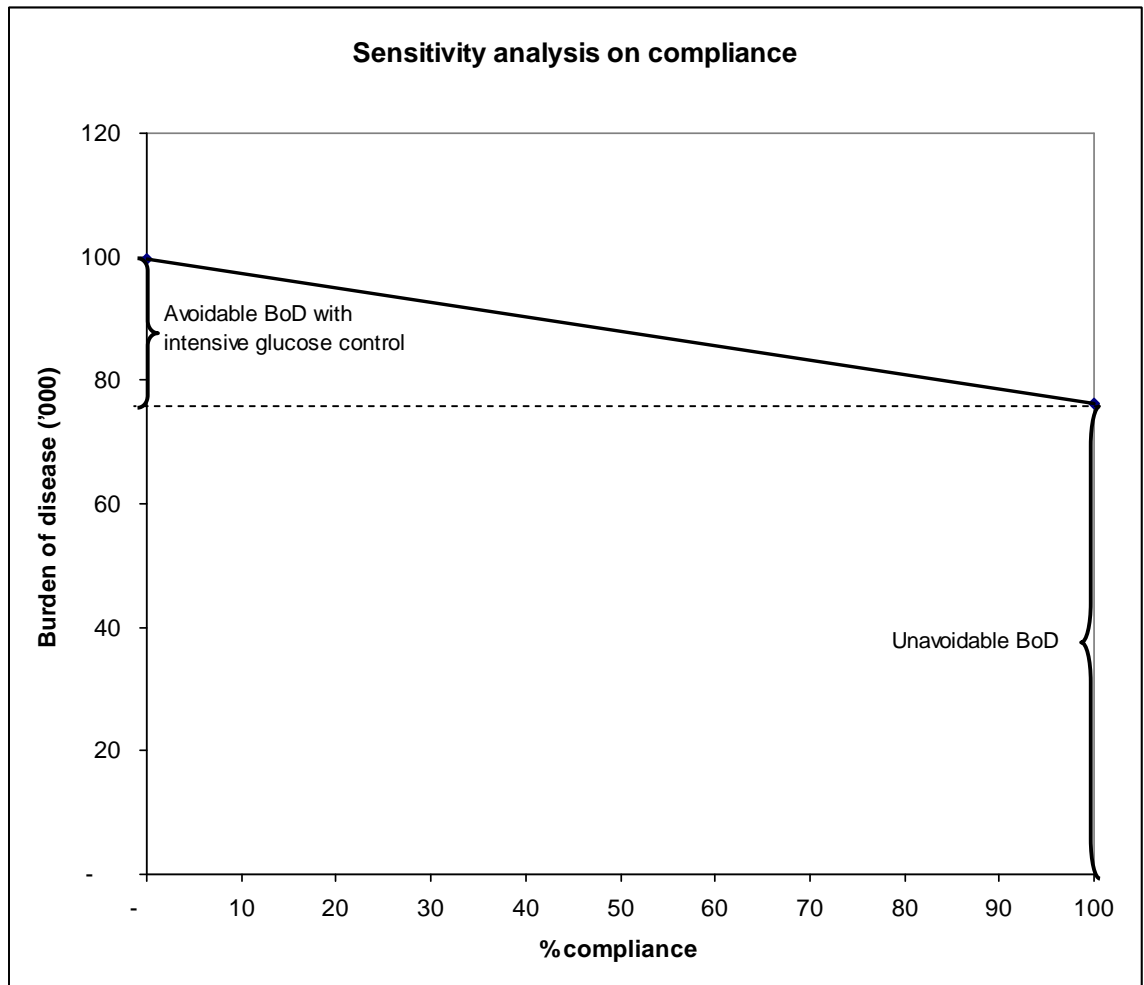
Figure 13 Estimates of annual BoD in undiscounted DALYs from type 1 diabetes in the steady state from 0 to 100% proportion of population complying with intensive glucose control

Another assumption worth testing is that of offering intensive glucose monitoring to all patients, including children and adolescents. On one hand, DCCT researchers were cautious about the use of intensive glucose monitoring in children because of the increased risk of hypoglycaemic events. On the other, the low proportion of adolescents with glucose concentration below the recommended level might signal the rebellion against parental or medical authority suggesting the possibility of very low compliance rates with intensive treatment. Our model, however, assumes that most microvascular complications arise after the age of 15 (with the exception of ulcers which we assume

occurs at any age and amputation which we assume occurs only in people older than 30) and excluding these age groups from the analysis would not significantly impact on the estimates of health benefits: the estimate of the 'avoidable' burden of disease offering intensive glucose control only to people aged 20 or older is just 0.1% lower both in the short run and in the steady state. This result should be interpreted with caution because our Markov chain assumes that the incidence of microvascular complications from the age of 15 (or 30 for amputation) is independent from glucose concentrations maintained in childhood and we did not find evidence to support or dismiss this assumption. Clearly, however, the exclusion of children and adolescents from intensive glucose monitoring would have an impact on costs. The sensitivity analysis shows that the reduction in costs by providing intensive treatment only to patients who are 20 years old or older is 50m in the short run and 60m in the steady state which would imply a lower loss in net output in the first five years (£170m compared to £220 in the base case) and a higher net gain in output in the steady state (£410m compared to £350 in the base case).

To test the robustness of our cost estimates, we also assumed the use of insulin pumps to replace the base case assumption of multiple daily injections. There is growing interest in the use of insulin pumps as an alternative treatment to manage diabetes. In comparison with multiple daily injections, insulin pumps improve quality of life in terms of their higher efficacy on controlling glucose concentration, of reducing incidence of adverse events (i.e. hypoglycaemic events) and their flexibility of lifestyle. However, they are not currently considered cost-effective because of their higher cost (Colquitt, Green et al. 2004). If all patients use insulin pumps (using the average annual cost from Colquitt *et al.* (Colquitt, Green et al. 2004)), the incremental cost of insulin prescribing and monitoring would be £515m in the short run (annualized figure over first 5 years) and £547m in the steady state. This would consistently lower the net gains from Table 7; however, although this is an extreme and unrealistic assumption, the results would still be a loss in the short run (£470m net loss in output) and a gain in the steady state (£75 net gain in output).

We also assumed a cost of acute care (inpatient and outpatient) 27% higher than the national average cost (Currie, Kraus et al. 1997). Under this scenario, the estimate of the total current cost of insulin and microvascular complication increases from £370m to

£515m per year; the increase in spending from intensive glucose monitoring reduces from £250m to £210m and from £180m to £105m in the first five year model and in the steady state respectively assuming telephone discussion with a specialist nurse rather than monthly visits to the diabetes clinic; from £330m to £285m and from £270m to £190m in the first five years and in the steady state model assuming monthly visit as in the original DCCT study. This is as expected because the higher cost of acute care increases the savings from treating microvascular complications, and this determines a lower net loss in the short run (£170m compared to £220m in the base case assuming monitoring with nurse on the phone) and a higher gain in net output in the steady state (£430m compared to £350m in the base case).

We ran a sensitivity analysis on the cost of peritoneal haemodialysis, assuming the use of continuous ambulatory peritoneal dialysis (CAPD) instead of continuous cyclic peritoneal dialysis (CCPD) which is cheaper although currently not considered cost-effective (MacLeod, Grant et al. 1998). The resulting reduction in cost does not significantly affect results (£169m current cost vs. £162 in base case; same reduction in short run; 76m reduction nephropathy cost in steady state vs. £79 base case cheap or £84 base case DCCT).

Finally we tested the monetary value of health benefits with two sensitivity analyses. First, we use a lower figure of £20,000 as advocated by part of the literature (e.g. Williams 2004). Second, we used the health benefits using the value of a statistical life (HM Treasury, 2003). Both sensitivity analyses confirm a net loss of more than £200m (£230 and £240 respectively) in the short run and a net gain above £180m in the steady-state (£180 and £260 respectively).

4.3.4   Discussion

This paper aimed to explore how disease models could be used in setting priorities for strategic commissioning for populations. To set priorities using evidence, it is essential to estimate impacts of interventions at the level of populations, but this can only be done by disease modelling. An obstacle to the use of such models is that they are often highly complex, demand rich sources of data, and take a long time to develop.

We have described the development of a parsimonious transparent model of the size and timing of costs and benefits of intensive glucose control in the type 1 diabetes population, which has produced approximate estimates that are adequate for priority setting as shown by validation and sensitivity analysis. This paper has shown, that:

- The current BoD from type 1 diabetes disease from microvascular complications and premature mortality is about 100,000 DALYs of which one third is attributable to low quality of life and two thirds to premature death. This is an underestimate of the current burden of disease from diabetes type 1, because it does not include disability due to acute diabetic events (ketoacidosis), non-fatal myocardial infarctions, non-fatal strokes and coronary revascularisations.

- Introducing intensive glucose control, in the short run, will almost double the spend for monitoring glucose, prescribing insulin and treating microvascular complications but have small effect in reducing the burden of disease (a 1-2% reduction).

- Introducing intensive glucose control, in the long run, reduced the BoD by about 30%: with this being approximately equally divided into benefits from lower mortality and lower morbidity. The lower cost of treating complications in the long run will still not offset the increased cost of monitoring and insulin prescribing (50% higher than conventional care); however, the value of the health benefits more than compensates the increase in costs.

The study also highlighted inadequacies in the data that are routinely collected in England: chronic diseases, such as diabetes, are frequently not reported on death certificates thereby masking the impact of long term consequences; there are significant gaps in data on the type of diabetes, age of the patient, duration of diabetes, presence of complication with degree and duration, sex and current treatment regime. In England many of these data are in principle available for purchase from the General Practice Research Database that offers a sample of about 7,500-8,000 type 1 diabetes patients, that is about 4.5% of the total type 1 diabetes population (Soedamah-Muthu, Fuller et al. 2006, Soedamah-Muthu, Fuller et al. 2006). These data ought to be collected in disease registers to support evidence-based policy making. An initiative that has the

potential to provide this information in England is the current national Programme for IT in the NHS, *Connecting for Health*.

The final point concerns the approach to modelling illustrated by this paper. In setting priorities, information on costs and benefits in the short and long run for options for type 1 diabetes is obviously insufficient. We have applied our approach to a number of different interventions: suicide prevention, treatment of depression, prescribing of statins to reduce cholesterol, and various options for the prevention and treatment of strokes [86]. In all this work, it seems to us that relatively simple models, similar to that in this paper described for type 1 diabetes have been adequate in making comparisons for setting priorities for strategic commissioning. Indeed we see the key next step as not the development of more complex models for each of these but developing a simple method to generate adequate estimates for the wide range of interventions that must be considered by strategic commissioners.

4.4   Appendix: Model parameters

Table 14 Parameters shared by the renal and eye disease model: mortality rate of the non-diabetic population and incidence rate of diabetes

age	}	ſ
under 1	5.457821	0.000149
1_4	0.237416	0.000149
5_9	0.101432	0.000149
10_14	0.119732	0.000149
15_19	0.327034	0.000149
20_24	0.493336	0.000149
25_29	0.547027	0.000149
30_34	0.718174	0.000149
35_39	0.966249	0
40_44	1.506267	0
45_49	2.376491	0
50_54	3.811951	0
55_59	5.864163	0
60_64	9.851112	0
65_69	15.91389	0
70_74	26.90164	0
75_79	46.63052	0
80_84	76.82135	0
85+	172.5086	0

Table 15 Incidence rates of sores/ulcers and amputation

Degree of severity of retinopathy	Incidence of sores and/or ulcers	Incidence of lower extremity amputation
No retinopathy	1.45%	0%
Mild or Moderate retinopathy	2.25%	0.35%
Proliferative Diabetic Retinopathy	3.66%	1.95%

Table 16 Transition probabilities in the renal disease complication model

age	Excess mortality				Transition probabilities					
					Intensive glucose control			Conventional care		
	$\sim'(s_0)$	$\sim'(s_1)$	$\sim'(s_2)$	$\sim'(s_3)$	$\gamma_{0\to1}$	$\gamma_{1\to2}$	$\gamma_{2\to3}$	$\gamma_{0\to1}$	$\gamma_{1\to2}$	$\gamma_{2\to3}$
under 1	0.006092	0.008683	0.011820	0.000000	0	0	0	0	0	0
1_4	0.005047	0.006595	0.008166	0.000000	0	0	0	0	0	0
5_9	0.005020	0.006541	0.008071	0.000000	0	0	0	0	0	0
10_14	0.005024	0.006548	0.008084	0.000000	0	0	0	0	0	0
15_19	0.005065	0.006631	0.008229	0.000000	0.022	0.02	0.05	0.034	0.06	0.05
20_24	0.005099	0.006697	0.008345	0.030809	0.022	0.02	0.05	0.034	0.06	0.05
25_29	0.005109	0.006719	0.008383	0.030755	0.022	0.02	0.05	0.034	0.06	0.05
30_34	0.005144	0.006787	0.008503	0.030584	0.022	0.02	0.05	0.034	0.06	0.05
35_39	0.005193	0.006886	0.008676	0.107831	0.022	0.02	0.05	0.034	0.06	0.05
40_44	0.005301	0.007103	0.009054	0.107291	0.022	0.02	0.05	0.034	0.06	0.05
45_49	0.005475	0.007451	0.009664	0.106420	0.036	0.03	0.05	0.057	0.03	0.05
50_54	0.005762	0.008025	0.010668	0.145978	0.036	0.03	0.05	0.057	0.03	0.05
55_59	0.006173	0.008846	0.012105	0.143926	0.036	0.03	0.05	0.057	0.03	0.05
60_64	0.006970	0.010440	0.014896	0.176940	0.036	0.03	0.05	0.057	0.03	0.05
65_69	0.008183	0.012866	0.019140	0.195137	0.036	0.03	0.05	0.057	0.03	0.05
70_74	0.010380	0.017261	0.026831	0.184149	0.036	0.03	0.05	0.057	0.03	0.05
75_79	0.014326	0.025152	0.040641	0.164420	0.036	0.03	0.05	0.057	0.03	0.05
80_84	0.020364	0.037229	0.061775	0.134229	0.036	0.03	0.05	0.057	0.03	0.05
85+	0.039502	0.075503	0.128756	0.128756	0.036	0.03	0.05	0.057	0.03	0.05

Table 17 Transition probabilities in the eye disease complication model

age	Excess mortality				Intensive glucose control			Conventional care		
	$\sim'(s_0)$	$\sim'(s_1)$	$\sim'(s_2)$	$\sim'(s_3)$	$\gamma_{0\to1}$	$\gamma_{1\to2}$	$\gamma_{2\to3}$	$\gamma_{0\to1}$	$\gamma_{1\to2}$	$\gamma_{2\to3}$
under 1	0.0015	0.005	0.033186732	0.0331867	0	0	0	0	0	0
1_4	0.0015	0.005	0.025356124	0.0253561	0	0	0	0	0	0
5_9	0.0015	0.005	0.025152148	0.0251521	0	0	0	0	0	0
10_14	0.0015	0.005	0.025179598	0.0251796	0	0	0	0	0	0
15_19	0.0015	0.005	0.025490551	0.0254906	0.039	0.02544	0.01855	0.13	0.048	0.035
20_24	0.0015	0.005	0.025740004	0.02574	0.039	0.02544	0.01855	0.13	0.048	0.035
25_29	0.0015	0.005	0.025820541	0.0258205	0.039	0.02544	0.01855	0.13	0.048	0.035
30_34	0.0015	0.005	0.026077261	0.0260773	0.039	0.02544	0.01855	0.13	0.048	0.035
35_39	0.0015	0.005	0.026449374	0.0264494	0.039	0.02544	0.01855	0.13	0.048	0.035
40_44	0.0015	0.005	0.027259401	0.0272594	0.039	0.02544	0.01855	0.13	0.048	0.035
45_49	0.0015	0.005	0.028564737	0.0285647	0.039	0.02544	0.01855	0.13	0.048	0.035
50_54	0.0015	0.005	0.030717927	0.0307179	0.039	0.02544	0.01855	0.13	0.048	0.035
55_59	0.0015	0.005	0.033796245	0.0337962	0.039	0.02544	0.01855	0.13	0.048	0.035
60_64	0.0015	0.005	0.039776668	0.0397767	0.039	0.02544	0.01855	0.13	0.048	0.035
65_69	0.0015	0.005	0.048870835	0.0488708	0.039	0.02544	0.01855	0.13	0.048	0.035
70_74	0.0015	0.005	0.06535246	0.0653525	0.039	0.02544	0.01855	0.13	0.048	0.035
75_79	0.0015	0.005	0.09494578	0.0949458	0.039	0.02544	0.01855	0.13	0.048	0.035
80_84	0.0015	0.005	0.140232025	0.140232	0.039	0.02544	0.01855	0.13	0.048	0.035
85+	0.0015	0.005	0.2837629	0.2837629	0.039	0.02544	0.01855	0.13	0.048	0.035

Table 18 Disability weights

Health state	Disability weight (95% C.I)	Health state description in disability weight source	Corresponding EQ 5D+ classification	Source
No complications	0.07 (0.047-0.094)	"Uncomplicated diabetes mellitus"	111111 (90%), 112221 (10%)	Stouthard *et al.* 1997, p.73
Macroalbuminuria and ESRD	0.29 (0.201-0.380)	"Diabetes mellitus with nephropathy"	112121 (80%), 113231 (20%)	Stouthard *et al.* 1997, p.73
Moderate retinopathy (BDR, non severe PDR)	0.17 (0.073-.278)	"[Diabetes mellitus with] moderate [vision disorders] (i.e., great difficulty reading small newspaper print, some difficulty recognizing faces at 4m. distance"	112121	Stouthard *et al.* 1997, p.75
Severe retinopathy	0.43 (0.339-0.521)	"[Diabetes mellitus with] severe [vision disorders] (i.e. unable to read small newspaper print, great difficulty or unable to recognize faces at 4m. distance)"	123121	Stouthard *et al.* 1997, p.75
Sores, ulcers and Lower extremity amputation	0.19 (0.128-0.255)*	"[Diabetes mellitus] with neuropathy"	111111 (75%), 222221 (20%), 222331 (5%)	Stouthard *et al.* 1997, p.73

*the global burden of disease study uses 0.3 for foot amputation and 0.102 for toe amputation (Murray and Lopez, 1996); there is no disability weight for amputation in the paper by Stouthard *et al.* (1997) which we used as the main source for weights in our study. The 0.19 weight for neuropathy in the Stouthard *et al.* paper is an average across different degree of severity and we use it both for sores/ulcers and amputations.

5   Portfolio decision analysis for population health

Abstract

In this chapter we discuss the application of Multi-Criteria Portfolio Decision Analysis in healthcare. The problem which we consider is that of allocating a limited budget to healthcare for a defined population. In this context, the healthcare planner needs to take into account the state of ill-health of the population, on one hand, and the costs and benefits of providing different healthcare interventions, on the other. To date, two techniques have been applied widely to combine these two perspectives. One of these techniques, Generalized Cost Effectiveness Analysis, relies on simulating the impact of a portfolio of interventions on the costs and health benefits for a given population. The other technique, Program Budgeting and Marginal Analysis, emphasises the need to include more than the 'health benefit' criterion to capture the objective function of the health planner and to engage local stakeholders to articulate their values and assessing interventions. We present a case study to illustrate how a simple, formal Multi-Criteria Portfolio Decision Analysis model can structure the engagement of local stakeholders in exploring the resource allocation problem explicitly. The case study also highlights current challenges for the research community around the use of disease models, capturing preferences relating to health inequalities, and handling unrelated future costs.

5.1 Background

Many public sector planners have responsibility for defined populations, and work in environments where key dimensions of performance are hard to measure, values are contested, and decisions have to be negotiated between stakeholders within and beyond the organization, and with the general population. This is particularly true of healthcare. Despite the existence of a vast medical evidence base, interpreting that evidence in the context of a particular population is not straightforward; tradeoffs between different sorts of treatments for different sorts of patients inevitably arise; and the professional status of healthcare workers and the intense public interest mean that making decisions unilaterally behind closed doors is not regarded as acceptable.

In this chapter we outline the challenges of applying Portfolio Decision Analysis to maintain and improve population health. In some healthcare systems, the scope of such planning will be limited to public health interventions, as primary and acute care will be delivered by organizations (such as insurers) which do not have responsibility for a defined population, but which compete for business in a market. In other systems, such as the English National Health Service, with which we are particularly familiar, almost all healthcare is commissioned (at time of writing) by geographically defined health authorities called "Primary Care Trusts".

To understand the background, healthcare planning is dominated by two communities of analytic professionals which represent two different perspectives on the meaning of 'healthcare need' which have been labeled 'humanitarian' and 'realist' (Acheson 1978). A difficulty in healthcare planning is integrating these two perspectives.

On one hand, public health analysts tend to take a 'humanitarian' perspective. In this perspective, the focus of analysis is the identification and measurement of existing suffering. The analysis usually takes the form of 'needs assessment', which is a snapshot of the health status of the population under investigation in terms of disease prevalence and mortality rates. Needs assessments is often very revealing: psychiatric morbidity surveys reveal very substantial untreated mental illness; and rising rates of obesity in the UK herald higher rates of diabetes, heart disease and so on in later life. At the same time, however, needs assessment is not enough by itself, as many conditions generate

substantial morbidity (for example chronic conditions) but little can practically be done about them.

On the other hand, health economists who take a 'realist' perspective, focus on what could be done to improve health and on whether the cost of doing so is affordable by focusing on the choice between particular interventions, such as surgical procedures or pharmaceuticals. Health economists assess the value and affordability of an intervention through 'incremental cost effectiveness analysis' (e.g. Williams 1985, Gold, Siegel et al. 1996, Drummond, Sculpher et al. 2005): each intervention is assessed compared to the next best alternative in terms of its additional costs, and the additional benefits it is expected to generate for the average patient.

Benefits are usually not expressed in monetary terms but using metrics such as the Quality Adjusted Life Year or "QALY" (Williams 1985). These metrics are calculated as the product of life duration expressed in years and a quality of life weight represented on a scale ranging from 0 to 1, where 0 corresponds to the quality of life equivalent to being dead and 1 to that of 'full health', respectively. Thus, the unit on the QALY metric, i.e. one QALY, represents *the equivalent of* a year spent in full health. In informing resource allocation decisions, health economists estimate the 'incremental cost-effectiveness ratio' of treating an additional patient, i.e. the ratio between the additional costs and the additional QALYs of an intervention compared to the next best alternative and recommend the funding of all interventions below some critical ratio. From a Portfolio Decision Analysis perspective the approach proposed by health economists has a normative basis, in that the critical ratio can be interpreted as the Lagrange multiplier associated with the budget constraint in some implied optimization problem. However, unless one knows the extent of disease in the population, one has no idea of the cost coefficients in the budget constraints, and thus what the critical ratio should be.

The remainder of the chapter is organized as follows. First, we review two techniques which have received particular attention in the literature, one which has emphasized the need for explicit Portfolio Decision Analysis and one which has focused on the need to include multiple criteria and to engage key stakeholders. Second, we present a case study to illustrate how Multi-Criteria Portfolio Decision Analysis, broadly

in the spirit of Phillips and Bana e Costa (2007), can further the development of techniques to support healthcare planners. Finally, we will offer some reflections on the particular challenges of doing Portfolio Decision Analysis in a population health context.

In Chapter 8 of this thesis I also reflect on the overall learning from the action research case studies presented in Chapters 5, 6 and 7.

5.2   Existing techniques

The challenge for the working health planner is to draw on available information about healthcare needs of the population and cost-effectiveness, and synthesize this information in order to establish a programme of activity which will give greatest value for the locality for which she is responsible. Various approaches have grown up for helping decision makers with this decision and two have received particular attention: Generalized Cost Effectiveness Analysis and Programme Budgeting and Marginal Analysis.

Generalized Cost Effectiveness Analysis (GCEA) is the tool which the World Health Organization recommends for planning population health (Hutubessy, Chisholm et al. 2003, Tan-Torres Edejer, Baltussen et al. 2003). Similarly to the cost-per-QALY tool of economists, interventions are assessed compared to an alternative in terms of the ratio of added costs and benefits. The alternative intervention is a counterfactual which usually corresponds to what would happen in the absence of the investigated intervention and costs and benefits are assessed explicitly through a simulation model. Benefits are measured by Disability Adjusted Life Year (DALY), which are a measure substantially similar to QALY, although with a negative sense: the DALY is a "bad" whereas the QALY is a good. Airoldi and Morton (2009) have shown that the way that the DALY is currently computed makes it problematic as a measure (for example, increases in length of lifetime may increase DALYs). On the other hand, Morton (forthcoming) has argued that a suitably corrected version of the DALY could have an

interpretation as a metric with similarities to the well-established poverty metric in the domain of income.

A further distinction between the canonical health economics approach, which stresses incremental cost-effectiveness, and the GCEA approach, is that in the latter benefits and costs are estimated at the population level rather than for the average patient and the alternative uses of limited resources are taken into account, making the objective function and the budget constraint explicit. In this respect GCEA stands as a direct descendant of the Global Burden of Disease studies pioneered by the World Health Organisation (Murray and Lopez 1996), which can in turn trace their lineage to the techniques of public health needs assessment.

Its proponents highlight that GCEA, although highly informative in its own right, needs to be integrated with further analysis to take into account other concerns of the health planner. In particular, the framework models the objective of maximizing population health explicitly within limited resources, but health planners will be interested in achieving other goals such as health equity and system responsiveness (Hutubessy, Chisholm et al. 2003).

A competitor approach, which aims at taking into account the multiple concerns of the health planner explicitly, is Program Budgeting and Marginal Analysis (PBMA). PBMA is an economics-inspired approach to public sector planning designed to aid local decision makers (Mitton and Donaldson 2004). PBMA uses the principles of opportunity cost and marginal analysis: the "opportunity cost" of providing an intervention is the value of the best available alternative use of the same resources; and "marginal analysis" consists in focusing on the additional costs and benefits associated with the proposed change in service provision (rather than the average costs and benefits of the resulting, overall portfolio of healthcare services).

PBMA covers a variety of different practices, with a similar process but different evaluation procedures. The process is led by a facilitator and consists in several steps: to determine the aim and scope of the analysis, to identify where resources are currently spent, to form a panel of decision makers, to determine locally relevant criteria for decision-making, to identify options for investment and disinvestment, to assess options

against the set criteria, to validate results and recommend resource re-allocation (Mitton, Patten et al. 2003).

The evaluation procedure recommended in PBMA is multi-criteria decision analysis, but the set of criteria and the form of the value function differ between case studies to reflect the preferences of the local stakeholders. In some cases, the composite concept of cost-effectiveness is used as a criterion for a multi-criteria value function (Mitton, Patten et al. 2003). In other cases, the cost-effectiveness of intervention is derived by calculating the ratio of the multi-criteria benefit score and the cost of each intervention (Wilson, Rees et al. 2006, Baughan and Ferguson 2008, Kemp and Fordham 2008, Robson, Bate et al. 2008). In some cases, the concept of need and that of effectiveness are entered as separate criteria (e.g. Wilson, Rees et al. 2006), in others, the improvement of the health of the local population, although the single most weighted criterion, accounts for about 30% of the scores given to each options (Robson, Bate et al. 2008).

PBMA practitioners recognize the need to provide more guidance on the criteria and the shape of the value function. Peacock, Richardson et al (2007), for instance, propose a Multi-Attribute Utility function with three criteria, and Wilson, Peacock et al. (2008) report and reflect on current practices for assessing the value of interventions and to arrive at a priority ordering. They do not discuss explicitly the link with the Multi-Criteria Portfolio Decision Analysis literature. Criteria used in selected articles are summarized in Table 1 on page 43.

Whilst GCEA highlighted the need to formalize the resource allocation problem, PBMA highlights the need to consider multiple criteria and to engage key stakeholders in the process. PBMA proponents envisage developing their techniques by drawing more systematically on models to assess the benefits and costs of interventions from the health economics and Multi-criteria Decision Analysis literatures (Mitton and Donaldson 2004).

5.3   Case Study

Over the last few years we have used Multi-criteria Portfolio Decision Analysis, broadly in the spirit of Phillips and Bana e Costa (2007) to support a number of local Primary Care Trusts (Primary Care Trusts) in England. Primary Care Trusts are organizations responsible for purchasing healthcare services on behalf of their local population. At the time of writing this chapter, there are 152 Primary Care Trusts in England with an average population of 330,000 people each.

The problem of selecting which services to purchase and their scale is a portfolio allocation problem. Primary Care Trusts purchase services from local providers such as hospitals, ambulance and community care services. The purchase of services is formalized in separate contracts between the Primary Care Trust and each healthcare provider, to plan the provision of the anticipated type and volume of care for the local population in the forthcoming year. These services are funded from general taxation and offered 'free of charge'; the available resources are exogenously determined according to the healthcare need of each Primary Care Trust using a formula which takes into account factors such as size of the population, its demographic characteristics and its socio-economic deprivation (Department of Health 2008). The available resources are typically insufficient to provide all services that would benefit the local population and Primary Care Trusts are responsible for deciding which interventions should be funded.

In this section we describe the use of Multi-criteria Portfolio Analysis to support the allocation of resources in a public health programme called 'Staying Healthy' in a Primary Care Trust in central London. The key aim of the programme is to prevent disease through disease prevention and the promotion of healthy lifestyles. We will start by explaining the problem faced by the Staying Healthy board and by formulating the underlying multi-criteria Portfolio Decision Analysis problem. We will then describe how we engaged local stakeholders in identifying options for resource allocation, and helped them to express their objectives operationally, to assess the options against the criteria and to interpret the results of the Portfolio Decision Analysis model.

5.3.1   Framing the problem

The Staying Healthy Board is responsible for a wide range of activity to reduce the prevalence of risk factors such as high blood pressure, obesity and smoking. The underlying logic of the programme is that by reducing the prevalence of risk factors in a

population, the incidence of diseases such as circulatory diseases or cancers can be reduced and hence the number of premature deaths and preventable ill health would be lower.

The information available to plan interventions in a 'Staying Healthy programme' is predominantly the current health state of the population in terms of risk factor prevalence, disease incidence, disease prevalence and associated mortality statistics. Assessing the value of interventions which promote healthy lifestyles and prevent diseases is more difficult than assessing the value of curative or palliative services because the causal chain between action and outcome is longer and more tenuous, and as a result, the evidence base is weaker.

We used Multi-Criteria Portfolio Decision Analysis in a series of interactive workshops or "decision conferences" (Phillips 2007) to facilitate the Staying Healthy Board in integrating the current information about current health state of the population with the expected benefit of potential interventions, with a focus on the prevention of cardiovascular diseases (CVD).

To present the decision model underpinning the workshops formally we use the following notation:

I: set of healthcare interventions, indexed by *i=1,...,n*

G: set of healthcare intervention groups, indexed by *g=1,…,q*

A: set of attributes or criteria, indexed by *a=1,…,m*

We use the notation "*i∈g*" to mean "intervention *i*" falls within group *g*.

The parameters of our model are as follows:

B: available budget

c_i: cost of intervention *i;*

s_a^i added value in terms of attribute a which would be generated by implementing intervention for all $i \bar{\in} g$. Scores were elicited by participants by attribute a, one group g at a time;

$v_i = \Sigma_a \, w_{ag} * s_a^i$ value of intervention for all $i \bar{\in} g$

$w_{within \, a}$: within criteria weight

$w_{a \, across \, g}$: across criteria weight

$w_{ag} = w_{within \, a} * w_{a \, across \, g}$: weight of attribute or criterion a for scaling interventions in group g

The decision variable is $x=(x_1,..., x_i,..., x_n)$ which is a vector of n elements $x_i \in [0,1]$ to represent the extent to which the proposed intervention could be funded, with 0 representing no funding and 1 representing full funding.

The implied optimization model is hence described by Equation 8:

Equation 8

Max $\Sigma_i \, v_i * x_i$;

subject to $\Sigma_i \, c_i * x_i \leq B$

In the rest of the section, we describe how members of the Staying Healthy Board and a group of stakeholders of the Primary Care Trust used this framework to inform priority setting by expressing their knowledge and value judgments in the model parameters and by reflecting on the model results.

5.3.2 Planning the workshops

The facilitators and two directors in the area of Public Health discussed the problem faced by the Staying Healthy Board and agreed to explore their investment priorities with a focus on reducing cerebrovascular diseases (CVD), that is circulatory diseases which could cause damage to the brain (e.g. stroke) or to the heart (e.g. coronary heart diseases). The facilitators described the process of Decision Conferencing, which is a set of working meetings led by an impartial facilitator to build a formal model of the problem and explore the solution space (Phillips and Bana e Costa 2007) and proposed to formalize it through a Multi-Criteria Portfolio Decision Analysis model.

The directors decided to engage members of the Staying Healthy Board and key stakeholders (including family doctors, health visitors and patient representatives) to populate the model during three half-day meetings at about ten days intervals. The participation to these workshops ranged from six to ten people.

In the first meeting participants framed the decision problem in terms their fundamental objectives and by generating a list of alternatives to achieve them (i.e. the set of criteria A; and the set of interventions I, grouped in thematic areas G), which we describe in Section 5.3.3. In the second meeting they scored each intervention against the set criteria, which included cost (i.e. parameters c_i and s_a^i), as we describe in Section 5.3.4. Finally, in the last meeting, participants assessed the trade-off across the different criteria to generate an overall benefit score for each intervention (i.e. the two sets of weights $w_{within\ a}$ and $w_{a\ across\ g}$), explored the results produced through a Multi-Criteria Portfolio Decision Analysis software[2] and engaged in a discussion to inform priority setting (Sections 5.3.5 and 5.3.6).

5.3.3 The strategic decision frame: objectives and alternatives

Following the *Value-focused thinking* framework (Keeney 1996), we engaged participants in defining the decision problem starting with the identification of their values and by articulating them in fundamental objectives. In keeping with Keeney, we distinguished 'fundamental objectives', i.e. the ends that participants valued in the

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[2] We used 'Equity', that is a software which was developed by the LSE and currently maintained by Catalyze Ltd (www.catalyze.co.uk).

context of allocating resources to health promotion and prevention activities, from 'mean objectives', i.e. the methods to achieve those ends.

Participants initially identified their objectives with the three overall aims articulated by the Board of the Primary Care Trust:

- to reduce the prevalence of key risk factors for premature mortality;
- to reduce premature mortality;
- to reduce inequalities in prevalence of risk factors and premature mortality.

For each objective, participants discussed the reasons for their importance, in order to distinguish mean objectives (e.g. reducing prevalence of risk factors) from end objectives (e.g. reducing premature mortality). The discussion led to the identification of additional end objectives and to their operational definition. The final list of end objectives was as follows:

- *Reducing Premature Mortality (a1):* The extent to which an intervention reduces premature CVD mortality in the medium-run (5-10 years) and the long-run (10+ years)
- *Improving Individual Quality of Life (a2):* The extent to which an intervention improves an individual's overall well-being (as defined by the individual).
- *Improving Social Quality of Life (a3):* The extent to which an intervention improves an individual's overall opportunities (in employment, education, etc) and their engagement in social life.
- *Reducing Health Inequalities (a4):* The extent to which an intervention reduces the unjustifiable and avoidable gaps in health status among different social groups in the local population

With these end objectives in mind, we invited participants to suggest well-defined interventions which the Staying Healthy Board should finance. An intervention was considered "well-defined" if, *in principle,* it could have been possible to answer the following questions:

1. How much does it cost to provide the intervention?
2. How many people benefit from it?
3. How exactly do people benefit from it?

Each participant worked individually and listed five to six interventions, writing each of his or her proposals on a different piece of paper. Participants revised the list of

interventions, eliminating duplicates and asking clarifications on the details of the interventions and then clustered them in homogenous areas. The clustering facilitated the assessment of the extent to which each intervention contributed to achieving the stated objectives, because clusters of interventions could be associated with a risk factors and the associated epidemiological and clinical literature on its effectiveness. The final list of interventions for prioritization consisted of twenty interventions grouped in the following six areas: Smoking, Physical activity, Blood pressure (pharmacological interventions), Statin (i.e. pharmacological interventions to tackle hypercholesterolemia), Diet and Alcohol. Due to time constraints, Diet and Alcohol were later excluded from the formal analysis. The list of the fourteen interventions included in the model is summarily reported in Figure 14 and Table 19. For each area, participants also defined a baseline of care (i.e. current care).

6		Physical environment +		
5	Tobacco control +	Workplace +		
4	Cessation pregnancy +	Level 3 +	Better monitoring +	
3	Cessation Lvl 2/3 +	Level 2 +	Prescribing +	Secondary prevention +
2	Brief intervention +	Brief intervention +	Better detection +	Primary prev - HR +
1	Do nothing +	Do nothing +	Do nothing +	Do nothing +
	Smoking	Physical activity	Blood pressure	Statin prescribing

Figure 14 The final model showing the interventions in each of the six areas

Table 19 Description of interventions (*i*) by group (*g*)

Area (g)	Intervention(i)	Description
Smoking	Do nothing	Current care with no additional effort by Primary Care Trust
	Brief	Brief interventions by range of practitioners (GPs, practice nurses, pharmacists, other clinicians) The intervention will benefit all registered people who smoke who would like to give up
	Cessation "Level 2 & 3"	Intervention in the community including Nicotine Replacement Therapy and counseling through smoking cessation clinics
	Pregnancy	Smoking cessation in pregnancy
	Tobacco control	Home and workplace interventions to promote smoking cessation (including stop smoking advice, having a smoke free environment, clamping down on illegal sales, stopping sales to children)
Physical activity	Do nothing	"G-Pack" currently provided, which includes assessment, advice and follow-up
	Brief	Opportunistic interventions in primary care and community services
	Level 2	Targeted intervention to about 1,000 beneficiaries at risk. The intervention consists in fostering motivation, goal-setting, follow-up, and coaching in general. It will be delivered by health trainers
	Level 3	Intensive 10-week programme targeted to about 500 beneficiaries post diagnosis (already commissioned)
	Workplace	Health promotion activities within workplace environment in a proactive manner.
	Physical environment	Influencing transport, urban planning, buildings, children (in practice this will involve hiring two persons to implement actions)
Blood pressure	Do nothing	Current care with no additional effort by Primary Care Trust
	Better Detection	Opportunistic screening (everybody visiting a GP), receiving current care in terms of monitoring and prescribing
	Prescribing	prescribing, following good practice for those currently detected
	Better Monitoring	better monitoring of those currently detected

Area (g)	Intervention (i)	Description
Statin	Do nothing	Current care with no additional effort by Primary Care Trust
	Primary prevention – High Risk patients	Primary prevention: Identifying people at high risk (without disease). Different strategies: - Random assessment. - Patients who are over 50, then patients who are over 40. - Prioritise patients by their age. - Prioritise patients by a prior estimate of cardiovascular (CVD) risk. - Reduced CVD risk (73 CVD events avoided annually in the PCT ~£176K saved). Based on NICE costing estimates: all people between 40-75 at increased CVD risk (20%) over 10 years; 16,800 (8,500 men and 8,300 women). Additional systematic assessment (3,230 people) £66K Additional primary prevention of CVD with drug therapy (7,000 adults) £273k (treatment includes statins, aspirin and antihypertensive therapy)
	Secondary prevention	1/Secondary Prevention: Treating people with disease. Different strategies: - Higher versus lower intensity treatment with statins - Titration strategy

5.3.4 Scoring

The scoring process aimed at eliciting parameters s_a^i in the Multi-Criteria Portfolio Decision Analysis model. These assessments were done through direct rating, one area g at a time and one criterion a at a time. The scores should reflect the 'added value' of the intervention in achieving the considered objective or criterion, where a score of 0 was assigned by default to the current care (interventions labeled 'do nothing' in each area). For example, the group first considered all the interventions in the Smoking area. We asked the group to identify the one intervention that provided the most impact on "Reducing health inequalities" in the local population. After some discussion, the group agreed it was "Tobacco control", so that intervention was assigned a preference value of 100. Next, the group compared each of the other interventions in the Smoking area to this one as a benchmark and judged the relative value each intervention would contribute to "Reducing health inequalities". Thus, when participants argued that "Cessation Level 2&3" would provide about 40 percent as much value as "Tobacco control", a score of 40 was assigned. In all cases, the ratios of the numbers reflected ratios of participants' relative strength of preference for the two interventions. The group arrived at these relative judgments through discussion and consulting available evidence. Consistency checks were particularly useful to revise the group's assessments. For example, if interventions A, B and C scored 100, 20 and 80, respectively, then participants were asked if B and C together created the same value for the criterion under investigation as project A alone. If not, then revisions were made to the scores until consistency was obtained. Thus, each benefit score gave the relative *added* value attributable to Reducing health inequality from commissioning that intervention.

The process was applied to the projects for all four benefit criteria. Participants were encouraged to assess scores representing the value associated uniquely with each criterion, thus avoiding double counting.

The assessment of the value of each intervention to reduce premature mortality drew from participants' knowledge of the clinical and epidemiological literature and the model enabled them to translate the knowledge for their concrete problem. Similarly, for some interventions participants could draw on the growing body of models which

estimate the impact of interventions in improving the quality of life of patients (measured in QALYs).

On the other hand, the assessment of the 'Reducing health inequality' criterion proved more difficult, as different participants seemed to attach very different interpretations to the word "inequality". The exercise highlighted how this concept, although of general concern both in the local and in the national health policy debate, it is not operationally clear.

In discussing the value of interventions, participants also clarified how the intervention should have been implemented in practice and were able to estimate its costs. The working definition for defining the cost of each intervention was "the cost of providing an intervention to a pre-specified population group (i.e. the ongoing cost, over and above the status quo)".

The overall available budget B was never explicitly defined, although the limitedness of resources was clearly a constraint for two reasons. Firstly, even though the Staying Healthy programme had a set budget constraint, the exercise only covered a subset of the activity they fund; secondly, the aim of the exercise was to explore a systematic assessment of options which could be used to formulate business cases for negotiating additional resources. The parameter B remained a variable which defined different funding constraint scenarios.

5.3.5   Weighting the criteria

Total benefits cannot be calculated until the units of benefit from one area to the next and one criterion to the next are equated. This was accomplished in three steps. First, the benefit scores on a given criterion for a particular area were added and normalized so that the resulting scale extends from 0, representing least preferred, to 100, representing most preferred. Take the scores on Reducing premature mortality for interventions in the area "Physical activity" as an example. The first column of numbers in Table 20, below, gives the scores for the Reduction in premature mortality. The second column gives the cumulative sum of the scores. The third column shows the normalization, which results in a *preference value scale*. For this scale, 100 represent

the total achievable reduction in premature mortality associated with all six projects, assuming they are successful.

Table 20   Normalizing scores on Reduction in premature mortality for the area "Physical activity"

Intervention	Assessed Scores	Cumulative Score	Normalized Scores
do nothing	0	0	0
brief	70	70	23
Level 2	50	120	39
Level 3	30	150	48
workplace	60	210	68
Physical environment	100	310	100

This normalization process was carried out on each of the benefit scales for every area.   Thus, input scores associated with different criteria were all converted to cumulative preference (or value) -scales.   However, the benefit scores for different criteria were always assessed relative to a different, arbitrary 100 for each scale.   Thus, the weights for these criteria have to be judged.

Two types of weights are required within this model.   One set compares the scales for a given criterion from one area to the next; these are known as *within-criterion weights*.   The other set of weights compares the benefit criteria to each other; these are called *across-criteria weights*.   These weights reflect the *trade-offs* in values between the areas and between the criteria.   When any normalized preference score is multiplied by these two weights, it can then be compared to any other doubly weighted preference score.

The group began the weighting process by considering "Reducing premature mortality" (*within-criteria weighing*).   Participants were asked, "If you only cared about Reducing premature mortality and you could implement all the interventions in one of the four areas (Smoking, Physical activity, Blood Pressure, Statin), which area would you choose?" The group suggested they would choose Smoking, so that scale was given a weight of 100.   Participants were then asked to judge what area they would choose next; the group agreed it was Blood pressure, which was judged to meet 75% as much

Reduction in premature mortality. Statin, followed with 55%, and finally Physical activity with 25%. The process was then repeated for the remaining criteria.

Figure 15 Within criteria weighting demonstrates the principle of swing weighting by comparing Physical activity with Smoking on the Reduction of premature mortality criterion. The swing in preference from doing nothing to doing all the projects listed is compared between the two areas. The Equity software normalized values from doing nothing to doing all the intervention on a 0-100 scale. For the criterion 'Reducing premature mortality', the group agreed that going from doing nothing to doing all the considered intervention in Staying Healthy was about a quarter as valuable as going from doing nothing to doing all the interventions in the Smoking area. Thus, the weights for the preference scales are in the ratio of 25 to 100.
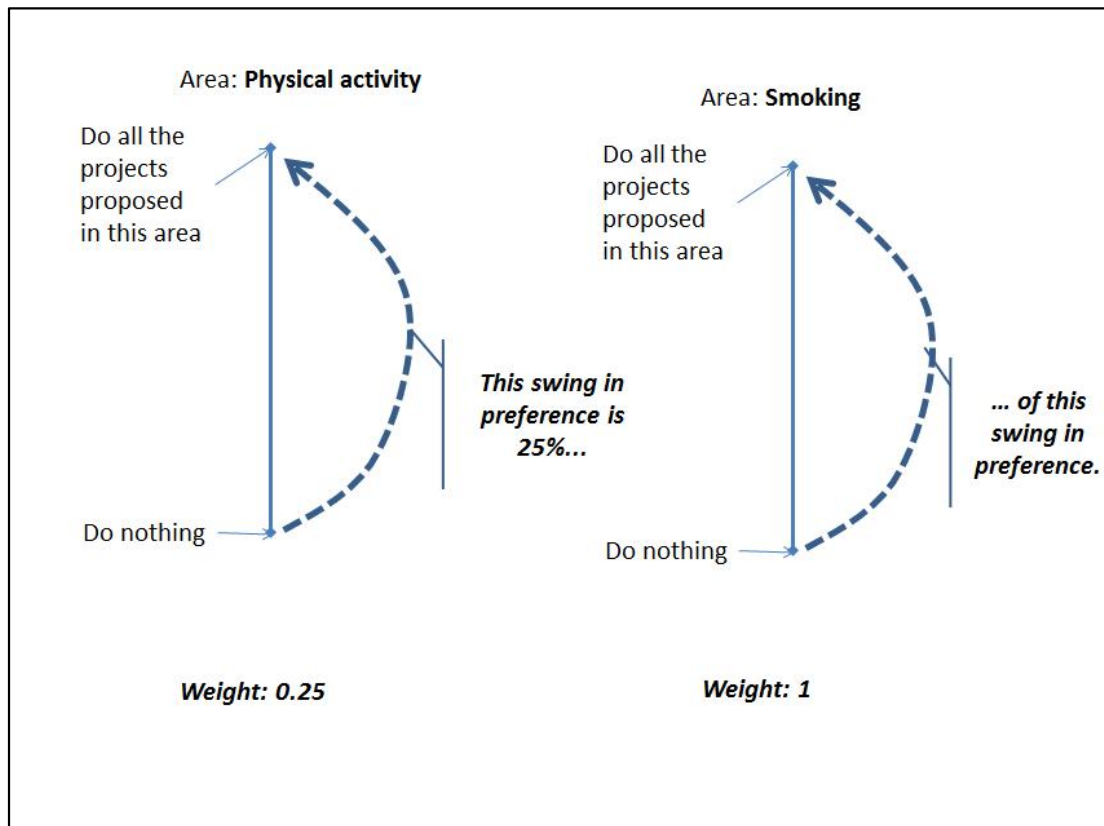

Figure 15 Within criteria weighting

The third and final step in weighting required a comparison of the *relative* importance of the benefit criteria themselves (*across-criteria weighting*). Participants were asked to compare the swing in preference for the scales given within-criterion weights of 100. The *within-criteria weights* resulting from the previous discussion are

reported in the first rows of Table 21, whilst the *across-criteria weights* appear in the bottom row. The group compared the weights of 100 in Reducing health inequality by doing all intervention in the Smoking area, with Reducing premature mortality by doing all the intervention in Smoking, with Improving individual quality of life by doing all interventions in Physical activity, with Improving social quality of life by doing all interventions in Physical activity (highlighted in bold font in the table). The group exhibited as strong a preference for the potential Reduction in health inequality or the potential Reduction in premature mortality by doing all the interventions in Smoking. They also felt as strong a preference for the potential Improvement in individual or that in social quality of life that would be achieved by doing all the interventions in the Physical activity area. However, they thought these were about half as valuable as the Reduction in health inequalities and premature mortality from the Smoking area. Thus, across criteria weights of 100 were assigned both to Reducing health inequalities and premature mortality; and across criteria weights of 50 were assigned to Improving quality of life both from the individual and the social perspective.

Table 21 Within- and Across-criterion weights

		Cost ongoing	Health Inequality	Premature mortality	Individual QoL	Social QoL
Within-criteria weights	Smoking	-	**100**	**100**	90	20
	Physical activity	-	50	25	**100**	**100**
	Blood pressure	-	70	75	50	15
	statin	-	10	55	20	2
	diet	-	0	0	0	0
	alcohol	-	0	0	0	0
Across-criteria Weights:		100	100	100	50	50

5.3.6   Results

The weighting procedure illustrated above allowed the group to arrive at an overall aggregated value of intervention in terms of its expected contribution to reduce mortality with its expected contribution to reduce health inequalities and its expected contribution in improving quality of life. We then divided the benefit score by the estimated costs of implementing the intervention to assess its value-for-money. We can think of each of the 14 assessed interventions in terms of a triangle that has the benefit score in the vertical side and the cost necessary to generate those benefits on the horizontal side. Thus, the slope of the hypotenuse can be used to signal value-for-money: the steeper the slope, the better the value-for-money. Some writers have criticized the use of the value-for-money ordering (Kleinmuntz 2007) as it is not an optimizing algorithm for the problem represented by Equation 1 However, we find that it captures simply and directly the critical insight that projects which deliver high benefit are not a good buy if they simultaneously cost a great deal of money, and allows non-technical people to understand and "own" the results of the model.

To illustrate the impact of the approach, in this section we first report a graph with the value-for-money triangles for each area separately; then we combine all triangles in a single graph to facilitate a comparison of the value contributed by intervention in different areas.

An examination of the ordered benefit-cost curve for each area is instructive, for the curves often give an overall view of the areas that is not obvious by looking at the individual interventions. This curve is a partial efficient frontier, with interventions ordered by their efficiency score, which is represented by the steepness of the hypotenuse of the associated Value-for-Money triangle. The illustrative example of the partial efficient frontier for the 'Smoking' area is reported in Figure 16.
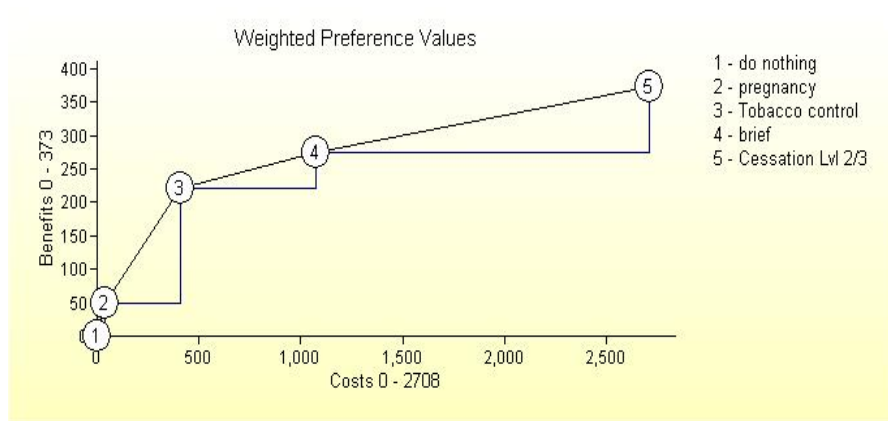
Figure 16 The partial efficient frontier for the 'Smoking' area

The partial efficient frontier in Figure 16 shows that targeted interventions to smoking pregnant women (i.e. "pregnancy") have a very good value-for-money. The intervention is a small scale intervention (hence the small triangle almost completely hidden by the circles with number '1' and '2') which seems to offer good benefits in terms of the criteria at a relatively low cost. If we look back at the group assessments (see Appendix), although the intervention has very small benefit in Reducing premature mortality, it has a relatively large impact on Improving the quality of life from the individual's perspective and some impact on Reducing health inequality for a very small cost (£40,000) compared to other interventions in this area. The next intervention in terms of Value-for-Money is "Tobacco control" (i.e. the triangle between point '2' and '3' in the graph), which contributes a large benefit for a relatively low cost. In fact, according to the group's judgments, "Tobacco control" contributes the greatest benefit in smoking. This can be seen in the graph by observing that the vertical side of the associated triangle is the longest vertical side of all the four triangles in this graph.

The model combined all the interventions into one curve of cumulative benefit versus cumulative cost. This is shown in Figure 17. The shaded (green) area represents the location of all possible combinations of interventions (360 possible portfolios).

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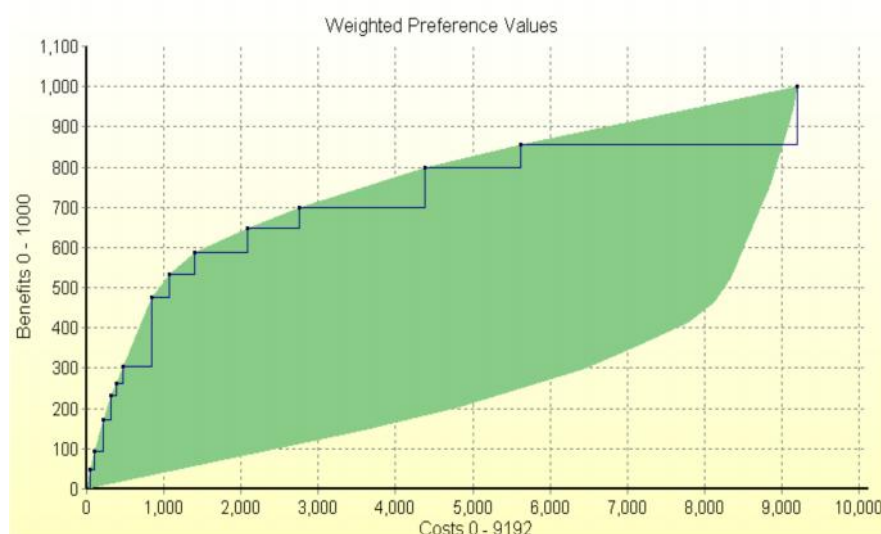
Figure 17 The efficient frontier, showing the 14 interventions in Value-for-Money order

5.4   Discussion

The systematic process we used is similar to that of PBMA and the feedback from the director of Public Health who promoted the exercise confirms findings of PBMA practitioners (Kemp, Fordham et al. 2008): the process enabled to quantify the relative Value-for-Money of potential interventions using the principles of marginal analysis and opportunity cost; the assessment was possible even though the available information was incomplete; the process also enabled a structured discussion between key stakeholders, both with clinical and managerial backgrounds and perspectives.

The underlying model used, however, drew more systematically on the normative basis of Portfolio Decision Analysis by making the objective function and the nature of the budgetary constraint explicit, which is a feature emphasized by the technique of GCEA advocated by the World Health Organization. In addition to GCEA, however, we included multiple criteria explicitly using Multi-Criteria Decision Analysis techniques and engaged local stakeholders to articulate their mental model, to contribute their specialist knowledge and to confront key trade-offs openly.

Though based on a technically simple model, these decision conferences and the models developed in them have been well-received by the sponsoring organizations, who told us they have materially influenced spending decisions. In this sense, our

experience has been positive and our methods have been "successful". However, it is difficult to test the prescriptive validity of the approach. Indeed it is not possible to verify the material impact of the approach on decisions because there is no counterfactual, i.e. it is not possible to know what would have happened anyway. Similarly, it is difficult to verify that the commissioning process improved without a more formal assessment. In chapter 7 I built a more formal evaluation of the proposed approach with an independent evaluator who attended the events and interviewed participants before and after the decision conference.

The limited scale of the intervention described in the case study and the sheer complexity of healthcare means that many issues have been left unexplored, which we briefly review here.

5.4.1   Use of evidence and disease modeling

Our intervention described above uses direct assessment of health benefits, drawing on expert knowledge, informed by the clinical literature. It should be emphasized that when making judgments about the extent of a health benefit, despite the availability of clinical studies and meta-analyses, it is not generally possible to "read off" the health benefits from a clinical trial, as local populations may have particular characteristics which differ from the populations of the clinical trials (for example, subjects in clinical trials are normally more healthy than the typical patient).

One approach to achieving this local contextualization is to use or develop formal disease models. Such models do exist for most common conditions, and are often of considerable sophistication.   In these models, which use Markov chains, System Dynamics, or Discrete Event Simulation, a cohort of patients flows through a system of disease states over a period of time, under differing scenarios and treatment programs. Such models are themselves based on judgment, but judgments at a more disaggregated level; and some of the disaggregated judgments at least can be directly validated. We have developed such models ourselves (e.g. Airoldi, Bevan et al. 2008), but to do so is highly time-consuming.   In a time and resource-limited environment where one is charged with making assessments on multiple interventions for different conditions, building a disease model for each is simply not practical.

How problematic this reliance on direct expert judgment is depends on the quality of that judgment relative to the quality of projections of a disease model, and ultimately there is a cost-benefit tradeoff to be made about whether the improvement in the quality of the assessment of an outcome that comes from a disease model is worth the additional investment. We do not have a particularly good sense of how far this is the case. Certainly, initial assessments by workshop participants of the scale of the benefits of particular programs could vary massively. However, this could be viewed either as a cause for concern or as a healthy acknowledgement of uncertainty on matters which are the subject of continuing scientific dispute. More information or evidence on this point would be very useful.

5.4.2   Health inequalities

One of the fundamental findings of public health is there is, globally, a strong and persistent "socio-economic gradient" in health, whereby the lower socio-economic classes experience worse health, on a variety of dimensions, and overall, as measured on metrics such as Life Expectancy and Disability-Free Life Expectancy. In the UK, and in particular in England, the recently-deposed Labour government took strong but ultimately ineffectual action to reduce this gap, by allocating additional funds to so-called "Spearhead" Primary Care Trusts with more deprived population. A persistent suspicion is that much of these funds did not reach the more deprived populations for which they were intended, and that the beneficiaries of this expenditure were the more well-off people who happened to live in the more deprived areas. We are not aware of compelling evidence for this view, but it is consistent with what we know about healthcare consumption – that the vocal middle classes access and consume more healthcare than the remainder of the population despite their generally better health.

The difficulty of tracing the ultimate beneficiaries of these funds illustrates that most Primary Care Trusts – whatever they may or may not have done – did not have a transparent system for deciding how resources should be allocated across different subgroups within their population. The experience with the Primary Care Trust described above illustrates how difficult building such a system is: "inequality" evoked very different things for different people, and sometimes for the same people at different times. Thus, the inequality criterion in our model represented for the group, a composite of (at least) socio-economic, race, and gender inequality.

In ongoing work (Morton and Airoldi 2010), we are attempting to develop a clearer and more transparent framework for assessing values of healthcare interventions taking into account aversion to inequality, with a view to informing decisions about, the targeting of screening programmes on particular subpopulations. A difficulty in designing such an approach is that while people often feel strongly that health inequalities are unjust and should be tackled, they simultaneously reject any idea that one person's need for healthcare be weighted more heavily than another person's need for healthcare. It is not at all obvious how such conflicting moral intuitions are to be reconciled.

5.4.3   Unrelated future costs

An issue which surfaces from time to time in the health economics literature is the role of so-called "unrelated" future costs (Garber, Weinstein et al. 1996). These are the healthcare costs which accrue as a result of (for example) saving the life of someone who then goes on to incur further healthcare expenditures, which had he died, would have been avoided. The most prominent example of this issue is in the context of smoking: lung cancer is a quick and cheap way to die as there are no real effective treatments, and as a result, those who die of the disease save the public purse considerable sums of money, from long term care late in life, pensions and so on. Preventing such a death does incur costs to the system further down the line.

From the point of view of a Primary Care Trust these costs do not loom large, and we do not take them into account in our modeling. In a way this makes sense: Primary Care Trusts have responsibility for an annual budget which is set by the Department of Health, and this budget is determined based on the morbidity of their population. Thus, from the point of view of the individual Primary Care Trust, if they manage to keep more sick people alive, they will receive more funds (assuming the budget is exogenously determined). From the point of view of the system as a whole, however, this looks to us like a bias which promotes longer sicker lives at the expense of shorter healthier ones, by underestimating the cost of the former.

This offends our economic and moral sensibilities, and raises the concern that we are contributing to a situation where an increasing number of increasingly ill people consume an increasing amount of healthcare. How to introduce these so-called

"unrelated" future costs into models suitable for use by Primary Care Trust decision makers is a problem to which we do not yet have a solution.

5.4.4   Acute versus preventive

A feature of the intervention described in this chapter is that the decision makers were interested in considering activities which could ameliorate a range of different diseases. This is in contrast to the sort of situation where economic evaluation is most frequently used in healthcare. Often structured appraisal methods are applied in decision contexts where the choice is between different treatments for the same or similar conditions. However, restricting the use of analysis in this way means that the large scale context decisions are often made on the basis of unanalyzed intuitions and gut-feels. The challenge then, is to develop methods and procedures which can help decision makers compare treatments which may be very heterogeneous in terms of the disease which has been targeted and in terms of the characters of the beneficiaries – consider for example comparing hip replacement and gender reassignment surgery.

One of the biggest distinctions between different sources of treatments which often loom large in a practical appraisal context is the distinction between acute and preventive interventions. Acute interventions are typically developed in the hospital setting – for example surgical procedures such as coronary heart bypass surgery. Preventive interventions, on the other hand, may take the form of public information campaigns or the provision of services to help people in the community take better care of their health – such as services which help people to give up smoking. Acute and preventive interventions differ both in terms of the nature of the knowledge base underlying claims on the effectiveness. The effectiveness of acute interventions is typically studied by randomized controlled trials; while the evidence of effectiveness of preventative interventions (for example smoking cessation services) is of a different nature and considered to be weaker. Another and possibly more important difference between acute and preventative interventions, however, is that because the beneficiaries of acute interventions are named individuals whereas the beneficiaries of preventative interventions are statistical individuals, there is a natural constituency to advocate for the greater uptake of acute interventions.

An example which highlights the issues involved is that of national policy on stroke. According to modelling studies which we worked on in a separate but related piece of work (Airoldi, Bevan et al. 2008), preventive interventions for stroke offer excellent opportunities for improving population health at a moderate cost, whereas acute interventions such as stroke clinics and thrombolysis, although they may substantially reduce the disability associated with stroke for those unfortunate enough to experience it, nevertheless cannot compare in quantitative terms to the effectiveness of the preventative interventions. Yet national policy continues to stress the importance of implementing stroke clinics and thrombolysis to the almost complete exclusion of preventative interventions. Accordingly, we see a challenge for portfolio decision analysis in the healthcare arena as being to provide frameworks for decision makers to reflect on the appropriate balance between acute and preventive parts of their portfolios in the light of both the clinical evidence and their own value judgments.

5.4.5   The good death

In the intervention described in this chapter we used an evaluation scheme which depended heavily on the concept of 'health'. Some interventions, however, have no or negligible impact on health but rather are primarily intended to ease the process of dying (palliative care being the most obvious example). Comparing interventions which improve health with interventions designed to improve the quality of death seems to be something which people find difficult conceptually. Part of the reason for this may be that there is lack of empirical evidence (and a lack of standardised evaluation schemes) on what constitutes a 'good death' and such evidence as there is suggests that tastes may differ substantially within the population (for example while most people would prefer a painless death, some may prefer a death which is sudden whereas other may prefer to have a warning time long enough to set their affairs in order and say goodbye). Moreover, we suspect that people's preferences over different sorts of deaths are likely to be relatively labile, as the issue is one which most people are probably not given to thinking about deeply and frequently (different forms of bad health, on the other hand, are relatively familiar and someone who has experienced both a broken arm and a migraine can relatively easily say which is the more unpleasant experience). There is a need for decision analysis techniques to be developed which can compare life improving and death improving interventions.

5.5 Conclusion

In this chapter we have outlined the challenge faced by the healthcare planner, who needs to combine information about the current health status of a defined population with that on the costs and the effectiveness of possible interventions to improve the level of health and its distribution. Existing techniques to face this challenge tend to focus either on the process which the healthcare planner could follow to confront key tradeoffs (PBMA) or on the explicit modeling of the underlying disease to estimate the intervention impact on the local health economy (GCEA).

In the case study which we presented, we used the socio-technical approach of Decision Conferencing (Phillips and Bana e Costa 2007) to engage a group of key stakeholders in a Primary Care Trust in England and build a model for their resource allocation problem based on Multi-Criteria Portfolio Decision Analysis, learning from and building on both these traditions. Our engagement with the National Health Service continues, and we are refining our methods to deal with some of the challenges outlined in the previous section.

The approach that we used could be described as low-tech and participative, in the sense that the methods which we used are not fundamentally technically innovative. Rather, we have used our case study to draw attention to some of the deep and complex issues which beset decision making in this area – ambiguous or incomplete evidence, aversion to inequality, and costs associated with the prolongation of life. When the issues are so complex, theoretic development is not an optional extra: using population health metrics such as the DALY as a basis for prioritisation (Airoldi and Morton, 2009) or weighting health states to model inequality aversion (Østerdal, 2003) without proper understanding of what one is doing can lead to very odd results.

Yet theory by itself is not enough. Despite the long history of Operations Research in healthcare, and of the vast database of medical, epidemiological, and health economic evidence at their disposal, we find that Primary Care Trusts make limited use of structured techniques which could help them think systematically and quantitatively about the big questions of what they get for the money they spend. This is a

disheartening state of affairs, particularly considering the stresses which healthcare systems face in coming years. But it is also a reminder of the importance of usability and accessibility to decision makers operating in a challenging environment. Decision Analysts – with their respect for theory and their preoccupation with producing tools which actually work – are perhaps uniquely well-placed to play a role in pushing forward practice in this important and fascinating area.

5.6   Appendix

In this appendix I report the intervention details and their scores.

Smoking

Options

Name	Description
do nothing	
Tobacco control	Home and workplace interventions to promote smoking cessation (inc stop smoking advice, having a smoke free environment, clamping down on illegal sales, stopping sales to children)
Cessation Lvl 2/3	
brief	Brief interventions by range of practitioners (GPs, practice nurses, pharmacists, other clinicians) All registered people who smoke who would like to give up
pregnancy	Smoking cessation in pregnancy

Incremental Scores

Name	Costs		Benefits			
	Cost ongoing		Health Inequality	Premature mortality	Individual QoL	Social QoL
do nothing	0		0	0	0	0
pregnancy	40		20	5	100	5
Tobacco control	370		100	45	50	45
brief	666		10	25	20	25
Cessation Lvl 2/3	1632		40	25	100	25

Physical activity

Options

Name	Description
do nothing	G-Pack includes assessment and advice + follow up
Physical environment	Influencing transport, urban planning, buildings, children (hiring 2 persons)
Level 2	(1000) For those at risk (motivation, gola-setting, follow-up, coaching) Delivered by health trainers
workplace	Health promotion Internal Proactive
Level 3	(500) Intensive 10-week programme post diagnosis (commissioned) targeted intervention
brief	Opportunistic interventions in primary care and CS

Incremental Scores

	Costs		Benefits			
Name	Cost ongoing		Health Inequality	Premature mortality	Individual QoL	Social QoL
do nothing	0		0	0	0	0
workplace	60		50	60	30	60
Physical environment	120		100	100	50	100
Level 2	100		50	50	90	80
brief	65		10	70	15	70
Level 3	90		20	30	100	30

BP

Options

Name	Description
do nothing	
Better Detection	Opportunistic screening (everybody visiting a GP), receiving current care in terms of monitoring and prescribing
Drugs	prescribing, following good practice for those currently detected
Better Monitoring	better monitoring of those currently detected

Incremental Scores

	Costs		Benefits			
Name	Cost ongoing		Health Inequality	Premature mortality	Individual QoL	Social QoL
do nothing	0		0	0	0	0
Drugs	220		40	30	100	30
Better Monitoring	680		40	30	100	30
Better Detection	3570		100	100	85	100

Statin

Options

Name	Description
do nothing	
secondary prev	1/Secondary Prevention: Treating people with disease Different strategies: - Higher versus lower intensity treatment with statins - Titration strategy
primary prev-HR	Primary prevention: Identifying people at high risk (without disease) Different strategies: - Random assessment. - Patients who are over 50, then patients who are over 40. - Prioritise patients by their age. - Prioritise patients by a prior estimate of CVD risk. Reduced CVD risk (73 CVD events avoided annually in Lambeth ~£176K saved). Based on NICE costings All people between 40-75 at increased CVD risk (20%) over 10 years 16,800 (8500 men and 8300 women) (NICE costing methods) Additional systematic assessment (3230 people) £66K Additional primary prevention of CVD with drug therapy (7000 adults) £273k (treatment includes statins, asprin and antihypertensive therapy) - NICE costing methods

Incremental Scores

	Costs		Benefits			
Name	Cost ongoing		Health Inequality	Premature mortality	Individual QoL	Social QoL
do nothing	0		0	0	0	0
primary prev-HR	339		20	100	70	100
secondary prev	1240		100	80	100	80

6 Deliberative Cost Effectiveness Analysis to allocate a fixed budget

Abstract

The aim of cost effectiveness analysis (CEA) is to inform the allocation of scarce resources. Whilst CEA is routinely used in assessing the cost-effectiveness of specific health technologies by agencies such as the National Institute for Health and Clinical Excellence (NICE) in the England and Wales, there is extensive evidence that the CEA framework is not generally used by healthcare planners to allocate a fixed budget to a portfolio of interventions. CEA in its current form is problematic to use because the analyses are difficult to understand and it embeds unacceptable assumptions. Furthermore, it is not clear how interventions for which no published cost-effectiveness evidence is available should be considered in the resource allocation process. This paper argues for, and tests the feasibility of, a deliberative approach to CEA. The key characteristics of the approach are (i) the use of models of requisite detail to assess the cost-effectiveness of all interventions considered for resource reallocation drawing explicitly on health economic theory, and on epidemiological and clinical evidence; (ii) the engagement of key stakeholders in the interactive development of the models and interpretation of results.

6.1 Introduction

A central problem of healthcare systems funded through taxation or social insurance is to define the package of services to offer, given a limited budget. The economic evaluation techniques of Cost-Effectiveness or Cost-Utility Analysis (CEA or CUA) have been proposed as the tools of choice to solve this problem (Gold, Siegel et al. 1996, Drummond, Sculpher et al. 2005), and metrics such as the Quality Adjusted Life Year

(QALY)(Williams 1985) have been developed to assess the effectiveness of, or utility generated by, healthcare interventions.

There is a growing literature on the cost-effectiveness of healthcare interventions and bodies such as the National Institute for Health and Clinical Excellence (NICE) in England and Wales use CEA systematically to recommend whether a specific drug or procedure should be provided by the National Health Service (NHS).

There is also extensive evidence, however, that neither CEA principles nor published cost-effectiveness evidence are systematically used to allocate healthcare budgets to portfolios of interventions by local or regional health planners (Eddama and Coast 2008). CEA principles are difficult to use systematically because of the lack of cost-effectiveness evidence for all interventions considered for funding and the lack of time (and resources) to commission new CEA. This evidence shows that published cost-effectiveness studies are difficult to use because of "accessibility" and "acceptability" barriers, i.e. the ability to understand the details, and accept the assumptions, of the analyses, a characterisation borrowed from Bryan and colleagues (Bryan, Williams et al. 2007, Williams and Bryan 2007). Accessibility is compromised because of the difficulties of interpreting the results of CEA due to the need for specialist health economic skills, lack of access to data used in the analysis and an excessively technical presentation of results, making it difficult for non health economists to appreciate fully their meaning and robustness. The acceptability of CEA is limited because of institutional and political factors (e.g. the inflexibility of the healthcare budgets and the constraints of following national policies); ethical reasons (e.g. the focus on health maximisation and efficiency); concerns over the choice of the threshold cost-effectiveness value, over which interventions are deemed cost-ineffective and should not be funded. Evidence from the UK suggests that the range of threshold values used by NICE may be too high and that potentially highly cost-effective interventions for which no CEA is available may be displaced in order to fund others of lower but documented cost-effectiveness (Martin, Rice et al. 2008, Appleby, Devlin et al. 2009).

One approach that has been proposed and used to inform healthcare planning in practice is Program Budgeting and Marginal Analysis (PBMA) (Mooney 1978, Madden, Hussey et al. 1995, Mitton and Donaldson 2001, Mitton, Patten et al. 2003, Mitton and

Donaldson 2004, Peacock, Richardson et al. 2007, Peacock, Mitton et al. 2009). In PBMA a structured, deliberative approach is used to engage local stakeholders in considering current spend, and proposing a 'wish list' of new interventions and a 'hit list' of potential disinvestments from current activity to fund the new proposals. The value of considered interventions is generally assessed against all the criteria considered relevant by the local stakeholders using Multi-Criteria Decision Analysis (MCDA) (Keeney and Raiffa 1976) and participants are invited to assess the impact of changes to healthcare provision, that is the difference in benefits between funding the interventions on the wish list or on the hit list.

It is not clear, however, how the criteria proposed within PBMA relate to the normative principles of health economics, nor it is clear how epidemiological and clinical evidence can be integrated into the modelling in a consistent way. Furthermore, PBMA exercises often include a long list of criteria which may make the results of the analysis inaccessible (Mullen 2004) or may fail to meet the normative requirements of MCDA (Thokala 2011).

This paper contributes to the debate on overcoming barriers to the use of CEA, through an action research case study that describes the development and application of a deliberative approach to CEA. The key characteristics of the approach are (i) the use of models of requisite detail to assess the cost-effectiveness of all interventions considered for resource reallocation which draw explicitly on health economic theory, and on epidemiological and clinical evidence; (ii) the engagement of key stakeholders in the interactive development of the models and interpretation of results. Section two describes the research methods. Section three presents the case study in terms of context, terms of reference, the deliberative approach (formal analysis, communication procedure and interactive elicitation methods) and results. Section four discusses the strengths and limitation of the approach on overcoming barriers to the use of CEA; section five provides concluding remarks.

6.2   Methods

This paper employs action research. The term *action research* covers a multitude of activities and methods; their common feature is the participative engagement of the subjects of the analysis in the research, the research objective of analysing the world and trying to change it at the same time (Eden and Huxham 1996).

The research was conducted in collaboration with and for the Isle of Wight Primary Care Trust (PCT) of the English NHS in 2008. PCTs were responsible for designing contracts with providers defining the type and volume of activity they expect to purchase to meet the health needs of the local population of about 330,000 people on average. As the local planning and purchasing agency of the NHS, PCTs were funded through general taxation distributed by a capitation formula (Department of Health 2008).

The approach was organised around evaluation workshops with stakeholders which took the form of 'decision conferences'. Decision conferencing (DC), like PBMA, is a deliberative process. An impartial facilitator works iteratively with key stakeholders to generate a formal, 'requisite' model to assess options on multiple objectives using Multicriteria Decision Analysis (MCDA) and generate a summary benefit score (Phillips and Bana e Costa 2007). A model is 'requisite' when it is sufficient to represent the mental models, beliefs about uncertainty and preferences of the participants and additional model refinements do not generate new insights in the problem (Phillips 1984).

The analysis is based on extensive field notes, which include: the chronological development of the stakeholder engagement process and of the prioritisation technique, comments and reflections on these developments of the Strategic Planning group, semi-structured and unstructured interviews with clinical staff and PCT managers, email correspondence with PCT staff, direct observation of workshops, flipcharts produced by workshop participants, clarification questions and comments received on the report summarising the results of the analysis as well as follow-up interviews with participants.

In Chapter 8 of this thesis I also reflect on the overall learning from the action research case studies presented in Chapters 5, 6 and 7.

6.3 Case study

6.3.1 Organisational context and term of reference

The Isle of Wight NHS PCT was responsible for healthcare for an Island off the South-East coast of England with a population of about 140,000. The PCT was comparatively small and, in contrast to elsewhere in England, it was organised as an integrated healthcare system with both purchasing and provision responsibilities, but with governance arrangements to ensure separation of responsibilities.

The analysis of local mortality and disease morbidity conducted by the director of Public Health (Smith 2008) highlighted five key priority areas to focus on to reduce mortality and improve quality of life: cardiovascular disease, cancer, respiratory condition, mental and children health. The financial accounts highlighted a surplus of about £1m that the PCT could allocate on a recurrent basis from 2008 (Isle of Wight NHS PCT 2008).

The PCT, which had a duty to engage local stakeholders, used decision conferencing to involve stakeholders in the five identified priority areas to generate a robust plan for allocating the additional £1m. The PCT Board recommended looking at costs and using three criteria to identify value: to increase health, to reduce health inequalities, and to be operationally and politically feasible.

6.3.2 Deliberative CEA

The action research project ran from April to November 2008 and consisted of (i) a schedule of meetings (two initial meetings in the spring and then fortnightly from June); (ii) the design, in collaboration with the PCT, of a social process to engage key stakeholders (including managers, clinicians, patients and public representatives) and of a technical process to assess the relative cost-effectiveness of all interventions considered for funding; (iii) the guidance on extracting information from available demographic and epidemiological data to support the evaluation of different interventions; (iv) the facilitation of meetings with stakeholders; (v) the analysis of

results; (vi) the production of a report to document the process and to identify recommendations from the analysis; and (vii) follow-up assistance in performing supplementary analyses.

Executive level leadership was provided through a Strategic Planning group, which consisted of all eight executive directors (including Jenifer Smith) and the facilitator of the decision conferences (Mara Airoldi). Its remit was to design an engagement process, choose a prioritisation technique and put forward recommendations to invest available, additional resources.

The agreed engagement process consisted of two types of events. The first type was a two-hour meeting for each of the five priority areas to identify key issues in the provision of healthcare and to put forward a list of initiatives to improve quality of life and reduce health inequalities. A mix of stakeholders was invited, chosen by the commissioning managers to represent the diverse perspectives which they wished to consider in allocating resources and included: acute and community care clinicians, council representatives, voluntary sector representatives, nurses, public and patients' representatives, managers of the hospital and the ambulance service. The number of participants varied between 10 and 30 (a total of about 100 people were involved in total). The second type of event was a one-day decision conference to prioritise the proposed initiatives and to put forward recommendations to allocate resources across different priority areas. Twenty-five stakeholders attended the event: the eight executive directors of the PCT, nine commissioning managers, three patients and public representatives, four clinical experts and one representative of social services.

The area specific workshops identified twenty-one initiatives to be prioritized. Their total cost was over £5m. The proposed initiatives were described on a standard template reporting available information on: the expected costs, the estimated number of people who would benefit, a description of the 'average' beneficiary (in terms of demographics, severity of the condition, socio-economic background) and a quantitative or qualitative description of the health benefits to patients, their families and carers.

In the decision conference, participants built a formal model of the costs and value of all twenty-one strategic interventions (indexed by j) interactively, in terms of:

- *costs (c_j):* the additional annual funding (over and above the status quo) required in 2009 and 2010 to set-up and to run the intervention, in £'000. Set-up costs included training and equipment, and the running costs included staffing;

- *population health benefit (N_j*B_j):* the product of the number *(N_j)* of patients who benefit from the intervention and the potential benefit *(B_j)* in quality (and length) of life, assuming successful implementation, to the 'typical' beneficiary (e.g. QALY gains);

- *health inequalities (I_j):* the extent to which the intervention has the potential for reducing both differences in access and differences in health outcomes (across geographical areas, between men and women, of special groups);

- *feasibility (p_j):* Probability of success (from 0% to 100%) of achieving the assessed benefits, assuming funding is granted and taking into account: ease of implementation; availability of workforce; acceptability to stakeholders (e.g. willingness to make this change happen); process complexity (e.g. number of steps required). This criterion captures the concept of 'operationally and politically feasible' the Board asked the Strategic Planning group to consider in its terms of reference.

The formal model underpinning the evaluation is to Max Σ_j $E(v_j) * x_j$, where $E()$ indicates an expected value calculation, v_j is the benefit from intervention *j* (details of its calculation will be provided later), and x_j is an index variable with value 1 in case intervention *j* is funded, and value 0 in case it is not. In the model used during the workshop, we made a simplifying assumption about $E()$, namely we assumed that the intervention *j* would have been successful and deliver its benefits in full with probability p_j%; if unsuccessful ($1-p_j$% probability), it would have delivered no benefit. This assumption was subjected to sensitivity analysis after the stakeholder event through a parameter $k \in [0,1]$, which represented the proportion of benefits which would have been achieved in case of unsuccessful interventions. The formal model and its notation were hidden from workshop participants, who were presented with simpler, accessible visual aids for each step of the process, which will be described below. The budget constraint was not modeled explicitly because the PCT had some flexibility on allocating

resources in the current and the subsequent year. The aim of the technical model was hence to generate a priority list of the twenty one interventions in terms of their cost-effectiveness and to agree the exact amount of available budget after the analysis.

Participants revised the information provided by the standard template which put forward each initiative and scored them one commissioning group at a time (*g*, which corresponded to the priority area specific stakeholders' workshops), one criterion at a time as illustrated in Table 22 which reports the assessment for the three proposed interventions by the commissioning lead for cancer services, who also commissioned all palliative care. This required:

- Validating the number *N* of people who benefit (using demographic and epidemiological statistics, data on hospital admissions and expert judgment).

- Providing a description of the 'average' beneficiary of the proposed intervention and agreeing a qualitative description of the expected benefit (derived from clinical evidence of effectiveness and expert judgments).

- Quantifying the health benefits *B* to beneficiaries attributable to action within the budget period (over the beneficiaries' lifetime, assuming successful implementation and compliance). This assessment was informed by evidence (e.g. of QALY gains) whenever available. Due to time constraints and the exploratory nature of this approach, we used direct rating with a Visual Analogue Scale (VAS) technique (von Winterfeldt and Edwards 1986, Parkin and Devlin 2006) on the basis of the evidence brought to bear by clinical experts attending the meeting as follows: participants identified the option providing the greatest individual health benefit which was assigned a score of 100; they then scored the remaining interventions relative to this benchmark score of 100 and a fixed benchmark of 0 corresponding to 'no additional health benefits compared to current care'. A rectangle summarised the population health impact *N*B* visually (Figure 18), with the numbers who benefit on the horizontal axis and the average benefit per person on the vertical axis. The area of the rectangle is the expected overall benefit of the intervention in the population.

- Assessing the impact on reducing health inequalities *I* on a VAS. Interventions which had no impact on health inequalities were given a score of zero. Participants identified the option with the greatest potential to reduce health inequalities (assuming successful implementation and compliance); this was assigned a score of 100 and the remaining options scored relative to this benchmark.

- Assessing the operational and political feasibility of the option by asking participants their degree of belief that it would deliver the stated benefits in probabilistic terms *p* (with 100% representing absolute confidence).

In case of disagreement, participants explored the reasons and sought a consensus view, which was usually reached. If a consensus view could not be arrived at, the range of proposed values was recorded for sensitivity analysis purposes and the majority's view at the end of the discussion used for the base model.

Table 22 Example of template and scores: options for cancer (a similar template was used for each of the other four priority areas and their eighteen interventions)

| Initiative [j]	No. who benefit per year [N_j]	'Average' beneficiary	Description of individual benefit compared to current care	Health benefit per person score [B_j]	Health inequality reduction score [I_j]	Feasibility (Probability of success) [p_j]
Early detection & diagnosis in cancer	200	Person in her/his mid-60s, more likely to be female and from "hard to reach" groups in society	Earlier diagnosis is associated with better prognosis (we assume no benefit for people screened and with negative results)	100	100	95%
Palliative & End of Life care (all diseases)	1,500	Person in her/his late 70s, with life limiting long term health condition, equally likely to be from any socio-economic groups	Benefits to carers/family/friends. Benefits to patient: no change in life expectancy but a better quality of life in its last months	75	50	70%
Relocation of active treatment in cancer	300	Person in her mid-60s, more likely to be female; extremely severe illness	Patients are already receiving this treatment off the island, but there are psychological benefits of providing the service locally	25	0	10%
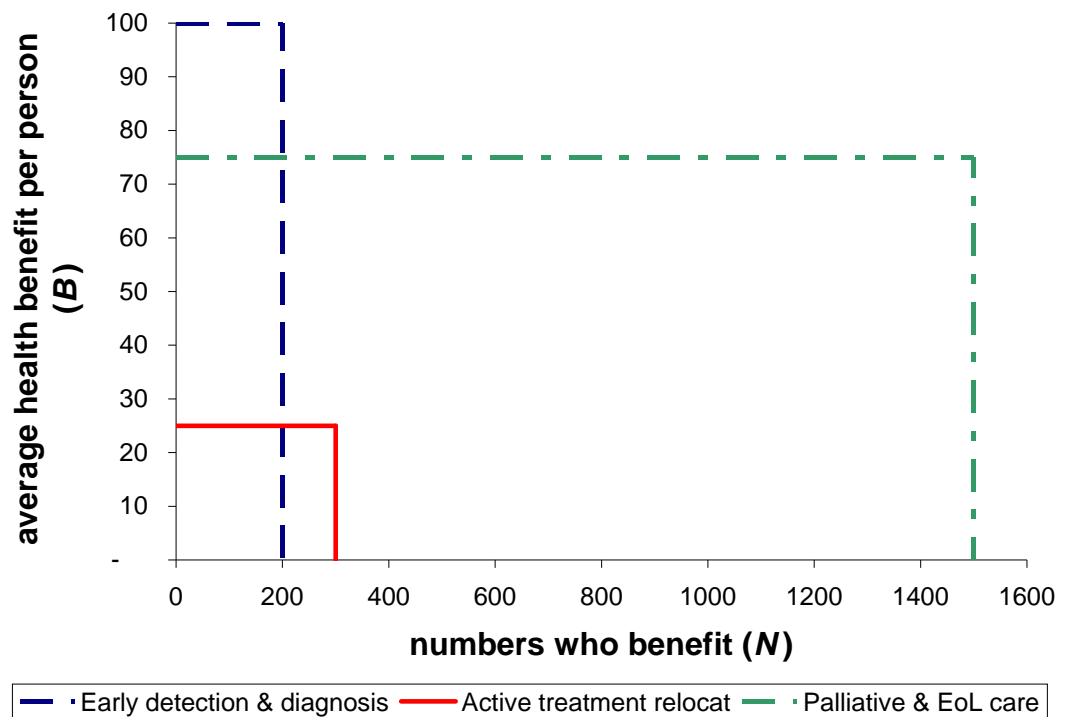
Figure 18 The rectangles of health benefit to the population for the three proposed initiatives in Cancer. Similar rectangles were drawn for each of the five areas and their interventions.

The facilitator elicited three vectors of weights from participants in order to convert the scores on the three criteria on a common metric (Goodwin and Wright 2004). With the first vectors of weights, $w^B = (w_1^B, ..., w_g^B, ..., w_6^B)$ and $w^I = (w_1^I, ..., w_g^I, ..., w_6^I)$, participants considered one objective at a time and assessed the *relative contribution* to achieving the given objective by investing in a set of interventions in a disease group *g* (e.g. all proposed initiatives in the Cancer area) compared to another (e.g. all proposed initiatives in the Respiratory one). These weights are rescaling factors to convert scores for the same criterion in different disease areas on a common scale. Twelve within-criteria weights were elicited in total and a weight of 100 was assigned to the highest w_g^B and the highest w_g^I. Then participants considered health benefit and inequality reduction criteria and assessed their *relative contribution* to achieve the PCT's objectives, to convert scores on different criteria on a common value scale. To elicit this

weight, participants considered the disease areas which received the highest within criteria weights of 100. A single rescaling factor W was sufficient to render scores on the health inequality criterion commensurable with scores on the health benefit criterion. The weighting judgments express critical value tradeoffs, and the facilitator encouraged participants to discuss these tradeoffs openly, noting uncertainty and disagreements to be explored by sensitivity analysis.

Defining $g(j)$ as the commissioning group of intervention j, the expected value of each intervention was hence calculated as indicated by Equation 9 (assuming k=0 during the decision conference and $k \in [0,1]$ in sensitivity analysis after the event):

Equation 9

$$E(v_j) = p_j \cdot \left(w^B_{g(j)} \cdot N_j \cdot B_j + W \cdot w^I_{g(j)} I_j \right) + (1 - p_j) \cdot \left[k \cdot \left(w^B_{g(j)} \cdot N_j \cdot B_j + W \cdot w^I_{g(j)} I_j \right) \right]$$

Thus, at the core of the analysis was a value model based on the expected value, with value computed as a weighted additive combination of health gain and inequality reduction. There is precedent for this model structure in the decision analysis literature: for example, Keeney and Winkler (1985) also present an additive model with absolute and distributional components for evaluating risk reductions.

Third, participants were presented with a triangle that focused the discussion on the cost-effectiveness of each intervention (Figure 19). The horizontal side of the triangle represents the additional cost c_j associated with the intervention; the vertical side represents the additional expected benefit score $E(v_j)$; and the slope of the hypotenuse of the triangle represents cost-effectiveness with steeper hypotenuses representing higher cost-effectiveness. Showing the triangles stimulated a discussion both for their comparative size and slope. In most cases participants recognised the comparison as a fair representation of their intuitive judgments, but they had now a language to entertain a more informed discussion. In few cases results were less intuitive and explored extensively by revising the assessments of costs and benefits that constituted the scale and slope of the triangle creating a better understanding of the appraised

interventions. Whenever necessary, assessments were revised following this exploration.
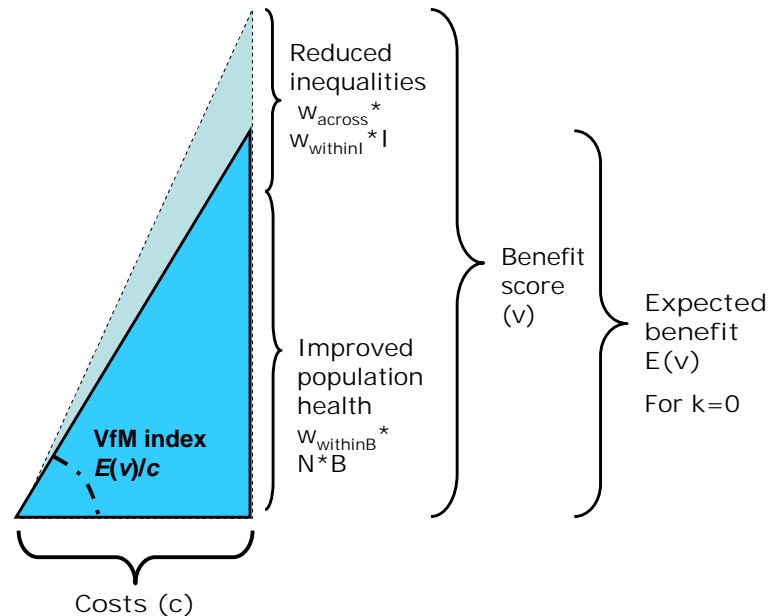
Figure 19 The structure of a value-for-money triangle

6.3.3   Results

The triangles were used to generate a priority list in which interventions were ranked according to value-for-money (Table 23). This ranking is similar to a cost/QALY league table. Extensive sensitivity analysis was used to explore the uncertainties and disagreements among participants and the model proved robust. Figure 20 shows the same information in graphical form. The visual display generated important learning: for example, one intervention the evaluation of which had attracted considerable attention within the organisation, was represented by a triangle which was not only shallow (and thus relatively poor value-for-money), but tiny, because it touched such a small number of people. Thus, from a population health perspective, and from the point of view the cost imposed on the system, the intervention had little impact.

Table 23 Priority order according to Value-for-money ('league table') for k=0.5.

| Commissioning area [*g*]	Intervention [*j*]	Additional cost in £k [c_j]	Additional benefit [$E(v_j)$]	VfM ratio [$E(v_j)/c_j$]	Cumulative cost in £k	Cumulative benefit
RESPIRATORY	pneumonia	£75	11.84	0.1579	£75	11.84
MENTAL HEALTH	Dementia services	£50	5.18	0.1036	£125	17.02
CVD	TIA & 2ndary prevention	£130	5.40	0.0415	£255	22.42
MENTAL HEALTH	Prison MH	£150	4.51	0.0301	£405	26.94
CHILDREN	Obesity training	£60	1.73	0.0289	£465	28.67
CHILDREN	Workforce development	£100	2.78	0.0278	£565	31.44
MENTAL HEALTH	Psych therapies	£120	3.05	0.0254	£685	34.49
CANCER	Early detection and diagnostics	£300	5.74	0.0191	£985	40.23
CHILDREN	CAMHS School	£160	2.76	0.0173	£1,145	42.99
CVD	Prevention	£650	10.48	0.0161	£1,795	53.48
CHILDREN	CAMHS 1:1	£80	1.26	0.0157	£1,875	54.73
CVD	Cardiac Rehab	£100	1.29	0.0129	£1,975	56.02
MENTAL HEALTH	Alcohol misuse svc	£300	3.77	0.0126	£2,275	59.78
MENTAL HEALTH	Social inclusion	£300	3.75	0.0125	£2,575	63.54
CANCER	Palliative & EOL	£760	9.05	0.0119	£3,335	72.59
CHILDREN	Obesity 1:1	£140	1.22	0.0087	£3,475	73.81
CHILDREN	Primary prevention	£600	4.61	0.0077	£4,075	78.42
CHILDREN	Access to dental	£480	3.24	0.0068	£4,555	81.66
CANCER	Active Treatment	£50	0.31	0.0062	£4,605	81.97
CVD	Stroke emergency	£600	3.37	0.0056	£5,205	85.34
CVD	CHD acute	£300	0.78	0.0026	£5,505	86.12
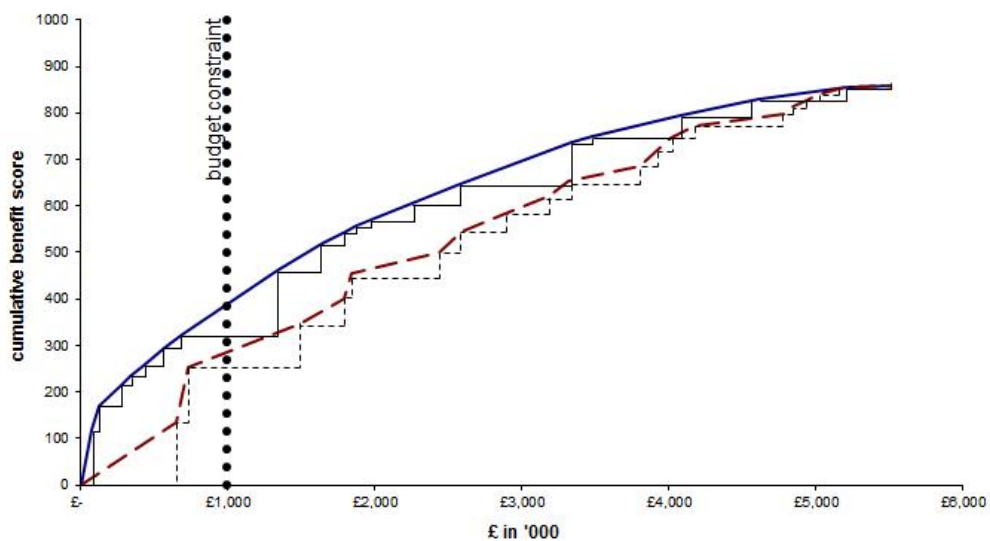
Figure 20 The efficient frontier of triangles ranked by value-for-money (solid triangles) and the frontier with the ranking by overall benefit score (dashed triangles)

Three weeks after the decision conference, participants received a copy of the report for consultation. The report summarised the approach, documented each step of the process, and the results of the base models and of sensitivity analyses. The executive directors and commissioning leads discussed the results and proposed an investment plan based on the analysis to the IoW NHS Board for approval. The proposal followed the ranking of Table 23, with the exception of End of Life care for which separate funding was sought in addition to the planned £1m.

The IoW NHS Board received the results of the analysis favourably and approved the proposed operational plan, including the provision of additional funds for End of Life care. The following year, 2009, the PCT hired a private consultancy firm of trained decision analysts able to replicate the approach and participants from the previous year confirmed their willingness to engage in the workshops, which were extended to more people. Thus the approach which we introduced was seen as adding value, and had momentum.

6.4 Discussion

This section discusses how the use of requisite models and the engagement of stakeholders in a facilitated, deliberative process contributed to the systematic use of CEA principles. We frame this discussion in terms of the concepts of *accessibility* and *acceptability* as used in Bryan and colleagues (Bryan, Williams et al. 2007, Williams and Bryan 2007).

6.4.1 Accessibility

The visual aids proved essential to make the CEA framework accessible to non-health economists. The use of rectangles to visualise the population health gain helped clinicians and patients to share their knowledge and to articulate their expert opinion on the impact for the individual patient; and it enabled participants to discuss more clearly the details of the implementation, the number of beneficiaries and the associated costs, and to document the rationales of agreed changes. The visualization of cost-effectiveness through triangles and their aggregation in an efficient frontier was particularly useful to communicate the principles of CEA as evidenced by comments from several participants (mostly managers and patients representatives), who felt they could fully appreciate the meaning of cost per QALY estimates for the first time.

The understanding of the evaluation framework was crucial both to incorporate available clinical and epidemiological evidence and to assess interventions for which evidence was missing or weak. This was particularly evident for interventions in primary prevention, for which hard evidence was not available and local characteristics of the health economy were particularly critical: it enabled participants to volunteer estimates and for these to be challenged by others.

At the decision conference it became evident, however, that the definition of health inequalities was not as accessible as we would have liked. In particular, if health inequalities are measured in terms of the health gap between different groups in the population measured for instance in terms of quality-adjusted life expectancy at birth, one would expect that the higher the number of health-poor people affected, the higher the impact on health inequalities. Participants, however, did not consider the number of

people affected by the intervention unless prompted by the facilitators and the rationales used to defend their health inequality score usually reflected their personal view of the extent of "health-poverty" of a typical beneficiary or his/her deservingness of better health. The development of a more intuitive and theory-based approach to modelling health inequality is the focus of ongoing research (Morton and Airoldi 2010).

6.4.2 Acceptability

The Strategic Planning group (with the objection of one member) found the approach generally acceptable in terms of the included criteria, their definition, their operationalisation, and the method to translate values into a priority order, with the exception of the evaluation of palliative and end of life treatments.

The objecting member raised a general concern with the use of "an approach which aims at getting the greatest good for the greatest number". This was clearly a rejection of the utilitarian principle embedded in the 'a QALY is a QALY is a QALY' principle commonly applied in health economics. At its core the objection was a pragmatic one: she contended that it would have been difficult to defend hard choices based on the utilitarian principle in front of the public or the courts. The majority of the Strategic Planning group, however, thought that the utilitarian principle was acceptable and highlighted the hard trade-off involved in funding decisions; they agreed to aggregate health gains across people additively in the analysis and to discuss the political feasibility of the recommended set of interventions to be funded at the end of the process.

For the specific assessment of palliative and end of life interventions the executive directors and commissioning leads judged the approach unacceptable and decided to ignore the results for these treatments. They felt that the value of these interventions is to provide 'a good death' and this could not be captured by the criteria used in the approach. They were not able to articulate a general, acceptable definition of 'good death' as different patients and their families may have very different needs at this difficult time of their life and 'a good death' may have more to do with allowing them time to understand these needs and respecting their wishes than with a specific healthcare intervention (Sandman 2005). The difficulty of using a common tool to assess curative and palliative interventions is not unique to this case study, as demonstrated by the debate about the appropriate cost-effectiveness threshold within the cost per QALY

approach for end of life treatments, in which some authors advocate for the appropriateness of a higher cost per QALY threshold (Mason, Jones-Lee et al. 2009, Towse 2009).

The assessment of preventative interventions and potentially life-saving interventions posed a similar challenge, with participants invoking the 'rule of rescue' principle – the moral imperative to rescue identified people in immediate peril regardless of the costs – to express their difficulty in comparing the relative health benefits across these interventions. The director of public health noted that "It is quite hard for all of us to weigh up the difference between treating one or two very seriously ill people with the latest technology or treating very large numbers out in the community who may not be perceived to be as acuely in need of health service. This [approach] is a means of translating that into measurable benefits of some sort" (The Health Foundation 2010, p17). The executive directors decided to exclude 'rule of rescue' considerations in the formal analysis in order to be able to quantify and to face the hard trade-offs between investing in prevention compared to treatment. They recognised that the choice between prevention and potential cure is an intrinsically difficult value judgment but also highlighted the value of visualising the opportunity cost of providing additional treatment to inform their decisions. Their difficulty is consistent with the current absence of any clear, agreed operationalisation of the rule of rescue principles (Cookson, McCabe et al. 2008). Despite the dismissal of 'rule of rescue' considerations, however, the analysis identified most preventative interventions as cost-ineffective because they were usually also associated with a relatively low probability of success (which reduced their expected value).

The opportunity cost of alternative budget allocations was modeled explicitly by using requisite cost-effectiveness models. Indeed the Board found the efficient frontier particularly insightful, because it enabled them to articulate a clear rationale for the proposed allocation based on the principles of opportunity cost. For instance, the analysis did not support funding for a major package of primary prevention of cardiovascular disease. The Board discussed the cost of including this package in terms of the forgone benefits from interventions that would be displaced. As Dr Smith commented: "[with this approach] you are able to show the board that what you want to invest in could get 10% more benefit in terms of health outcome than doing it

another way. The board doesn't always want to do that, but it's a very good way to understand the basis on which you're making the decisions" (The Health Foundation 2010, p19).

6.5   Conclusions

The paper presents a case study to illustrate, and to demonstrate the feasibility of, a deliberative approach to CEA. The proposed approach is not a substitute for methodologically rigorous CEA for the purpose of technology assessment at the national level. It does show, however, that health planners at the local or regional level could use CEA principles systematically even if evidence on the effectiveness and cost-effectiveness of considered interventions may not be available, and could overcome known accessibility and acceptability barriers of CEA.

The distinctive characteristics of the approach are the use of requisite detail to assess the cost-effectiveness of all interventions considered for funding, the use of visual aids to make CEA concepts accessible to non-health economists, and the engagement of key stakeholders in the interactive development and interpretation of the models of cost-effectiveness and the underlying data. Deliberative CEA requires a facilitator trained in health economics and MCDA. This is because the requisite models use health economic principles and concepts to combine evidence from public health, demographic surveys, health economic studies, RCTs, local administrative and accounting systems. The proposed visual aids enable those with no training in health economists to understand CEA principles and to contribute value judgments, and expert knowledge in interpreting available evidence. Stakeholders can also assess judgmentally the cost-effectiveness of interventions for which no published CEA is available. Although these estimates are necessarily approximate, they are better than the alternative, which is no information. Furthermore, these estimates represent explicitly the values and knowledge of those involved in the resource allocation process and help them to communicate and explain the rationale of their recommendations.

The success in facilitating clear, value-driven and evidence-based discussions are attributable to the intellectual robustness of the underpinning health economic theory, and indeed, where our methods were less informed by health economics – as in the modelling of inequality, and the health benefit/ inequality tradeoff – we were less

successful in facilitating such discussions. In areas where health economics theory has less purchase as a normative theory – in particular in the valuation of End of Life care – decision makers set aside the priorities as assessed by the model and, for explicit and legitimate reasons, made their decisions on the basis of other concerns. This is as it should be, for in an arena where values are as contested as healthcare, the highest aspiration for analysis can only be to provide a basis for thoughtful and informed moral choices.

7 Disinvestments in practice: overcoming resistance to change through a socio-technical approach with local stakeholders

Abstract

For health care, economists have developed Cost-Effectiveness Analysis (CEA) as a "rational", analytical tool to set priorities. Attempts to use CEA to decide how to cut expenditure, however, have been met with stakeholders' resistance. This paper presents an illustrative case study of the application of an approach explicitly designed to engage stakeholders with conflicting objectives in confronting tightening budgets. The outcome of this process, which engaged a group of stakeholders including patients, carers, clinicians and managers, was a strategy that reconfigured services to produce more health gain at reduced total cost. I argue that the key factors that led to overcoming resistance to change were: (i) the collective character of the deliberations; (ii) the analysis of the whole pathway; (iii) the presence of patients; (iv) the development of a model based on CEA principles which provided a credible rationale for difficult decisions.

7.1 Introduction

For the United States and many developed countries, fiscal problems mean constrained growth in the costs of health care against the pressures of inflation from advances in technology offering the potential to do more to relieve the suffering of

aging populations. Although there have always been constraints on the total costs of health care, which are manifested in different ways according to each country's systems, the prospects for the next decade are that these pressures will become more intense.

Economics is the discipline that aims to enable these pressures to be tackled so that we can achieve the greatest benefit from increasingly scarce resources. For health care, economists have developed methods to assess benefits without using money as the numeraire, namely gains in health as measured in Quality-Adjusted Life Years (QALYs; Williams 1985), to be used in Cost-Effectiveness Analysis (CEA; Gold, Siegel et al. 1996, Drummond and McGuire 2001, Drummond, Sculpher et al. 2005). CEA aims at informing evidence-based policies by allocating resources to maximise QALYs. The basic idea consists in 'marginal analysis, that is in deriving an 'incremental cost effectiveness ratio' (ICER) for each intervention compared to the next best use of resources. This number is the ratio between the additional costs and the additional benefits measured in QALYs attributable to the intervention. The lower the ICER, the higher the cost-effectiveness of the intervention. One may imagine a table with all possible health interventions ranked from most to least cost-effective. According to CEA, resources should be allocated according to this ranking, funding interventions from the top of the list and drawing a line when all available resources are spent. QALYs and CEA are famously used in the English National Health Service (NHS) in deciding whether new therapies ought or ought not to be funded from the NHS budget by the National Institute for Health and Clinical Excellence (NICE). Because it is not feasible to draw a table with all possible health care interventions, NICE uses a threshold value of between £20,000-£30,000, i.e. interventions with an ICER below the threshold are generally considered cost effective and funded (National Institute for Health and Clinical Excellence 2008).

From a 'technical' perspective, CEA could support equally well decisions about funding new interventions or about disinvestments. Disinvestments are "the process of (partially or completely) withdrawing health resources from existing health care practices, procedures, technologies, or pharmaceuticals that are deemed to deliver little or no health gain for their cost, and thus do not represent efficient health resource allocation" (Elshaug, Hiller et al. 2008, p2). In particular, to advice about disinvestments, analysts could aim at identifying interventions that are currently available but have an

ICER above the threshold. If these disinvestments do not release sufficient resources, the threshold should be lowered.

In practice, however, the context of funding new interventions and that of disinvesting are significantly different. Indeed, attempts to identify interventions for disinvestments through cost/QALY analyses have not been successful to date (Elshaug, Hiller et al. 2008, Garner and Littlejohns 2011).

A fundamental difference between the investment and disinvestment scenario is that the loss of an actual service is perceived differently from the failure to obtain a new service. Key actors in a health-economy (e.g. pharmaceutical companies, providers of care, clinicians and patients) have hence very different incentives to share private information about the costs and benefits of specific interventions in the two scenarios. In the case of investments, they volunteer business cases and models to argue for additional resources as in the English experience with NICE. In the case of disinvestments, a health planner wishing to identify a list of interventions that should be investigated as potential disinvestments is immediately confronted with fierce stakeholder resistance, for putting a procedure under the lens would automatically stigmatise it (Elshaug, Moss et al. 2009).

In this paper I present an illustrative case study of priority setting in health care in a context of decreasing resources, to illuminate how the framework of CEA and a participative process led to a strategy to reconfigure services to produce more health gain at reduced total cost. The case study has been conducted in the English NHS, but results are in principle generalizable to health care systems funded through taxation such as Australia, Canada or the Medicare and Medicaid programmes in the United States. The paper is structured as follows. The next section discusses the methods. The following section presents the case study in detail, including background and terms of reference, participants, the socio-technical process, results and actual impact. In the final session I reflect on the political and institutional dynamic created by the socio-technical process and how it contributed to attain agreement to cut spend.

7.2 Methods

In this paper I use case study and action research. Case studies are particularly useful to describe how and why an intervention worked in a given time and setting (Yin 2009). Action research is characterized by the participative engagement of the subjects of the research and by its aim to analyse and at the same time change the organisation (Eden and Huxham 1996, Gray 2009). Action research is particularly useful for obtaining a rich description of the intervention in order to generate an emergent theory of what happened (Montibeller 2007).

The approach I took was decision analytic. Decision analysis is a discipline which aims at helping an individual or group to formally represent a problem at hand, systematically analyse it and to agree a course of action. The approach consisted of a series of workshops in the form of 'decision conferences', which are working meetings attended by key stakeholders, led by an impartial facilitator, to build a 'requisite' model of the problem on-the-spot to incorporate available data and judgments of participants (Phillips 2007). A model is requisite when it is sufficient to represent the mental models of participants by representing their beliefs about uncertainty and their preferences and refinements to the model do not add insights into the problem (Phillips 1984).

The analysis of the case study is based on extensive field notes and a report produced by an independent evaluator who interviewed participants before and after the decision conferences. The field notes include the chronological development of the engagement process, comments and reflections on its development by the Steering group who oversaw the process, semi-structured and unstructured interviews with clinical staff, patients and health care managers, email correspondence with event participants during and after these events, direct observation of workshops, flipcharts produced during the workshops, minutes of Board meetings following the events, comments and reflections of participants at a follow-up afternoon with a wider set of stakeholders to present the methods and results to key actors in the local health economy, and two follow-up interviews with the health care manager responsible for managing change (at one and at two years follow-up). The independent evaluator (David Collier) is a professional consultant who has extensive experience with decision conferencing and deliberative approaches to support decision making in the public sector. He conducted a series of interviews with the Steering group, the top

management of the PCT and some workshop participants before and after the events, on an expenses-only basis.

In Chapter 8 of this thesis I also reflect on the overall learning from the action research case studies presented in Chapters 5, 6 and 7.

7.3   Case study

7.3.1   Background and term of reference

Sheffield PCT was a large Primary Care Trust (PCT) of the English NHS responsible for purchasing health care on behalf of a population of about 550,000 people living in Sheffield, a city in the North of England. It had an annual budget of about £900 million. PCT budgets were determined by the Department of Health through a capitation formula (Department of Health 2008).

The PCT had a recurrent overspend on its budget and it expected an increase in demand because of demographic change. It hence set an organisational goal to save £40 million and 400 additional lives by 2012 (Sheffield PCT 2008). The action research project described here aimed at strengthening the prioritisation process to contribute to this goal. The 'strengthening' consisted in enabling the PCT: to assess the relative cost effectiveness of different health care interventions, to use this information in resource allocation and to communicate planned changes to stakeholders.

In this paper I present the work conducted for and in collaboration with the lead commissioner for mental health services. He proposed to focus on the care provided to patients affected by eating disorders, which fell under his remit. These patients have an abnormal attitude towards food that changes their eating habits. Some may starve themselves in order to lose weight (anorexia nervosa), others feel compelled to overeat (binge eating) and others may combine binge eating with forced vomiting (bulimia).

Expenditure in hospital treatment for patients with eating disorders had been rapidly increasing and the PCT was reacting to demand because it lacked a clear

strategy. The lead commissioners suspected that resources spent in this area were not delivering good value-for-money and he wished to improve the priority setting process to better allocate available resources.

The strengthened prioritisation process, if successful, will have informed the development of a commissioning strategy. At the time of writing this paper, eating disorders services were provided in Sheffield by NHS statutory services, the voluntary and the private sector in a variety of settings from primary care (e.g. family doctors) to tertiary care (e.g. specialist, residential care units). The PCT commissioned services from each of these providers on an incremental basis in order to meet existing demand. Through the design of a commissioning strategy, the commissioning manager aimed at agreeing - in collaboration with providers and users of the services - a more proactive role for the PCT to improve patients' health, their experience with the service and at the same time contain costs.

7.3.2   Participants

A Steering group was set up to oversee the process. It consisted of the Director of Public Health, three commissioning managers (including that of mental health services), the Head of Finance, and managers responsible for information services and data analysis, for patient and stakeholders engagement, and for PCT commissioning. The Steering group met fortnightly from June to November 2009. The Director of Public Health chaired these meetings and regularly briefed the CEO and the Director of Strategy on the progress of the work.

The decision conferencing process involved twenty-four key stakeholders, selected by the manager responsible for commissioning eating disorder services (17 stakeholders attended the first meeting and 19 the second one; 14 attended both). These were nine managers of provider organizations, five clinicians, five patients and carers, two managers of the PCT, and two representatives of the Mental Health Partnership Board (a partnership with members from all the main health organizations promoting mental health in Sheffield which facilitates inter-organizational collaborations to improve health and reduce health inequalities). I facilitated both meetings, with the support of two analysts. Before the second meeting, which was attended by several patients, I was also

briefed by an expert in eating disorders on the characteristics of these patients and on how I could best enable them to feel comfortable and to contribute to the discussion.

7.3.3 The socio-technical process

The social dimension

The case study took place between June and December 2009. The timing of the key events is summarised in Figure 21. The scoping of the work was drafted by the commissioning manager and signed off by the Steering group in July. The scoping document identified nine intervention areas which broadly captured the PCT spend of about £1.5million in this disease area: inpatient admissions to specialist hospitals, day-services, specialist services in the community (Sheffield Eating Disorder Service or, simply, SEDS), emergency hospital admissions, services offered by community Mental Health team, specialist services offered by nurses at the University clinic, services offered by general practitioners, those offered by voluntary sector, and admissions to acute psychiatric wards.

Two decision conferences which ran from 10am to 4pm engaged the local stakeholders in iteratively assessing the value and the value-for-money of current services. The aim of the first meeting was to consider available clinical and epidemiological evidence on the services provided and to explain the methodology. A data-pack with evidence from the literature and data available to the PCT was tabled on the day. The aim of the second meeting was to build a model of the PCT spend across the identified interventions and their outcomes, based on the best available evidence, expert and value judgments of participants and to develop insights to inform a commissioning strategy.

A further meeting was organised to follow-up on the results of the analysis and to explore possible improvements to available services in a climate of decreasing resources. Participants were stakeholders who took part in the decision conferencing process and additional representatives of primary care commissioning.
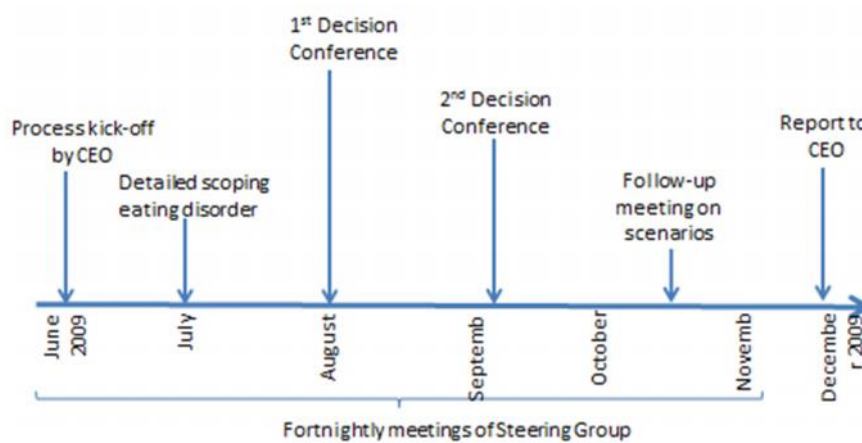
Figure 21 Timeline of the case study

The technical dimension

The stakeholders engaged throughout the process did not typically have any health economic training and the experience of building the model enabled them to understand the evaluation framework through 'learning by doing'.

Between the first and the second decision conference, the stakeholders contributed information for each service following a common template to provide: a description of the interventions provided by the service; the annual cost of providing the service in the previous year; the number of patients accessing the service; the characteristics of these patients (both clinical, e.g. severity of the disease, and socio-demographic); the effectiveness of the intervention (either quantitatively or qualitatively); the benefits from the intervention beyond the patient (e.g. on carers); feedback from service users about the quality of the service, if available; and any other information considered relevant (e.g. trends in cost and volume of service use, expected changes).

The stakeholders agreed that this information is in principle necessary to decide on how to allocate healthcare resources, yet the exercise of collecting it systematically

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revealed significant gaps. In fact, whilst data on the number of patients accessing the services and its costs to the PCT were routinely collected, no such information could be retrieved on the benefit of the service to patients and their carers. This information was hence generated in the second meeting though deliberation and expert consensus as I will explain later.

I facilitated the interactive construction of a model with participants drawing from the templates and participants' judgment during the second decision conference. Through the model participants assessed the value of each service based on: c*ost (c_i)* defined as the annual funding provided by the PCT to offer the service; and *population health benefit (v_i)* defined as the product of the number of patients who benefit from the service (N_i) and the average health benefit per person in terms of quality and length of life (B_i); i.e. $v_i=N_i*B_i$.

The population health gain was assessed in three steps:

1)   Participants assessed the quality of life of patients with mild, moderate or severe eating disorders. The classification in these severity classes drew on the standard classification used by health care professionals in Sheffield (based on body mass index, frequency of laxative abuse or induced vomiting, physical complications and duration of the illness). I helped participants to assess quality of life using a direct rating technique (von Winterfeldt and Edwards 1986) by introducing the visual analogue scale commonly used in health economics with 0 representing the quality of life equivalent to being dead and 1 the quality of life of being in full health. I divided participants in groups ensuring that multiple perspectives were represented (clinicians, managers, patients and carers). Within each group participants assessed the quality of life of the three different severity classes. To facilitate the assessment, and to generate scores which were consistent with quality of life scores available in the literature, I provided the quality of life weights associated with related conditions – i.e. obsessive compulsive disease (OCD) and anxiety or depression (Stouthard, Essink-Bot et al. 1997). This benchmarking technique is used in the field of risk analysis to represent and communicate hazards through 'risk ladders' (Sandman, Weinstein et al. 1994, Connelly and Knuth 1998). Each group identified a range of values. In a plenary discussion each group explained the rationale of the identified

range of values to generate a consensus quality of life weight for each of the three severity group. The consensus weight consisted in a range of values that participants believed to represent 90% of the patient population with that severity and a median value representing the 'typical' patient. Higher and lower values were noted. The example of 'mild eating disorders' is provided in Figure 22.

2)   Participants revised the health state of patients accessing each service. In principle these assessments could be evidence-based. A review of the literature on the effectiveness of interventions, however, showed that the evidence was usually not conclusive because most studies had small samples of patients. The use of the methodological principles of case-control studies helped to structure the discussion systematically and to generate a transparent audit trail to justify and challenge these judgments. I invited participants to consider the health gains for patients who engaged with the services over a one-year period. Participants with expertise in each service contributed on the spot to a simple model to represent their knowledge and judgment by considering the average quality of life of patients who engage with the services; the average quality of life after one year assuming they received the intervention; the average quality of life after one year assuming they had no access to the intervention (the 'counterfactual'). For simplicity, we assumed a linear change in quality of life from the beginning to the end of the year. An example of the model to estimate the average, individual health gain from engaging with the Specialist Eating Disorder Services is provided in Table 24 and Figure 23.

3)   Participants revised the number of patients accessing each service each year to estimate the population health gain, i.e. the product between the average health gain constructed in step 2 and the number of beneficiaries. A simple visual tools of 'rectangles' represented population health benefits as illustrated in Figure 24: each service is associated with a rectangle reporting the numbers who benefit on the horizontal side, the benefit per person on the vertical side; the area of the rectangle represents graphically the population health benefit.
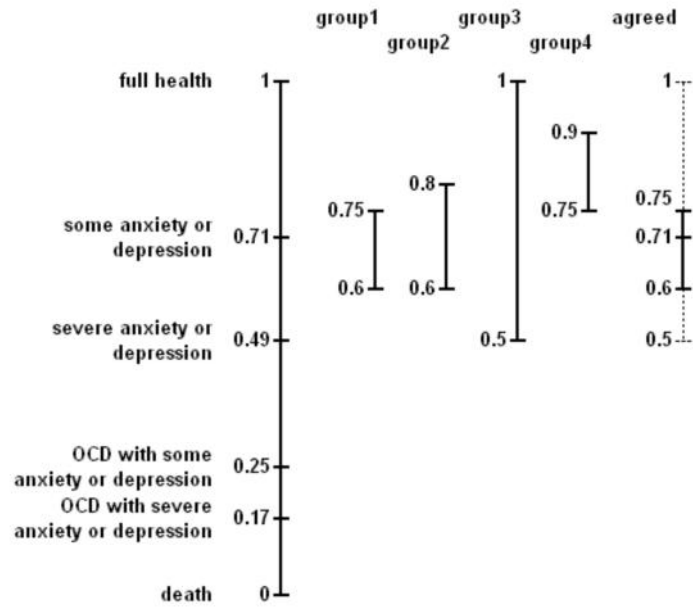
Figure 22 Assessing the quality of life weight of 'mild eating disorders'

Table 24 Health benefit (compared to counterfactual) generated by Specialist Eating Disorder Services (SEDS)

| Number and severity at start of engagement with service	In care (treatment condition)		Counterfactual ('control' condition)	
	Health after one year	Quality of life weight	Health after one year	Average quality of life weight
150 patients. All severe (Quality of life at presentation: 0.12)	2% become more severe	0.1	15% become more severe	0.1
	10% stay the same	0.12	30% stay the same	0.12
	20% improve but remain severe	0.15	35% improve but remain severe	0.15
	38% improve to moderate	0.5	10% improve to moderate	0.5
	20% improve to mild	0.71	5% improve to mild	0.71
	10% recover	1	5% recover	1
Quality of life (weighted average) after one year	0.476		0.239	
Health gain from one year engagement with the service	(0.476 – 0.239)/2 **= 0.118**			
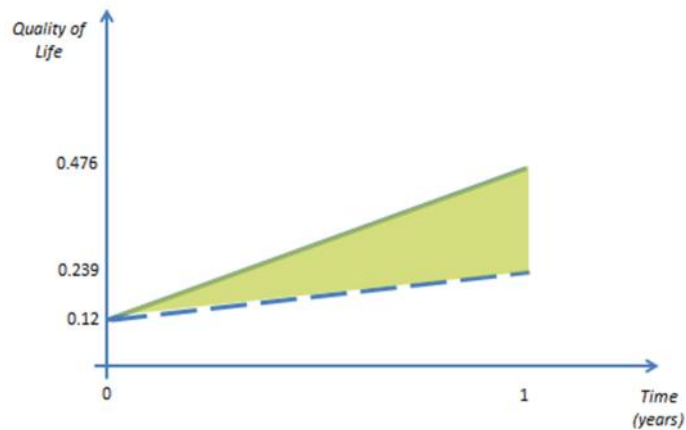
Figure 23 Assessing health gains. The solid line represents the simplified health profile of the average patient engaging with Specialist Eating Disorder services for one year (from a quality of life of 0.12 to 0.476); the dashed line the counterfactual (from a quality of life of 0.12 to 0.239); the shaded area is the health gain, i.e. (0.476-0.239)/2 = 0.118.
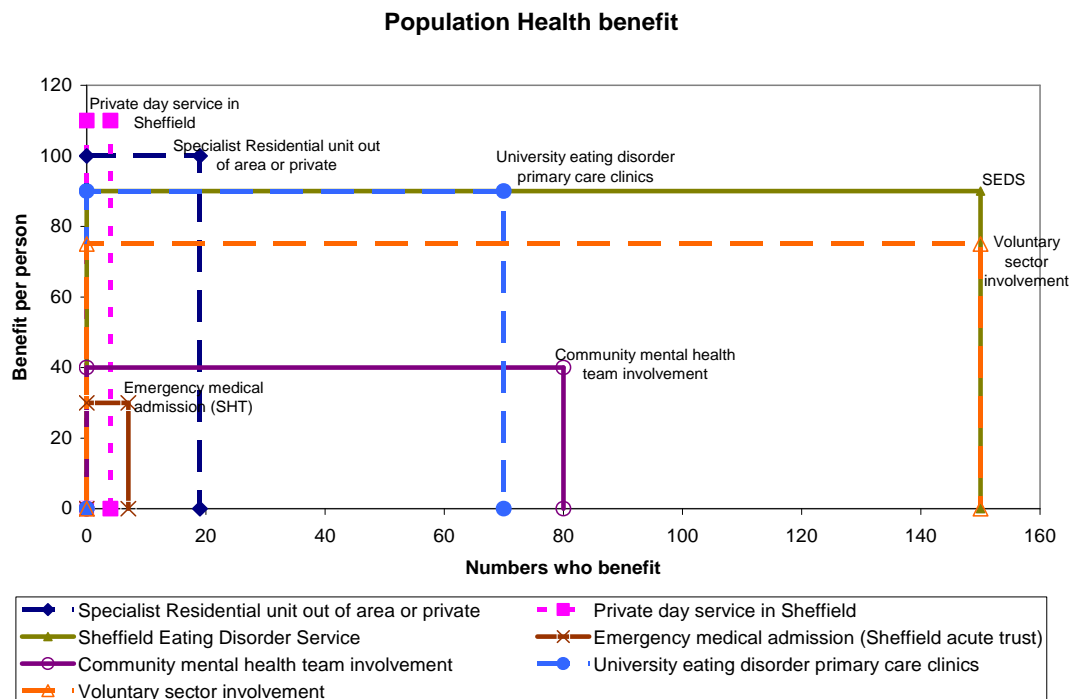
Figure 24 Assessed population health benefit represented by the area of the 'rectangles' (i.e. numbers who benefit times benefit per person).

To represent the relative value-for-money of each service, information on costs and population health gains was represented through 'Value-for-money triangles' as illustrated in Figure 25: the horizontal side of the triangle reports the annual cost of the service, the vertical side its value in terms of population health benefits and the slope of the hypotenuse its value-for-money (the steeper the slope, the higher the value-for-money).


Assessed benefits
of service i (v_i)

$v_i = N_i B_i$

v_i/c_i
Value-for-
Money index

Cost of service i (c_i)
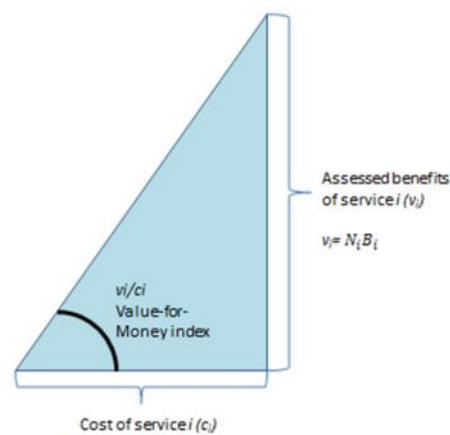
Figure 25 Value-for-Money triangle

7.3.4 Results

Drawing from CEA, interventions were ranked according to their cost-effectiveness ratio as reported in Table 25. The visual aid of 'Value-for-Money triangles' was used to help participants understand the cost-effectiveness ratio (i.e. the inclination of the hypotenuse) and the resulting production function, i.e. a graph representing cumulative expenditures and cumulative benefits ranking interventions from the most to the least cost effective, as illustrated in Figure 26.

Table 25 Interventions ranked by cost-effectiveness or 'Value-for-Money'

| Intervention i	Cost to PCT in £000 [c_i]	Benefits [v_i]	Cumulative cost	Cumulative benefit	Value-for-Money ratio (unit of benefit per pound spent) [v_i/c_i]
University eating disorder primary care clinics	12	7.91	12	7.91	0.659
Voluntary sector involvement	30	11.22	42	19.14	0.374
Sheffield Eating Disorder Service	214	21.74	256	40.87	0.102
Private day service in Sheffield	48	1.5	304	42.38	0.031
Emergency medical admission (Sheffield acute trust)	64	0.85	368	43.22	0.013
Specialist hospital or Residential unit out of area or private	971	7.44	1,339	50.66	0.008
Admission to acute psych wards	46	0.04	1,385	50.7	0.001
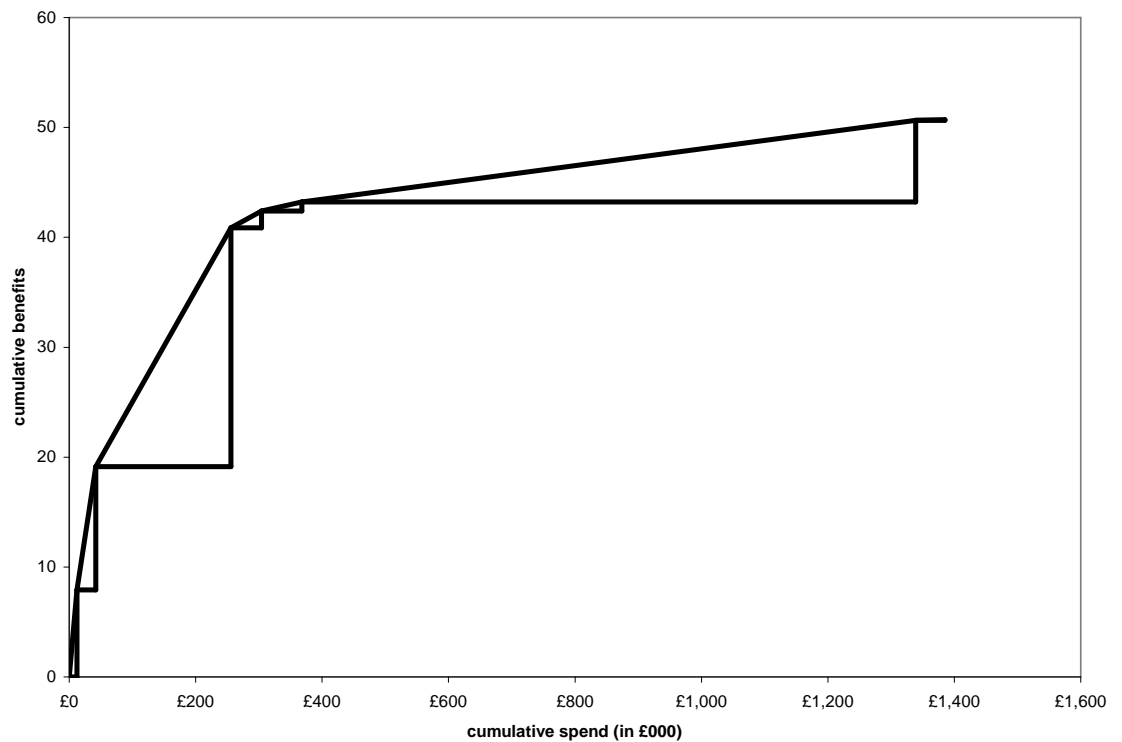
Figure 26 Production function: health benefits to the population with eating disorders at different level of expenditure. The seven 'triangles' correspond to the seven assessed services in order of their value-for-money, i.e. (starting from the origin of the graph) 1) University eating disorder primary care clinics; 2) voluntary sector; 3) Sheffield Eating Disorder Services (SEDS); 4) private day-services; 5) emergency medical admissions; 6) inpatient admission to specialist hospital; 7) admission to acute psychiatric wards

Figure 26 shows that three interventions are relatively high value for money (i.e. the clinic offered by nurses on the University campus, the services provided by the voluntary sector and SEDS). These services cost about three hundred thousand pounds in aggregate, i.e. about 20% of the budget spent by the PCT on eating disorders, but produced about 80% of the estimated population health benefits. The other four services offered much lower value-for-money. In particular the graph showed clearly that inpatient admissions were absorbing a very large proportion of the budget and did not generate much value.

The relatively low value-for-money of services for most severe patients focused the discussion on preventing the progression of the disease to advanced stages. This discussion took the form of 'what-if' scenarios developed during a follow-up meeting. Based on the results, participants considered the expansion of primary care support by replicating the service offered at the University eating disorders clinics and to boost services provided by SEDS in collaboration with the community team. Table 26 and Figure 27 illustrate one such alternative.

Table 26 One of the explored scenario for resource re-allocation: assessed costs and benefits of interventions post-reallocation ranked by Value-for-Money

Intervention i	Cost to PCT in £000 [c_i]	Benefits [v_i]	Cumulative cost	Cumulative benefit	Value-for-Money ratio (unit of benefit per pound spent) [v_i/c_i]
University eating disorder primary care clinics	92.3	62.4	92	62	0.68
Voluntary sector involvement	30	11.22	122	74	0.37
Sheffield Eating Disorder Service	282	27.6	404	101	0.10
Private day service in Sheffield	48	1.5	452	103	0.03
Emergency medical admission (Sheffield acute trust)	32	0.42	484	103	0.01
Specialist hospital or Residential unit out of area or private	485	3.72	969	107	0.01
Admission to acute psych wards	46	0.04	1015	107	0.00
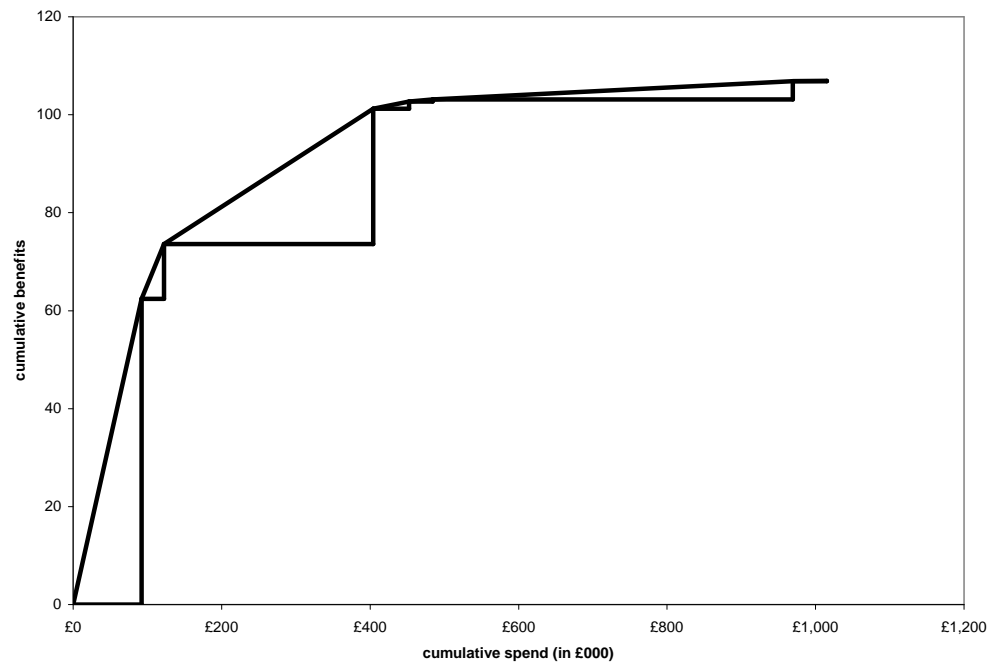
Figure 27 Estimated production function following the potential resource re-allocation detailed in Table 26

7.3.5 Impact

Before the intervention, a relatively high number of patients became so severely ill to need access to residential care for several weeks.

Following the insights of the scenario analysis, the PCT developed a formal business case to reallocate resources. The core idea of the business case was to reduce the number of referrals to residential care by expanding capacity in primary care and increasing services offered in the community or as outpatient treatments at the local hospital (e.g. SEDS). The case took into account that this change might have led to an increase in emergency admissions. The decision conferences and the follow-up meeting on scenarios provided the evidence of cost-effectiveness and of stakeholders' support.

The senior management team considered the business case in July 2010. They approved an expansion of local and community services (SEDS). The aim of this expansion was to provide the capability of treating border-line cases locally, rather than referring them to residential care. The model helped to identify financial incentives to

prevent supplier-induced demand, i.e. that less severe patients would have been referred as a result of available beds. Managers and clinicians from SEDS estimated the potential 'savings' from expanding their unit's capacity to treat borderline cases locally. To ensure they revealed their best clinical judgment, however, they had to contribute to the cost of private tertiary care services above their prediction. They estimated a 50% reduction in the cost of these referrals.

The financial net effect of this change was a reduction of spend for the eating disorder programme by more than 15%. At two-year follow-up, the new care pathway has been implemented and the savings realised.

7.4   Discussion

The case study described in this paper is a successful example of disinvestments in health care, as resources were partially withdrew from residential care services. The case study is relatively small in scale by focusing on a narrow patient population and by achieving a relatively small disinvestment compared to the overall PCT budget of about £1bn. Its small size, however, allowed a close observation and a detailed discussion of the process with participants in order to draw insights on the role of the analysis and of the process to support disinvestment decisions as argued in the following sections.

7.4.1   'Live' model building with stakeholders increases buy-in of recommendations

The first decision conference opened in a tense atmosphere of mistrust. Participants were aware of the recurrent overspend of the PCT budget and expected hard decisions, namely cuts, if they could not articulate the value and cost-effectiveness of particular services. The mistrust and resistance to change was vocalized earlier on in the meeting, after one participant articulated that she felt "to be in front of a judge, with everybody here to defend their own corner, to prove the value of the hard work they do". At the end of the first decision conference, however, the atmosphere was radically different and evidenced by participants' willingness and actual effort in the ensuing weeks to collect and to share both data and expert knowledge to build the analytical model and to inform the process.

What did account for this shift in attitude? Participants overtly reported at the end of the workshop that they could appreciate two attractive features of the approach. First, the process is participative in assessing the relative value-for-money of local, current services on the whole pathway of care. Participants noted that, although they knew of each other and regularly interacted between them, the decision conference was the first occasion in which they all met. In particular, participants praised the framing of the problem in terms of informing an overall strategy 'to benefit the patients they served' rather than engaging in hard negotiations between service providers and service purchaser. Within this frame, participants volunteered private information. The shift from a defensive to a participative attitude is crucial to enable education and communication about the logic of change and, as a result, to reduce resistance to change (Kotter and Schlesinger 1979). The change in attitude is consistent with that generally observed by practitioners of Decision Conferencing, who claim it enables a shared understanding of the problem, a sense of common purpose and a commitment to the way forward (Phillips 2007).

Second, the 'live' development of a model also helped participants to understand the principles of cost effectiveness analysis, which are usually difficult to grasp for people without a health economic training and hence not used systematically to inform policy (Bryan, Williams et al. 2007, Eddama and Coast 2008). Participants could hence contribute data, expert and value judgments, and could use the framework to articulate alternative resource allocation scenarios. One GP who participated in the events noted that "[the facilitators] explained [the approach] very well. Even if you'd been there without knowing the background, it was all talked through. […] There was a good opportunity for people to be heard and the process wasn't too cumbersome" (The Health Foundation 2012). The independent evaluator also noted that "people from a wide range of backgrounds who would not normally have contact with such approaches seemed to understand it". A senior manager, who oversaw the process as members of the Steering group but did not participate in the event, was particularly surprised that participants could come to a shared view on benefit assessment despite their different backgrounds.

Model ownership (through stakeholder engagement) is considered a key factor in generating buy-in for difficult decisions, as discussed in the environmental risk literature (Renn 1999).

7.4.2   Shifting negotiating powers by assessing all services simultaneously

The simultaneous engagement of representatives of services along the pathway of care also changed the negotiating powers radically. In theory the PCT should negotiate with several providers for each service which, competing in order to secure a contract, have an incentive to increase quality and reduce costs. In practice, however, in the case study there were only one or two providers for each service who, at least in the short term, faced no competition. Through one-to-one negotiations between the PCT and each individual provider, the PCT could not control costs easily. This was particularly difficult for tertiary specialist hospitals because admissions to these services were agreed for named individuals, case by case on the basis of medical need. Once a patient was so severely ill to require these services, the PCT paid for them. By considering the whole pathway of care simultaneously, providers of services who could prevent disease progression or could manage severe (yet not extremely severe) cases could argue a case for additional resources by showing the potential savings and the PCT could held them to account for these estimates through risk sharing.

The change in negotiating power described above is similar to that reported by Treasury officials in the UK as a result of the creation of the Public Expenditure Survey Committee (PESC) in the 1960s to control public expenditures (Clarke 1978). The Committee provided projections of public expenditure by each department assuming current policies would continue. Before PESC, Treasury officials negotiated the budget with each Government department, one at a time and felt to be 'nibbled to death' (Heclo and Wildavsky 1981, p 207). After PESC, requests for additional funding needed to be traded-off against other claims for the same resources and, by the end of the 1960s, the Treasury had regained control on public expenditures (Clarke 1973).

Two senior managers of mental health commissioning noted that, outside the decision conferencing environment, participants often returned to their usual negotiating strategies rather than focusing on the entire pathway to benefit patients.

A key element which during the decision conferences prevented the discussion to gravitate around partisan interests was the mere presence of patients. The commissioning manager of mental health services commented that "Just to have service users involved – any at all – was an enourmus plus. They did provide insights we might not have had otherwise. They were good at feeding in information such as what it's like to spend two or three weeks on an acute medical ward as an eating disorder patient. […] It's a brilliant way of getting interested parties to sit down and try to overcome their particular interest in order to develop a rational approach" (The Health Foundation 2012; p17). Dr Harvey, a GP who attended the decision conferences, said "I have experience of dysfunctional commissioning in other areas, where someone external seems to make the decisions and no-one can influence them. But this was a good opportunity for people providing services at all levels to be heard' (The Health Foundation 2012; p18).

7.4.3 Role of the "CEA" to generate a credible rationale for difficult decisions

Fourteen months after the process, the commissioning manager reported that the analytical model had been fundamental to create a business case for change. He believed that the resources consumed by tertiary services were not delivering as good value-for-money as other services even before the Decision Conferencing process, but in the past he had been unable to make a compelling case for shifting resources.

The model developed in the case study draws from QALYs and CEA principles. The use of quality of life weights (Figure 2) and their combination with time (Figure 3) draws from the QALY literature. The estimate of the incremental health gain from an intervention (compared to the status quo, as described in Figure 3 and Table 1) and the focus on the ratio of incremental costs and benefits (i.e. the slope of the hypotenuse of the value-for-money triangles) draw from CEA principles.

The use of QALYs and CEA principles helped to overcome resistance to change, even in the absence of rigorous information on the effectiveness of treatments, because of the deliberative nature of the process. The principles from QALYs and CEA provided a framework for organizing information, preferences and beliefs of participants, and a compelling logic to embed them in resource allocation. Once these principles were established, the use of local agents' expert judgment and of extensive sensitivity analyses to overcome gaps in evidence on effectiveness became acceptable.

The analysis, however, was substantially different from rigorous CEA (Gold, Siegel et al. 1996). "Value-for-money" has not been defined at the margin but at a very high level of analysis, i.e. that of a service offering a variety of interventions. It was hence not possible to derive prescriptive policy recommendation about disinvestments by withdrawing funding from specific interventions of low cost-effectiveness. These departures from CEA made it feasible to consider the entire pathway of care within the available time. Considering the pathway of care is particularly important in a disinvestment scenario to ensure that funded services can sustainably meet the health care need in the population. Indeed, attempts to disinvest from named procedures without considering the impact on the pathway of care have usually not been successful (Kemp, Fordham et al. 2008). This comprehensive exercise helped to avoid the stakeholder entrenchment typically associated with listing a set of candidate procedures for disinvestments.

Furthermore, the provision of information on the scale of benefits and costs, which is not usually provided by published CEA studies, proved fundamental to focus participants' attention in generating ideas on improving services with fewer resources and to focus managerial attention to lead implementation. For instance, although the analysis indicated that 'admissions to acute psychiatric wards' had the lowest value-for-money, disinvesting from this intervention would not release any significant resource. Representing the scale of costs and benefits from each service helped participants to talk about 'the elephant in the room', i.e. that about 70% of the resources were spent on inpatient admissions to specialist hospital but they did not have a significant impact on population health (they were highly beneficial, but only for a very small number of patients as indicated by the rectangle of population health in Figure 4). Participants did not need the analysis to learn about this problem. The analysis, however, helped them

to see the opportunity cost of admitting patients in specialist hospital and to focus their efforts in generating ways to manage borderline cases in the community.

In summary, CEA provided a framework to structure the collection of local information, to overcome the lack of information on effectiveness by helping participants in articulating local knowledge, preferences and beliefs into a QALYs-like measure, and to define 'value-for-money' as the ratio of effectiveness and costs. It was hence a tool to support the organisation to make sense of its current situation and to use this understanding to prompt improvements (Weick 1995).

7.5   Conclusion

In this paper I describe the results of a piece of action research on re-designing a pathway of care for eating disorders in Sheffield (UK) at a time of financial pressure. This work turned out to be relatively successful in supporting a more cost-effective use of resources, to reduce total expenditure and to overcome resistance to change.

The following key features of the approach appear to be crucial to generate cooperation among healthcare stakeholders and to generate ideas for more effective healthcare delivery in the current economic climate: (i) the collective character of the deliberations, which generated ownership in the model and its results; (ii) the analysis of the whole pathway, rather than a particular treatment in isolation as in standard CEA, which helped participants to identify the opportunity cost of alternative budget allocations; (iii) the presence of patients, which reinforced the need to frame the problem in terms of 'benefit to the patients we aim to serve' rather than that of partisan economic interests; (iv) the development of a model based on a clear theoretical framework (i.e. CEA), which provided a credible rationale for difficult decisions.

In musing about whether the approach might work equally well for other efforts to make health services more cost effective, I identify three conditions that contributed to the success of the case study. First, the acceptability of the principles of CEA and of QALYs to assess the value-for-money of healthcare. The exercise presented in this paper was conducted in England, where these analytical frameworks are to a great extent legitimised by the work of NICE. There was hence little if no resistance to the idea of

developing a CEA-like model and in defining value-for-money as the ratio between health gains and costs. Second, the interventions or services being assessed should target a relatively homogeneous population. In this case study, it was hence possible for participants to agree on a stylized description of the 'typical' patient and the impact of a service on his or her health state. This would be probably too demanding for heterogeneous patient populations (e.g. orthopaedic or paediatric patients). Finally, the approach benefited from the leadership of the local commissioning manager, who succeeded in engaging local stakeholder to attend meetings, share some private information and provide expert judgments to overcome data gaps.

8   Critical discussion and conclusion

This dissertation consists of a review of the literature and five independent essays on healthcare priority setting, focusing on the value of formal analysis to support local healthcare planners in allocating a fixed budget. I argued that the available tools are inadequate; and I designed and tested an alternative.

This chapter is organised as follows. In the first section, I state the original contribution of my dissertation and support such statement with a critical discussion. In this chapter I provide a synthesis of the conclusions I have reached from my research, taken as a whole, rather than repeating the distinctive contributions of each individual chapter. In the second section I highlight the limitations of my work. In the third section I reflect on the direction of the field and position my research.

8.1   Critical discussion

This dissertation makes both an intellectual contribution to the field of healthcare priority setting and a practical contribution to the field of healthcare resource allocation. The intellectual contribution is a synthesis of both economics and decision analysis that is designed to remedy problems with each. The review of the literature showed that tools grounded in health economics are difficult to apply at national level because of accessibility issues and because of the need for a threshold. They also fail to contribute to local healthcare priority setting decisions. At the same time, tools grounded in (multi-criteria) decision analysis fail to incorporate the methodological advances of health economics. My thesis contributed to closing this gap. The practical contribution is that I

designed, and tested the value of, a process and of particular value functions that can be used by local healthcare planners within their limited resources.

In Chapter 2 I identified two sets of prescriptive approaches to support healthcare priority setting. On one hand I identified CBA, CEA and GCEA, which purport to operationalise the normative principles of welfare economics. On the other hand, I identified PBMA, which is an example of prescriptive approaches to engage key stakeholders in identifying the resource allocation problem and in assessing the trade-offs of alternative allocations against multiple criteria. PBMA draws on the normative principles of (multi-criteria) decision analysis.

Among the first set of approaches, health economists propose CEA as mainstream. A major reason to prefer CEA to CBA is that it does not require the monetisation of health benefits. GCEA is an extension of CEA proposed by a group of health economists and epidemiologists close to the World Health Organisation. Most of the criticisms to CEA also apply to GCEA. In the following paragraph I will hence focus on discussing the contribution with respect to CEA and PBMA only.

As I discussed, CEA is theoretically well grounded in welfare economics, but it this theory cannot be applied because of three main issues. First, the theory of CEA poses excessive information demands as it requires the broadest possible search procedure for alternative options and the evaluation of each. To overcome this limitation, health economists suggest the use of a threshold for cost-effectiveness. There is no agreement, however, on the appropriate level of this threshold. In fact, the need for a threshold raises the same issue CEA is intended to avoid, i.e. the monetisation of health gains. Second, CEA is difficult to understand for non health economists as it requires the ability to interpret and assess results from complex simulation models, as well as a critical understanding of the welfare economic principles embedded in health-related social welfare functions. It is hence inaccessible, i.e. difficult for those responsible for making recommendations to appreciate and interpret the results of CEA reports. Third, CEA usually requires good evidence on the effectiveness of interventions, e.g. RCTs. In the absence of RCTs, CEA are either not conducted or, if conducted, are considered less robust (Kelly, Morgan et al. 2010).

A different set of difficulties confronts local healthcare planners, who need to allocate a limited budget every year. Local healthcare planners do not have the economic resources to commission or even to interpret CEA reports. They also have to make resource allocation decisions in the absence of evidence of 'good quality'.

PBMA has been proposed as an alternative, pragmatic approach to support (local) healthcare planners. Indeed, PBMA takes into account the limited skills and resources available locally. As I discussed in Chapter 2, however, PBMA *applications* usually do not embed MCDA principles systematically. In particular, many applications do not employ the conceptual framework of resource allocation, but include cost and/or cost-effectiveness as criteria in a multi-attribute value function. The value functions are usually of additive form, often summing benefits to individual patients (e.g. intervention effectiveness for the average patient) to benefits at the population level (e.g. size of population affected), which is a practice that bears no relationship to health economic principles, or common sense. With the exception of one paper (Peacock, Richardson et al. 2007), there is no discussion to justify a particular form for the value function.

In my research, I have drawn from the strengths of both health economics and (multi-criteria) decision analysis to propose "Deliberative CEA". Deliberative CEA is a particular application of decision conferencing to the specific context of healthcare priority setting at the local level. As such, it can be characterized by its technical and social dimensions, and their interaction by means of a 'requisite model'.

In section 8.1.1 below, I discuss the normative validity of Deliberative CEA by reflecting on the different value functions I used in my research. In section 8.1.2 I discuss the prescriptive validity of the approach I developed by looking at the social process.

8.1.1   The 'technical' dimension: the value function

The 'technical' dimension of Deliberative CEA is embedded in framing the problem as a constrained optimisation (i.e. maximising a value function, subject to a budget constraint) and in the particular shape of the value function.

In my research journey I experimented with different forms for the value function: an additive form with four criteria akin to those used in PBMA (Chapter 5); a part additive, part multiplicative value function to trade-off population health and health

equity considerations (Chapter 6); and a multiplicative value function to focus the assessment on population health (Chapter 7).

It has been difficult to verify the theoretical robustness of the value functions used in Chapter 5 and 6. As I discussed in these chapters, participants have found the weighing procedure to trade-off conflicting objectives particularly obscure. In acting as a facilitator, I also found it extremely time consuming and cognitively challenging for participants to ensure robustness in the scoring procedure. In particular, the use of an additive function to trade-off increases in population health and reduction in health inequalities has been particularly problematic. On the contrary, the assessment of 'population health gains' was a relatively simple procedure that participants could understand and meaningfully discuss in the case studies described in chapter 6 and 7. Multiplying numbers of beneficiaries by the average benefit per person embeds the health economic principle of health maximisation and the egalitarian principle that 'a QALY is a QALY'.

On the basis of the research presented in this dissertation, I hence recommend the following model as a conceptual framework for Deliberative CEA:

Equation 10

$$\max \sum_{i=1}^{n} N_i * B_i$$

$$s.t. \sum_{i=1}^{n} C_i \leq K$$

where the index i refers to (divisible) healthcare interventions; N_i is the number of person benefitting from intervention i, B_i the health benefit to the average patient; C_i the cost of providing intervention i to the population (of N_i individuals) and K the available budget.

As I demonstrate in Chapter 6 and 7, this conceptual framework can be made accessible to non health economists by using the simple visual aids of population health rectangles (to represent N_i*B_i for each intervention), value for money triangles (with the vertical side corresponding to the population health gain N_i*B_i; the horizontal side

corresponding to the cost C_i; and the slope of the hypotenuse representing cost-effectiveness or value-for-money) and the efficient frontier to explain the rationale for ranking interventions according to value-for-money.

The greatest limitation of Equation 10 is the exclusion of other criteria that are considered relevant by healthcare planners, for instance those reported in the PBMA literature (see Table 1 on page 43). In some cases additional criteria might be redundant. For instance, one may argue that 'local and national priorities' are set in order to improve population health and it hence redundant to include it as a separate criterion. In other cases, some of the criteria used in PBMA are embedded in the framework of Equation 10. For instance, the benefit to the individual patient (B_i) captures 'quality of life' and 'effectiveness' (which, for instance, are two separate criteria in an additive function in Wilson, Rees et al. 2006) or 'improvement of efficacy/effectiveness', 'improvement in safety' and 'improvement in patient reported outcomes' (Tony, Wagner et al. 2011). In the case of equity, however, the proposed framework for deliberative CEA needs to be developed further. The analyses presented in this thesis, in particular in Chapters 5 and 6, suggests that an additive relationship between a population health criterion and a health equity criterion is unsatisfactory. It is in fact difficult to imagine interventions that can produce value by reducing health inequalities without producing any health benefit. Let us imagine for instance the option of introducing homeopathic treatments and to target them especially to the most deprived in the population. If the intervention is not producing any health benefit, the fact that it is targeted to the most deprived in society is irrelevant. Yet an additive value function would attach some value to this intervention. The identification of a theoretically sound way to include health inequality criteria is the subject of further research (Morton and Airoldi 2010).

My recommended form for the value function is derived from health economics and is a departure from much of the decision analytic tradition, and in particular from socio-technical approaches such as decision conferencing and PBMA. This means that there is a loss in flexibility, as this value function requires facilitators of deliberative CEA workshops to have a good understanding of normative health economic principles and that participants cannot simply include in the model all the criteria they consider important. But this has the massive benefit of preventing misleading recommendations.

For instance, in Chapter 3 I considered the practice of using local life tables to estimate the 'avoidable DALYs' in GCEA. This practice, which may make sense in the measurement of the current BoD would lead to troubling policy recommendations (i.e. to favour death over extending life for elderly patients with poor quality of life). Chapter 3 identifies the problem and proposes a solution by scrutinizing the theoretical robustness of the value function. I believe these exercises are necessary to improve our understanding and practice of priority setting in healthcare.

8.1.2   The social dimension: stakeholder engagement, power and trust

The social dimension of Deliberative CEA consists in the engagement of key stakeholders, such as clinicians, patients, carers, managers and public representatives; and in the impact of the approach on their interaction, in particular on power and trust as I discussed in Chapter 7.

The engagement of key stakeholders serves three main purposes (National Research Council of the National Academies 2008). First, some stakeholders are the intended beneficiaries of the decision (e.g. patients, carers, public representatives) and hence have the legitimacy to inform how such decisions are made. In some countries this legitimacy is formally recognised and patients, carers and public representatives have the right to be involved in the decision making process (e.g. in the "NHS Constitution" in England, Department of Health 2010). The importance of involving patients in healthcare decisions has been emphasised by the work on shared decision making (see the seminal work of Charles, Gafni et al. 1997). In my experience of working with the English NHS, the presence of patients has also been fundamental to keep providers focused on discussing benefits of alternative interventions rather than defending partisan's interests.

Second, much of the information necessary to evaluate alternative options is not available in routinely collected data or published reports. There may be experts, however, who could overcome gaps in information by sharing their professional knowledge (e.g. clinicians, managers, expert patients). The second purpose of the stakeholder engagement is hence to gather expertise to overcome information gaps. Finally, the stakeholder engagement ensures the approach is participative and that it

can hence generate ownership of the problem, it simplifies the process of explaining the rationale of a decision and it reduces resistance to change.

The value of the social engagement was emphasised in follow-up interviews both in Sheffield and the Isle of Wight. The commissioning manager of mental health services in the Sheffield case study commented that 'Just to have service users involved – any at all – was an enormous plus. They did provide insights we might not have had otherwise. They were good at feeding in information such as what it's like to spend two or three weeks on an acute medical ward as an eating disorder patient. […] It's a brilliant way of getting interested parties to sit down and try to overcome their particular interest in order to develop a rational approach' (The Health Foundation 2012; p17).Dr Harvey, a GP in Sheffield who attended the decision conferences, said "It was new territory to have everyone sitting down around a table, talking […]. I have experience of dysfunctional commissioning in other areas, where someone external seems to make the decisions and no-one can influence them. But this was a good opportunity for people providing services at all levels to be heard'(The Health Foundation 2012; p 17 and 18). These comments were also reported for the Isle of Wight case study by the director of public health: "It's hard work and people put real effort into it, but one of the big selling points is that it wasn't difficult getting people for a second year. They value their contribtion- getting their voice heard and really engaging in the process. […] The real benefit [of the approach] is engagement in the process" (The Health Foundation 2010; p17).

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As I discuss in Chapter 7 in particular, Deliberative CEA has also the potential of building trust and to overcome resistance to change. This was evidenced in follow-up interviews. The director of public health in Sheffield said that "The findings were easily accepted. The clinicians were comfortable with them. The socio-technical approach had made sure they were on board. Its unique benefit was combining that with the scientific and economic rigour necessary to demonstrate the change in spend was going to be effective" (The Health Foundation 2012; p18).

The independent evaluator also noted that "the presence of external stakeholders acted as a brake, along with independent facilitation, on defensiveness or falling back on

negotiating positions. The process as a whole did seem a useful vehicle for collaboration and engagement, and experience in follow up events suggests that the change of dynamic was a factor in promoting openness" (Collier, 2010).

Current prescriptive approaches involve stakeholders with similar aims. Contrary to stakeholder engagement in CEA, however, Deliberative CEA and PBMA focus the analysis on allocating a fixed budget rather than on estimating a precise ratio of costs to benefits. Stakeholders invited to participate in PBMA and in Deliberative CEA consider explicitly the opportunity cost of alternative courses of action. The ethical implications of the recommendations (i.e. possibly tragic consequences) can be hence discussed explicitly. Furthermore, in Deliberative CEA and PBMA, because the models are more accessible, stakeholders are deeply involved in building and validating the model. They are hence more likely to own the model and its recommendations.

8.1.3   Requisite models

One of the main criticisms to CEA/GCEA is that they rely on sophisticated simulation models to generate estimates of costs and benefits from alternative interventions. These models are expensive to produce and to interpret. They are appropriate for organisations such as the NICE, who has access to the necessary skills and resources. They are however not an option for local healthcare planners.

In this thesis I took the view that models are tools for thinking (Pidd 2003). In this view, the output of a model primarily resides in the discussion it enables, rather than the particular numerical results it produces. As Box and Draper effectively put it "*Essentially, all models are wrong, but some are useful*" (Box and Draper 1986, p424)*.* The concept of 'requisite model' proposed by Phillips (1984) provides a framework for assessing the appropriate upper-limit in the complexity of a model, i.e. "a model whose form and content are sufficient to solve a particular problem" (p29).

In this thesis I use the concept of requisite model both for epidemiological modelling and for option evaluation models or priority setting. The model developed in Chapter 4, to illustrate the short and long term consequences of glucose control in type 1 diabetes, is an example of requisite epidemiological model. The structure of that model was much simpler than existing epidemiological models as it did not capture, for instance, the

interaction across multiple complications associated with diabetes. The extensive sensitivity analysis and validation with existing models showed that the model was 'requisite' to assess the scale of population health benefits, although it could not be as precise as state-of-the art simulation models.

In Chapters 5, 6 and 7 I use the concept of requisite priority setting models. In these chapters I did not develop epidemiological models for each intervention in order to assess their costs and benefits. Although these models would add value, local healthcare planners do not have the capacity to develop them. Many models are available in published reports, but they may not have been designed to tackle the issue of estimating costs or health gains from the particular interventions the planners wish to assess. In priority setting, the fundamental issue is assessing the forgone benefits of allocating resources to a particular portfolio of interventions compared to its best alternative (i.e. the opportunity cost). The models developed in Chapter 5, 6 and 7 (and in PBMA) aim to be requisite in generating insights around the opportunity cost. As I argued above, however the form of the value function of Deliberative CEA is theoretically more robust than those used in PBMA.

8.2   Improving PBMA and CEA

In this thesis I present a new approach to priority setting and resource allocation. My research, however, also provides the basis for three recommendations to improve current PBMA or CEA practices as I discuss below.

One recommendation for the PBMA community is to generate samples of value functions that are theoretically robust and can be used to facilitate workshops. In this thesis I discuss three different value functions and recommend a function that includes the following criteria: numbers affected, health gains per person and cost (Equation 10).

A second recommendation for the PBMA and the CEA community is to consider the entire pathway of care in the context of disinvestments. As I discuss in Chapter 7, the analysis of the entire pathway contributed to overcoming resistance to change by shifting the discussion on caring for a patient population rather than on disinvesting from particular interventions. The analysis of different pathway scenario focused the

discussion on achieving the greatest population health benefits given the available budget.

A final recommendation for the PBMA and CEA community is to use visual aids in facilitating the discussion on value-for-money. In this thesis I propose three visual aids: rectangles of population health gains, triangles of value-for-money and the efficiency frontier. These visual aids proved fundamental to include non health economists in the discussion by making accessible the principles of health economics and CEA.

8.3   Main limitations and further research

At the beginning of this dissertation I presented Mark Moore's framework of public value in terms of substantive value, legitimacy and operational feasibility. The contribution of my dissertation is mainly limited to the first aspect of public value, by assessing the population health gain of alternative allocation of resources, assuming the list of alternative courses of actions was known. The identification of alternatives to be assessed requires a problem structuring method that was beyond the scope of my research.

Within this narrow scope, I focused on prescriptive approaches to healthcare priority setting. In Chapter 2 I proposed a simple 2x2 matrix to group these approaches as 'theoretically robust but impractical' (CBA, CEA, GCEA), or 'theoretically weaker but practical' (PBMA). Deliberative CEA is a compromise between these two. As I illustrate in Table 27, however, although theoretically stronger than PBMA, it is not as strong as CEA. And although more practical than CEA/GCEA, it is not as practical as PBMA. On one hand, deliberative CEA is not as theoretically strong as CEA because the models developed are crude. In particular, the measurement of individual health gains relies on the expert judgements of participants in interpreting and integrating available evidence. In these assessments, participants are more likely to consider a 'typical' (or modal) patient rather than the average patient. Yet, their assessment is used as a proxy for the average patient. These assessments take place in the head of participants during the discussion rather than through mathematical models that could be subjected to peer-

review. Participants' views on the benefits of each intervention do not follow the protocols for eliciting health-related utility scores.

On the other hand, deliberative CEA is not as flexible as PBMA because it requires a more sophisticated understanding of decision analysis and health economics. The availability of skilled facilitator is hence a significant barrier to the usability of the approach. Furthermore, the consistency checks required to ensure the theoretical robustness of the model take much time, limiting the number of options that could be assessed in the available time. The systematic use of health economic principles in defining the shape of the value function also limits the criteria that can be included in the analysis. Research into the (lack of) use of health economics suggests that local healthcare planners have other objectives, e.g. managerial objectives such as meeting national or local priorities (which may be necessary for the institutional stability of the organisation).

From a methodological perspective, the value function I propose in Deliberative CEA has several limitations. In particular, I did not provide a way to satisfactorily account for equity issues and to trade-off benefits occurring at different points in time. These are controversial issues currently debated in health economics and it is perhaps not surprising that I could not solve them. By using a decision analytic approach, Deliberative CEA can offer a useful perspective to tackle these issues in that it distinguishes clearly 'facts' (e.g. the socio-economic background of beneficiaries from health interventions) from 'judgments' (e.g. how much additional value we wish to attribute to improving health among the health-poor, who typically come from lower socio-economic groups). This separation enables those responsible for allocating resources to engage in an open debate with the local community and produce recommendations that reflect the preference and judgments of the local community. This is an area for further research, which can be enriched by testing alternative techniques to embed distributional concerns or inter-temporal trade-offs (e.g. Morton and Airoldi 2010).

Table 27 Deliberative CEA in the context of existing prescriptive approaches for healthcare priority setting

Theoretical robustness

	Weaker	Stronger
Higher	PBMA	
	Deliberative CEA	
Lower		CEA, GCEA, CBA

Pragmatic value (vertical axis label)
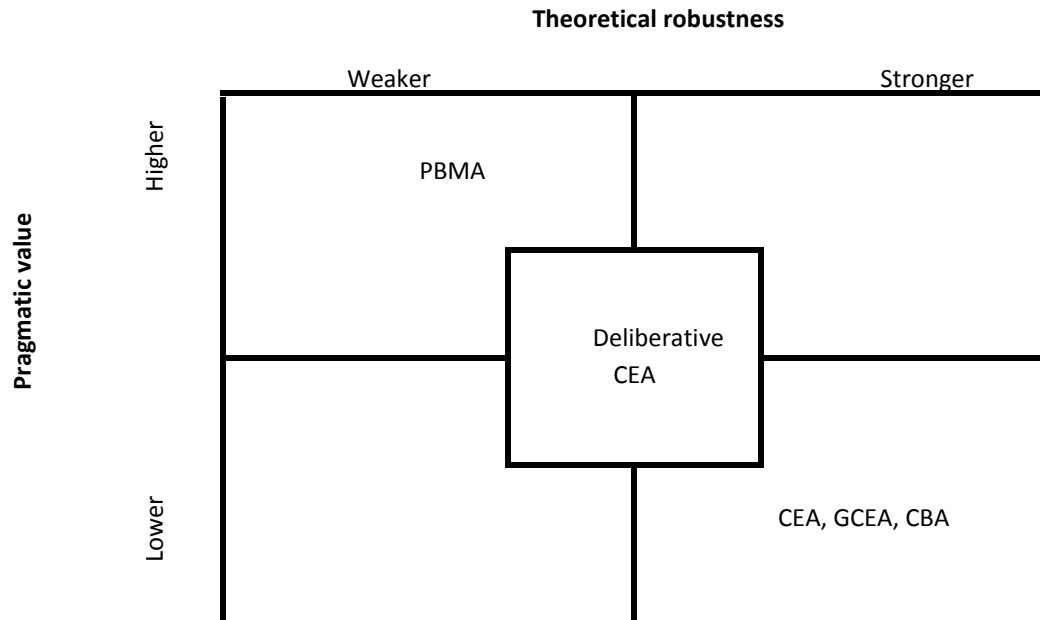
A further weakness of my research which warrants more work is the evaluation of Deliberative CEA. In this thesis I designed a new formal model to support resource allocation decisions. I then used action research through three case studies to investigate its prescriptive value. In keeping with action research convention, in each subsequent case study I amended the approach building on reflections from the previous case (Eden and Huxham 1996, Checkland and Holwell 2007). In Chapter 5, 6 and 7 I discuss the evaluation of the socio-technical approach in each case study. An important area for further research is to conduct a more systematic evaluation of the Deliberative CEA.

8.4   Reflections on the direction of the field

My research was conducted with PCTs of the English NHS. However the findings and the recommendations to the PBMA and CEA community are generalizable to the reformed English NHS as well as to commissioning organisations in other countries, such

as Health boards in Wales, Aziende Sanitarie Locali (ASL) in Italy, Local Health Integration Networks (LHINs) in Ontario, Canada, or Health Boards in New Zealand.

Priority setting will become a more pressing issue in years to come because of increase in demands from healthcare and a reduction in available public resources following the economic crisis. There are hence difficult decisions ahead that will affect the medical care we receive.

At the same time, there is an increased scrutiny and demand on transparency on decisions affecting the public, especially in healthcare 'disinvestments'. Mass participation in the emotionally charged debate about disinvestments can be easily manipulated (Fishkin 2009).

Both these pressures point to the need to engage those affected by rationing decision in a well-structured debate to inform decisions (Sen 1979, Renn 1999, Daniels and Sabin 2002, Fishkin 2009). The objective of my research has been to develop an approach which uses formal models that are theoretically robust and practical to structure these debates. My research shows that it will be crucial to engage stakeholders to overcome resistance to change and it provides a method for doing this.

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