Medicines and choices

Health policy and individual decision-making

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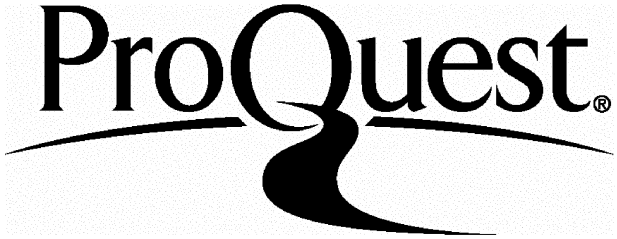

Abstract

Background and aims: Decisions about choices of medicines are made concerning a population or an individual health care user. Key aims of the thesis were first, to investigate authoritative decision-making in the British National Health Service (NHS) about the access to medicines; second, to investigate the views of health care professionals (HCPs) and patients on making choices about medicine treatment within the medical consultation, including the use of informed consent for medicines; and finally, to seek similarities and differences between the factors found to contribute to each type of decision-making about choices investigated before.

Methods: Health policy decision-making was investigated by using a document analysis and in-depth interviews with a range of policy-makers and stakeholders. Individual decision-making was explored by conducting in-depth interviews with doctors, nurses and hospital in- and outpatients from various medical specialities. Informed consent was chosen as an applied model of decision-making where the patient makes a choice.

Results: Cost containment and generating politically and legally defensible decisions were strong influences on the process and the outcomes of health policy decision-making. Although data about benefit, safety and cost considerations were used as the main legitimisation for the definition of access criteria, several informal and organisational factors were found to have significant influence on decision-making. The lack of information provision about side effects and treatment alternatives made it impossible for patients to make an informed decision about their medication. Informed consent was not regarded as a model to support patients in making informed decisions. Although doctors described patients as a 'partner' in the decisions, an eliciting and implementation of the patients' preferences and values was largely absent.

Conclusions: A more consistent application of a minimum moral standard of patient involvement in prescribing decisions could improve individual decision-making. Health policy decisions could benefit from an awareness of the influence of informal factors on health policy decisions.

To my parents

Overview of the thesis contents

Table of contents

List of tables

List of diagrams

List of Abbreviations

ACh	Acetylcholine
AChEI	Acetylcholinesterase inhibitor
AD	Alzheimer's disease
ADAS-cog	Alzheimer's Disease Assessment Scale – cognitive subscale
CHD	Coronary heart disease
DEC	Development and Evaluation Committee
DoH	Department of Health
DTC	Drug and Therapeutic Committee
EBM	Evidence-based medicine
ED	Erectile dysfunction
EMEA	European Medical Evaluation Agency
FDA	Food and Drug Administration
GSL	General Sale List
HA	Health Authority
HCP	Health care professional
HTA	Health Technology Assessment
IC	Informed consent
MHRA	Medicines and Healthcare products Regulatory Agency
MMSE	Mini Mental State Examination
NHS	National Health Service
NICE	National Institute for Clinical Excellence
NSF	National Service Framework
NNT	Number needed to treat
OTC	Over the counter
P	Medicines, which need to be sold under supervision of the pharmacist
PCG	Primary Care Group
PCT	Primary Care Trust
POM	Prescription-only-medicine
QoL	Quality of life
RCT	Randomised controlled trial
SHO	Senior house officer
SMAC	Standing Medical Advisory Committee
UK	United Kingdom
US	United States of America

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PART A: INTRODUCTION

Having the opportunity to choose is usually perceived as a positive value. A choice, in the sense of making a selection out of two or more possibilities or options, potentially increases an individual's control and tailors opportunities towards individual preferences. Traditionally, choices have been studied and discussed in economics and marketing where offering variety and customer's choice have a high value. Expressions such as 'freedom of choice' are well known and used as advertisements for business (Annas, 1998). Promotion of the value of choice went along with a social trend of increased individuality and autonomy as well as the democratic movement of increased lay participation (Donnelly, 2002). Examples are the civil rights movement and also the women's rights movement in the sixties and seventies. In economic terms it was the call for consumer protection, which urged providers to respond to the demands of a stronger consumer lobby (Richardson, 1983).

In health care as in other areas of daily life, several interest groups have demanded choices since there have been an increasing number of treatment options which vary in their effects and risks (Gillespie and Florin, 2002; Iles, 2003). From the point of view of the health care service user there is potentially the choice of a health care plan, which service will be provided (though not in all health care systems), the choice of an individual physician or other health care professionals (HCPs) and the choice of treatment (Brock, 2002). Other choices in health care are related to the societal level. In a publicly funded health care system, choices are made about the overall spending on health care and the amount of money allocated to a specific medical speciality, which has an effect on the number of health care choices offered on an individual level.

Choices in health care have not only been regarded as positive. On the contrary, they have been described as a dilemma and a provoker of conflict, where selecting means narrowing the variety of potential therapeutic options funded by a health care provider and potentially denying funded health care at all (Calabresi and Bobbit, 1978a; Ham and Pickard, 1998). In other words choices can restrict the access to health care. The introduction of renal dialysis into medical practice in the sixties is a frequently cited example, which illustrates dramatically how patients were excluded from access due to

the shortage of dialysis machines and as a consequence condemned to death (Veatch, 2000).

Others have argued that choices are often illusionary or meaningless (Annas, 1998). They are presented as opportunities for individuals to exercise autonomy and control whereas in fact other more powerful external factors determine the selection. Annas (1998) used choices in health care as an example and illustrates that the patients, unlike consumers, are not able "to shop for the best deal". The existence of choices is sometimes confused with freedom.

However, the call for offering choices also raised questions about the individuals' ability to select, which may require them to gather and to retain information, to draw conclusions and to imagine consequences (Schneider, 1998). Especially, in the field of health care it has been argued that the health care user is often ill, and therefore not in control or able to act autonomously (Tauber, 2001). In relation to the ability to make choices the term 'decision' is more commonly used (such as in 'decision-making ability'). It emphasises the aspect of judging and coming to a conclusion compared to 'choice', which has two meanings: first, the existence of several possibilities, a variety of options, from which one can be selected and second, the act of choosing between those options.

Commonly, the authoritative decisions about choices in health care which are made by governmental bodies or health care organisations (macro level) have been seen and discussed separately from decisions about the selection of treatment between a HCP and an individual (micro level). In addition to macro and micro level some authors also use the term 'meso' level where institutional and organisational decisions are made (Mariner, 1994). In this thesis, it will only be distinguish between micro and macro level, where 'macro' refers to governmental or institutional decisions. Commonly, these authoritative decisions determine health policy for a population, which can vary in size, from an entire nation to a region or only a locality, and in exceptional circumstances, the decisions can concern the care of identifiable individuals.

The United Kingdom (UK) government has promoted choices in health care. The "Patient's charter" (Stocking, 1991) defined seven rights of the patient, including the

right "to be given a clear explanation of any treatment proposed, including any risks and any alternatives before you decide whether you will agree to the treatment". The Scottish Charter which goes even further, states that the patient should be given choices wherever possible (Stocking, 1991). In the National Health Service (NHS) Plan in 2000 (Department of Health, 2000a) the government promised that patients' choice would be strengthened. This would include the involvement of patients in decisions regarding health care provision and access to health care as well as enabling the patient to choose their general practitioner and the representation of patients at all decision-making levels of the NHS. Recently, the government has promised that by increasing patient choice, the patient will be able to choose from at least four hospitals in case they need treatment and will have the right to register with more than one doctor (Dean, 2003). The national initiative can be seen as part of a world-wide trend towards patient involvement. The World Health Organisation (1994) published its declaration on the promotion of the right of the patient in Europe, which states, "there is now greater emphasis on the encouragement of individual choice and the opportunity to exercise it freely". It continues: "The informed consent of the patient is a prerequisite for any medical intervention." According to the document, choice means the right to be informed about the treatment proposed, its alternatives and the right to refuse an offered treatment.

This thesis explores decision-making about access and choices from two different perspectives, the macro (or health policy) level and the micro (or individual) level. As seen from mapping the field of choices in health care, there are many perspectives from which decision-making about access and choices of medicines can be viewed, for instance, using a political, historical or social science perspective. In addition, psychology, health economics, epidemiology, medicine or ethics would offer a valid perspective. The perspective of ethics was chosen, which is concerned with the question of how should decision-making about access and choices be made. Apart from ethical research in the field of medical decision-making, medical and pharmaceutical research has been used to complement the analysis of the health policy and individual decision-making.

The outline for the remaining part of the thesis is as follows: first, in part A, the theoretical and empirical background of the work will be presented. This includes the

ethical decision-making models developed for the macro and micro level decisions and the empirical work in the field of health policy and doctor-patient decision-making. Chapter 1 focuses on the macro level, Chapter 2 on the micro level. In part B the empirical research regarding macro level decisions will be addressed. The starting point for the study was the invitation to join a European-wide research project regarding access to health care technologies based on document analysis, which is described in Chapter 3. In a second stage the research was extended beyond the scope of the joint project in exploring the views of a wide variety of decision-makers and stakeholders. The methodology and the results of the in-depth interviews are set out in Chapter 4. Part C (Chapter 5) of the thesis focuses on the micro level of decision-making, where the patients' and HCPs' views of the process of decision-making and their limitations will be analysed including what role they prefer to take in the process of decision-making. Informed consent (IC) for medicines will be examined as an applied model in which the patient makes a choice. Finally, the last chapter will highlight the similarities and differences between the factors found to contribute to each type of decision-making about choices investigated before, present the conclusions of this thesis and identify areas for further research in the field.

CHAPTER 1: INTRODUCTION TO HEALTH POLICY DECISION-MAKING

1.1 Health policy decision-making about access to medicines

In this chapter some common terms used in the field of health policy along with four core principles frequently discussed, in the context of decision-making about access to medicines, will be introduced. The description of two ethical frameworks is used to illustrate how some authors argued decision-making on a population level should be done. This will be followed by the description of three health care reforms in Oregon in the United States of America (US), the Netherlands and New Zealand, which have tried to put some of the theoretical principles into practice, and a discussion of the findings. The recent health care reform in the British National Health Service (NHS), the decision-making bodies and the regulations in place which determine access to medicines in the current system will be the next focus of the chapter. In order to understand recent developments in decision-making about access to medicines, a summary of various information inputs into the decision-making process and some of the techniques of information search and evaluation will be given. Finally, empirical research investigating authoritative decision-making about access to medicines will be discussed.

Over the last two decades there has been increasing recognition that the resources available for health care are insufficient to provide all care which is thought to be beneficial for individuals (Mechanic, 1995). In this thesis the terms 'priority setting' and 'rationing' are used interchangeably to refer to this situation of limited resources, in which there are two or more options available out of which only one or some, but not all, can be provided at the same time. However, some authors distinguished between 'priority setting' and 'rationing', defining 'priority setting' as the allocation of a total budget (the majority of macro level decisions) whereas rationing is related to the allocation of care for an individual identifiable patient (micro level decisions) (Klein *et al*, 1995). The term 'rationing' is probably best known from the time of the second World War, where it meant the distribution of a fixed portion, in particular an amount of food allotted to a person. Rationing implies that what is denied is an option which the individual would have chosen and which would have offered benefit. This

means that individual choice is limited (Light and Hughes, 2001). In general, political publications often prefer the term 'priority setting' since 'rationing' is thought to have a negative image (Dewar, 1999). Both priority setting and rationing influence access to health care.

The terms 'priority setting' and 'rationing' usually imply that there is a fixed amount of resources, which needs to be split among competing parties. However, that does not necessarily reflect the reality of health care budgets, which are – to a certain degree - flexible rather than fixed (Mechanics, 1995). Commonly, scarce resources mean a limited financial budget and they are expressed in monetary terms even though resources can include more than finance, for instance the number of personnel, equipment, time etc.

1.2 Principles guiding the decision-making about access to health care

Relevant for examining decisions about access to health care is the contents of the decisions, the process and the principles on which they are based. The contents of a decision include the definition of who is entitled to have access to what kind of health care and to how much. The process entails the structure and the organisation of decision-making whereas the principles are what guide the decision-makers in making the decisions. Frequently discussed principles determining decisions about access to health care are: the principles of need, right, utility and desert (Malin *et al*, 2002). Each of the four principles will be addressed in more detail and the difficulties in applying them highlighted.

The principle of desert means the extent to which an individual is believed to 'deserve' access according to his or her past achievements or character. It has been used in the past to make decisions about access to renal dialysis at the time of its introduction into medical practice. Jonsen (1998a) describes that, when the first dialysis unit was established in Seattle (US), in 1962, it was found that the number of patients needing dialysis in order to stay alive would far exceed the resources to provide it. As a result, a committee of seven anonymous members was set up, comprising of two physicians

and five lay members deciding who of the medically suitable candidates for dialysis should receive dialysis. As there were no formal criteria, the committee decided to draw up their own. The first was that the patient should be from Seattle. The second was a combination of social factors, which were used to make judgements about "social worthiness", for example, "age, gender, marital status and the number of dependants, income and educational background, occupation and past performance as well as future potential". This resulted in much controversy, when it became apparent that it meant deciding between a businessman, a poet and a parent with three small children (Veatch, 2000).

Despite the variation in the definition of 'need', in most health care systems need is the single most important basis for what is regarded as a fair distribution of health care (Bernfort, 2003). Need can be ranked according to the threat posed by the condition for the life of an individual; for example, the need for health care to avoid immediate death would be allocated a higher priority than the need for cosmetic surgery. Another way to define need is on the basis of the ability to benefit. That may mean that the individual who is at immediate risk of death may not be considered a priority if the individual's chance of benefiting from the intervention is worse compared to another person at lower risk. One of the main difficulties in the application of the principle of 'need' is its assessment in practice and its distinction from 'want'. In order to achieve an objective assessment, it is usually the professionals' task to determine the need for health care, not that of the individual patient (Seedhouse, 1993).

Provision of access to health care on the basis of 'right' means that the national legislation or moral rights define the amount of health care each individual is entitled to receive (Malin *et al*, 2002). In the case of denial of health care, individuals have the option to appeal in court and claim their right. However, the legislation is usually not detailed and concrete enough to provide a useful basis to settle disputes efficiently (Newdick, 1995).

The principle of utility has gained much attention in recent years. In general, utility means that the decision is based on the values the decision-makers place on the outcome of the decision. In a situation of limited resource in a publicly funded health care system, this means that the policy-makers strive to provide health care which

offers a maximum return in quantity and/or quality of health care for the investment of the payers (Mechanic, 1995). In other words, the assessment of the efficiency and medical utility of a health technology such as a new drug therapy in economic and clinical terms becomes a predominant goal when making health policy decisions.

Which principles are used, and how they are defined and weighted in determining access to health care, differs between each health care system. Many authors have suggested a certain set of principles in which decisions about access to health care should be made (New, 1996; Berwick, 2002). Two theoretical frameworks will be used, one developed in the US, the other in the UK, to illustrate how some authors thought decision-making about access to health care should be approached (Daniels, 1985; Doyal, 1995). The authors' definition of the principles of allocation and their priority in determining access to health care will be discussed. Both models go beyond the definition of guiding principles. They argue that in addition to some general principles the process of decision-making needs to be just.

1.2.1 Daniels's model of priority setting

Daniels' work (1985) tries to answer some fundamental questions in health care: the question about the aim of a health care system and what principles should guide the distribution and access to health care based on this definition. According to Daniels, health care is unique compared to other goods in society because it has an impact on our opportunities to perform in life or in society. He argues that there is a wide agreement that society or the state should offer health care to all on the basis of need. A crucial part of his work is his definition of the concept of need and as a result of it the boundaries of health care provision: the overall aim of health care is 'to maintain species-type normal functioning' and to safeguard the 'equality of opportunity' (Daniels, 1985). Hence, the limits of health care are defined by services, which would aim to sustain individuals "as fully participating citizens – normal collaborators and competitors – in all spheres of social life" as he explained in one of his later publications (Daniels, 2001). An example would be the provision of growth hormone treatment for a child who is smaller compared to other children.

His definition of need and the boundaries of health care have provoked criticism (Silvers, 2001; Rhodes, 2001). The definition of need on the basis of normal functioning excludes the treatment of disabilities caused by genetics. Using the previous example, the growth hormone treatment for a dwarf child would be denied because it is outside the normal functioning of this child although the consequences of the disadvantage for a small normal child and a dwarf child are the same. This is further complicated by the fact that in some cases it may be impossible to diagnose the cause of the abnormal functioning (Silvers, 2001). Furthermore, some argue that his definition of need is too narrow and excludes other values that are important to individuals such as freedom from pain when pain is not necessarily preventing normal functioning (Green, 2001; Rhodes, 2001). Rhodes used the example of a patient dying from cancer, which has metastasised to the bone, where the pain treatment will not necessarily provide normal functioning in society, but nevertheless is regarded as vital. Similarly, the avoidance of loss of pleasure is another value that is important.

Daniels based his work on Rawls' political theory of justice as fairness and extended it to health care. Rawls for instance said that, if there are competing needs priority should be given to the least advantaged which have the greatest potential to benefit. This means that the principle of priority setting would be according to the outcome of the health care delivered.

Although Daniels' theory provides some guiding principles as to what fair access to health care could mean, he acknowledged that it is not possible to agree on a common hierarchy of principles determining fair access. Instead, he argues, it is necessary to agree on a process of decision-making. Together with Sabin (Daniels and Sabin, 1998) he developed a framework – the so-called 'accountability for reasonableness', which defines four conditions, which need to be fulfilled: publicity, relevance, appeals and enforcement condition.

1. The publicity condition means that the decisions and their rationales are publicly accessible.
2. The relevance condition is met if "fair-minded" people see the rationales for the decision as relevant in that context.
3. The appeal condition requires that there is a mechanism in place to guarantee the opportunity of an appeal.

4. The enforcement condition means that there is a voluntary or public regulation in place to ensure that the other three conditions are met.

The framework has received much international attention among policy-makers and has been called the "main pillar" for a "new phase of priority setting" (Kleinert, 2000). Some authors used the framework of 'accountability for reasonableness' to evaluate the fairness of decisions about access to health care or investigated its applicability to actual decision-making (Ham and Pickard, 1998; Martin, 2002).

So, in Daniels' work the provision of equal opportunities is closely related to the quantity rather than to the quality of care. In addition, there is no consensus over the health care services which aim to sustain normal functioning of individuals. Regarding the framework of accountability for reasonableness the procedural approach is perhaps more pragmatic than idealistic.

1.2.2 Doyal's model of ethical health care allocation

Doyal (1995) defined seven key principles of health care provision in the NHS (see Table 1.2.1).

Table 1.2.1: Seven procedural principles determining access to health care

Principles
• Health care needs should be met in proportion to their distribution within the population
• Within areas of treatment, resources should be prioritised on the basis of extremity of need
• Those in morally similar need should have an equal chance of access to health care
• Scarce resources should not be provided for ineffective health care
• Lifestyle should not determine access to health care
• Rationality should be optimised in the allocation of scarce health care
• The public should advise but not determine policy concerning the allocation of health care

His model is similar to Daniels' in stating that health care need is based on the ability of "successful social participation" and that priorities should be given according to the severity of need and threat posed by the conditions. Regarding the process of rationing, he also demands transparency of allocation in form of an open, rational and explicit debate and the inclusion of public representatives.

Importantly, in contrast to Daniels, he argues that if the need to treat is equal according to severity and threat posed by the condition, further choices should be made randomly, that means decided in a form of lottery. He argues against a utilitarian principle, where choices are made on the basis of the greatest benefit in terms of the population. Although scarce resources should not be provided for ineffective health care, utility data such as QALY data should only be used to compare treatments for the same medical condition, not between therapy for different diseases. He believes that there is no moral justification in discriminating against certain services and treatments as they might have the same consequences for the individual. He uses the example of cosmetic surgery to treat a disfigurement, and infertility treatment, which can have the same psychologically and socially disabling effects as other conditions. In addition, he emphasised that rationing should be made equally in all areas of health care provision, compared to cutting expenditure only in some sectors. Doyal's work differs in many ways from current health care systems where the formal use of the principle of lottery is commonly rejected as an allocation principle (Calabresi and Bobbit, 1978b). Instead, the utilitarian principle has widely been applied.

1.3 Models of health care allocation used in practice

Some health care systems have carried out reforms, where decisions about access are made in a systematic, explicit and transparent way. In the following section, the Oregon Health Plan, the Dunning Report, and the New Zealand health care reform will be examined as examples of such attempts. The fact that in reality, all of them are only partly implemented led to the conclusion that there is no general rule for these decisions and that the main difficulty is the application of the general principles into practice (Klein, 1995). It becomes clear from the international experience that decisions solely based on maximising the health gain for the investment made have widely been rejected. In addition, the examples demonstrate that public perspective on the provision of health care should be reflected in some way in the decision-making process and that there is also some consensus that ineffective treatment should not be provided.

1.3.1 The Oregon health plan

The example of the Oregon health plan in the US has been celebrated as a prototype of an explicit method of excluding certain provisions of health care (Bodenheimer, 1997). The aim of the health care reform was to extend the number of people covered by a health insurance through limiting the service covered. One of the novelties of Oregon was the involvement of the public in defining which sort of health care should be funded and which should be excluded from funding (Ham and Coulter, 2003).

Community meetings, public hearings and telephone surveys were conducted to gather public opinion about their concerns and values related to access to health care (Buist, 1992). A commission was set up to define a basic package of health care for the population ranking a list of 709 medical conditions and related treatments within 17 categories of health care services such as prevention, maternity care, palliative care etc on the basis of their 'net benefit'. The 'net benefit' derived from a combination of factors:

- the general values related to health care (based on the opinions voiced in community meetings and public hearings);
- the quality of life preferences (obtained from the results of telephone surveys);
- the effectiveness of medical treatment for the defined conditions according to main symptoms and treatment outcome after five years (based on the findings of the professional opinion survey).

These factors were incorporated into a formula determining the rank of the condition/treatment pairs. According to the total budget for health care, the list would allow the drawing of a line: above the line were all health services which were included in the basic health care package and below it all services which were excluded from the basic package.

This method of ranking according to their 'net benefit' and the definition of 17 categories had been established after the first list developed was found to be unacceptable, where for instance tooth capping was ranked higher than treatment for appendicitis (Giacomini , 1999). In addition, in contrast to the first list, where a computer determined the final ranking, on the second list it was done by commissioners, who changed the position of items on the list if it was against their intuitive ordering of items (Buist, 1992). This less often discussed step in the definition

of the basic package, which involved a considerable amount of judgement on the part of the commissioners, meant that in addition to the formula other factors such as the public health impact of certain diseases or the cost to the system and society determined the final ranking. This resulted in change concerning the funding status of 10% of the items. Despite a storm of criticism that several treatments were excluded, the list has been in use since 1994. However, commentators claim that there is a wide gap between the rhetoric and the actual performance of the health plan (Oberlander *et al*, 2001). For example, many services that had been excluded in theory were in fact reimbursed and the exclusion of certain services did not generate the promised savings. Another way to circumvent public critique was to exempt certain health care services from the priority setting process a priori, for instance long term care in order to avoid exclusion (Giacomini, 1999).

1.3.2 The Dunning Report

In the Netherlands, the Dunning Report (1992) suggested a framework for making decisions about a basic health care package rather than to define a list of treatment or services covered. The framework has been compared with a funnel in which the four criteria – necessary care, effectiveness, efficiency and individual responsibility – function as sieves. Necessary care was the first sieve, which was defined as services which either aimed to maintain or restore the ability to participate in society or are determined by the severity of the disease. Only health care, which passes through all four sieves, is provided in the basic package. The report was published at a time of discussion about health care reform and played a key role, although the ability of the funnel to exclude health care from the basic package has been limited (Stolk *et al*, 2002). Massive opposition against some of the governmental attempts to apply the criteria defined by the report successfully prevented its implementation. For example, the suggestion to exclude the provision of contraceptives was opposed by public and women's rights groups (den Broeder, 2000). However, ideas from the Dunning report remain a trend in government policy in the Netherlands.

1.3.3 The New Zealand health care reform

In New Zealand, the government established the National Advisory Committee on Core Health and Disability Support Services, which developed, similarly to the Dunning Report, a four-point framework for making decisions about allocation of the health care budget (Ashton *et al*, 2000). Treatments or services should provide benefit, value for money, represent a fair use of resources and be consistent with community values. The process of priority setting involves seven steps including the consultation of clinicians, service managers and consumers. Key initiatives have been the introduction of a point system for the waiting list for non-urgent surgical interventions and the development of clinical guidelines for a range of conditions. Although the process of consulting the public was judged as politically and socially useful, it did not result in defining explicit public values (Butler, 1999).

Overall, these examples illustrate that it is difficult to find common principles for decisions about allocation that work in practice. Each of the reforms faced strong opposition, which made the implementation of many decisions difficult. It shows the political nature of the decision, where excluding services is avoided to prevent unpopularity. Instead, there is a trend to target services on those patients most likely to benefit instead of on exclusion. None of the reforms have been so successful that they have been adopted in the same way in another country.

Exceptional international examples of health care reforms have been described, which tried to implement some of the theoretical work on health care ethics and resource allocation. The next part describes the organisation of access to medicines in the British NHS, which derived historically and is very different from the models discussed before. In order to understand the current developments the next section will start with the description of the main features of the NHS and the regulations and institutional bodies deciding about access to medicines.

1.4 The British Health Care System

1.4.1 General structure of the National Health Service (NHS)

The National Health Service (NHS) was established in 1948 as a tax funded health service free of charge at the point of delivery for the whole population. Core principles of the NHS are collective funding of health care, offering equal access for equal need, disregarding the ability to pay.

In addition to the NHS, the UK has a small but slowly growing private health care sector with about 10% of the population having some form of private health insurance. Although, 83% of the total health care expenditure is public spending, 13% of all elective surgeries are privately funded and most of the routine dental and eyesight care is bought privately (World Health Organisation, 2002).

In the NHS, general practitioners (GPs) are the first point of call providing basic general health care for the local population. Their role has been unchanged since the foundation of the NHS (Ham, 1999a). As gatekeepers they are referring patients to specialists in secondary care if necessary. The secondary care is strongly separated from the primary sector. GPs treat patients only in the primary care setting. Hospital care is in the hands of specialists. Private treatment delivered by the GP is possible for patients who want care outside the NHS. GPs are only allowed to treat patients privately if they are not registered as NHS patients with their particular practice. Specialists working in secondary care can increase their income by treating patients privately.

In contrast to the international examples of the three health care reforms described previously, the priority setting in the British health care system is less structured and architectural (Klein, 1995). Historically, in the NHS denial and deferral have been used as the main methods of allocating scarce resources compared to the definition of a basic health care package provided (Smith, 1991). For instance, the deferral of elective surgery via waiting lists is a widely used practice. The denial of service, for instance, has been reported for patients requiring renal dialysis, due to limited resources (Kee *et al*, 2000). In general, clinicians are in charge of making rationing decisions, which

meant deciding what amount of health care an individual receives (Klein, 2001). Although the decisions are heavily influenced by the budget available, clinical reasons are used to defend them. Since the decisions are made on a micro level, they are mostly invisible and less open to public scrutiny.

After several unsuccessful attempts to control expenditure through reorganisation of the management of the NHS during the 1970s and 1980s, a major reform took place in the beginning of the 1990s, which resulted in a split of provider and purchaser of health care (Barrett and McMahon, 1990). It was intended to create an 'internal market' where providers of health services competed against each other to obtain funding from the purchasers to deliver health care. This was thought to result in an improvement of quality and efficiency and intended as a shift from the welfare character of the NHS more towards a 'quasi-market' structure. During this time rationing became more widely discussed (Klein, 2001).

One element of the reform was the establishment of district health authorities (HAs) as bodies in charge of purchasing health care for the population within their region (Ham, 1993). Another was the introduction of so-called 'fundholding practices' which meant that GPs could allocate a certain budget – including funding to cover prescription of drugs - in order to provide a defined range of health services for the local population (Ham, 1999a). At the same time hospitals could become NHS Trusts, which were responsible for the provision of all the health services beyond primary care (Whynes and Baines, 2002). This means that HAs, GP fundholders and NHS Trusts were able to make decisions about access to treatment.

Health policy observers have commented that the reform changed only little and that the incentives given to save costs were probably too weak to result in major, measurable savings (Keeley, 1997; Ham, 1999a). The question was which health care services needed to be excluded. However, the explicit exclusion of the provision of certain health care remained an exception. For instance, some HAs created exclusion lists where tattoo removals and other cosmetic surgeries were the most common items to be excluded (Klein, 2001).

In 1995 an NHS Executive highlighted costs as the constraining factor and urged health policy-makers at the regional level 'to ensure that decisions about the provision and delivery of NHS services are increasingly driven by the evidence of clinical and cost-effectiveness...to ensure the greatest gain from available resources' (National Association of Health Authorities and Trusts, 1995). However, in practice decision-making in health policy did not follow clear evaluation principles and defined goals. Instead, many authors have described the NHS policy-making as incrementalistic or "muddling through" (Hunter, 1980; Klein, 1993; Hunter, 1995). The ends have not necessarily had priority over the means. Decision-making is more an answer to the most pressing problems rather than to find long-term solutions (Hunter, 1980).

The following part describes in more detail some of the recent changes, which are related to the decision-making about access to health care. These changes initiated a shift in decision-making about access from being implicit to a more explicit, accountable process, which fostered public involvement.

1.4.2 The NHS since 1996

The most recent changes in the NHS were introduced by the New Labour government in 1997, which further developed some of the earlier introduced strategic changes in the NHS. Laid out in the White Paper "The New NHS: modern, dependable" (Secretary of State for Health, 1997) the two important aims of this reform were first, to create more accountability for decisions made and second, to increase incentives for providers to deliver cost effective and high quality care.

In order to achieve the first goal, the previous reforms in giving GPs more responsibility over their expenditure was taken one step further. The local commissioning groups, which developed out of GP Fundholders, were transformed into Primary Care Groups (PCG), in which membership for GPs was compulsory (Secretary of State for Health, 1997). It was planned that the PCGs would be responsible for the control and allocation of resources in a local population of around 100,000 to 150,000 citizens and responsive to that population's needs. The task of the HAs, which were previously responsible for the control and the allocation of the financial budget within a region, shifted towards strategic planning of health care

services (Department of Health, 2000a). The PCGs took over more responsibilities from the HAs, so that by the end of March 2002 all PCGs became Primary Care Trusts (PCTs) which meant that they were now holding and managing facilities, such as community hospitals, in their own right (Wilkin *et al*, 2001). This also meant a joint responsibility for primary and secondary care funding allocation which was seen as one clear advantage over the previous models in which primary and secondary care were strictly divided (Ham, 1999a). The organisation of equivalent bodies in Scotland and Wales was slightly different.

The second goal of the reform was to create a system which offered incentives for the stakeholders to deliver high quality of care in the most cost-efficient way (Secretary of State for Health, 1997). In the past, quality standard of care provided and local funding differed considerably in various parts of the country. The place of residence decided about the access to treatment paid by the NHS, the so-called "post-code prescribing". This lead to the foundation of bodies to define national standards of quality such as the National Institute for Clinical Excellence (NICE) which produces national guidelines for drug treatment as well as for other therapeutic interventions (Department of Health, 1999a). It is mainly clinical and economic experts that judge about the allocation of resources for particular health care interventions. Additionally, there is a recent movement to strengthen a democratic approach in parallel with a technocratic approach whereby the views of the public are taken into consideration (NICE, 2003a). The National Service Frameworks (NSFs) were created alongside with NICE with the intention of defining standards of health care delivery in priority areas, against which the provision of care would be measured.

1.5 Regulation of access to medicines

Although the NHS was established as a health care system offering comprehensive health care and is still officially described in these terms, there are several regulations in place to limit access to treatment, which means that not all patients, who could potentially benefit from treatment, actually receive it. The following part describes some of the current regulations and the institutional decision-making bodies involved

in determining the access to medicine used in the NHS. Diagram 1.5.1 and Table 1.5.1 give an overview of the different levels of decision-making and their influence on the regulations.

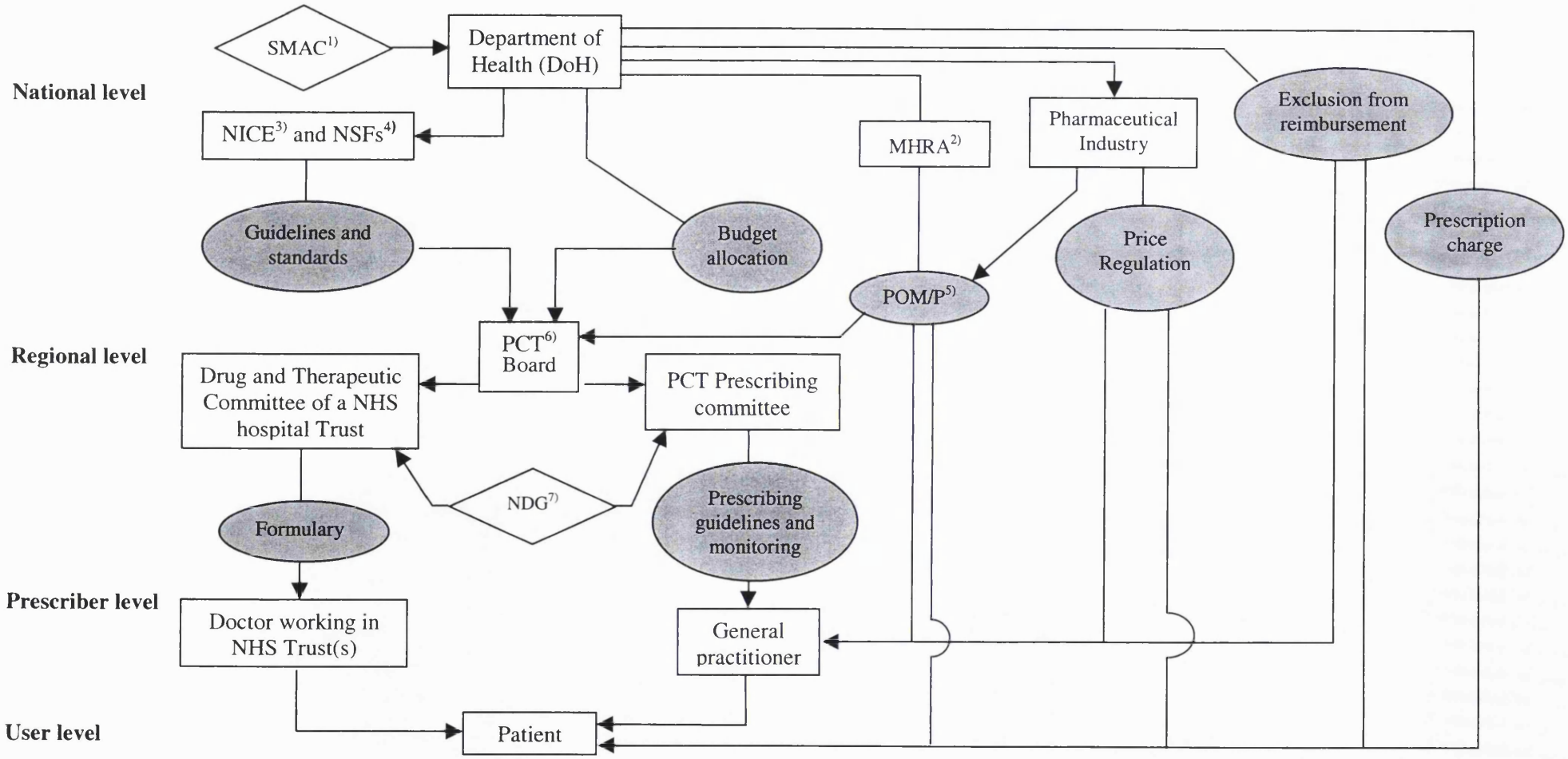
Priority setting and explicit rationing decisions have been traditionally focused on pharmaceuticals. This reaches back to the beginning of the 20th century when the British government tried to ensure that the prescribers were choosing the cheapest medicine out of the range of treatment options with similar efficacy (Abraham, 1995). A list was set up defining medicines, which were reimbursed according to their believed efficacy and costs. Compared to many other goods, the access to medicines has been strongly regulated. Since the Medicines Act of 1968 their marketing needs to be authorised by the national or European agency (Medicines Control Agency, 1996). Due to the requirements of the regulatory process most of the medicines have been tested via randomised controlled trials (Banta, 2003), which differentiate them from other medical interventions. This is probably one reason why pharmacotherapy has been so attractive to policy-makers and played an exceptional role in the process of explicit allocation decisions. Additionally, the prescribing budget is one of the fastest growing expenditure (Department of Health, 1999c). Currently, around 12% of the NHS budget is spent on medicines (Association of the British Pharmaceutical Industry, 2003).

Table1.5.1: Some regulations of the availability and the use of drugs

Level	Kind of regulation	Instrument	Decision-making body	Description
National level	Co-payment of drug therapy	Prescription charges	Government	Fees per item prescribed on the prescription
	Public access	Change of legal status of the drug from POM[1] to P[2]	Medicines and Healthcare products Regulatory Agency (MHRA)	Availability of the drugs without prescription
	NHS reimbursement	Schedule 10 and 11 of the NHS Regulation Act 1992	Government which might ask Standing Medical Advisory Committee (SMAC) for advice	Excluding drugs from reimbursement
	Drug price	Pharmaceutical Price Regulation Scheme	Government in cooperation with the industry	Periodical negotiation of the drug price
	National guidelines on the use of drug treatment	National Institute for Clinical Excellence (NICE) guidelines	NICE	Compulsory funding of certain treatments by the PCTs
	National standards on service provision	National Service Frameworks (NSF) guidelines	Assistance of external reference groups	Compulsory implementation of the standards defined for treatment
Local level	Local guidelines on the use of drug treatment	Delegation of the allocation decisions to the local level	Board of the Primary Care Trust (PCT), Drug and Therapeutic Committee (DCT)[3] of a NHS hospital Trust	Use of formularies to guide drug use

[1] POM: Prescription-only-medicines
[2] P: Medicines, which need to be sold under the supervision of a pharmacist
[3] Some trusts have in addition a New Drug Panel deciding about which drugs go on the formulary

Diagram 1.5.1: Overview of national and local regulations of the availability and use of drugs in the NHS



Legend: □ decision-making body, ⬭ regulation, ◇ advisory body, ➨ influence, [1)] SMAC: Standing Medical Advisory Committee, [2)] MHRA: Medicines and Healthcare products Regulatory Agency, [3)] NICE: National Institute for Clinical Excellence, [4)] NSFs: National Service Frameworks, [5)] POM: Prescription-only-Medicine/P: Medicine, which need to be sold under supervision of a pharmacist, [6)] PCT: Primary Care Trust, [7)] NDG: New Drug Group

1.5.1 Exclusion from reimbursement

As described previously, in Oregon the exclusion of certain services from the health care package was the main mechanism to determine access. Regarding access to medicines in the NHS, exclusion is more the exception than the rule. Legally, a physician is allowed to prescribe all drugs licensed in the UK unless stated otherwise (National Health Service Regulation Act, 1992). Medicines which are excluded from reimbursement by the NHS are listed in Schedule 10 and 11 of the NHS Regulation Act (1992). The lists exclude some medicines of unproven efficacy or less favourable cost-effectiveness ratio for the defined indication(s). Schedule 10 lists drugs which are not refundable ("blacklist"), whereas Schedule 11 ("grey list") contains drugs which can only be prescribed for certain indications. The lists are updated monthly and published in the Drug Tariff (Department of Health, 2002a). In case the government is considering changes in Schedules 10 or 11 it may ask for advice from the Standing Medical Advisory Committee (SMAC), an advisory body of experts from various health care specialities and geographical areas (Department of Health, 2002b).

1.5.2 Reducing the demand for publicly funded health care

Reducing the demand for publicly funded health care is another mechanism by which access to health care can be regulated. In terms of medicines, national regulations such as co-payment or the increasing number of drugs given over-the-counter (OTC) status may fulfil this function. They increase the payment contributed by the individual health care users. As a result, they may decrease the demand by those individual users who can neither afford the costs for payment nor are willing to pay for the medicines themselves.

1.5.2.1 Co-payment for prescriptions

Co-payment for prescriptions has been in place since 1952 (Association of the British Pharmaceutical Industry, 2000). The amount of payment per prescription or per item has been increased constantly from £0.05 per prescription form in 1952 to £6.30 per item dispensed in 2003 (Association of the British Pharmaceutical Industry, 2000; Department of Health, 2003a). Patients who obtain regular prescriptions are able to pay a fixed amount per four months or per year, which means that there is a maximum

of co-payment set (so-called prepayment certificate £32.90/£90.40 per 4/12 months) (Department of Health, 2003a). However, 85% of prescriptions are exempt from the charge since certain patient groups depending on their medical condition or their socio-demographics are excluded from the payment (Association of the British Pharmaceutical Industry, 2000). Various interest groups criticised the co-payment scheme as been "illogical, irrational and [...] against the principles of the NHS" (Andalo, 2002). For instance, some patient groups are entitled to claim exemption such as patients suffering from epilepsy, whereas others with HIV/AIDS are not exempt. The government promised to overhaul the regulation in the near future. Since the co-payment affects the health care users directly it can be seen as a mechanism of deterrence; individuals seeking care are discouraged to use the publicly funded health care service (New, 1996).

1.5.2.2 Increasing the number of medicines which are available over the counter

In some circumstances changing the legal status of the drug from being only available with prescription (POM) to a status of being sold under the supervision of a pharmacist (P) is another way to reduce demand of publicly funded health care. The government has announced plans to significantly widen the range of drugs available over the counter (Department of Health, 2002c). The programme has been promoted as strengthening the patient's freedom of choice. Customers are able to pay for the medicine at their own expense, saving GPs' time and NHS expenses (Bellingham, 2002; Britten, 2001).

1.5.3 Efficiency

1.5.3.1 Pharmaceutical Price Regulation Scheme

Lowering the prices of medicines increases the efficiency of the health care system and is in the interest of the health policy-makers. In the UK, the prices of medicines are negotiated between the government and the industry, the so-called Pharmaceutical Price Regulation Scheme (PPRS), which is a voluntary agreement between the UK government and the pharmaceutical industry to agree on drug prices and restrict profits (Department of Health, 1999b). It is a periodically negotiated compromise between excessive profits and the need for companies to get return for their investments. It has

been criticised that HCPs and patients are not taking part in the negotiations (Earl-Slater, 1997).

1.5.3.2 National guidelines and standards to regulate the use of drugs

Apart from excluding of services, another mechanism to regulate access is the definition of certain access criteria. That means that the services are offered, only under certain circumstances, which are defined by guidelines and standards. On the one hand, in case the guidelines target access to treatment to those who benefit most this could be viewed as increasing efficiency. On the other hand, if the guidelines use other criteria to limit the number of individuals eligible for treatment this could also be seen as a dilution of services.

The NICE is the main body defining national guidelines on the use of medicines along with other health care technologies in the NHS. Since January 2002, the NICE guidelines have become a statutory obligation for England and are required to be implemented within three months of publication (Kmietowicz, 2001), resulting in enormous additional financial burdens for PCTs in some cases. Hence, it is likely that guidelines can be only partially funded at first and stepwise implemented. The budget will be taken from existing funding and allocated to new high-profile drugs. On the one hand, this might abolish post-code prescribing for new drugs. On the other hand, it will create post-code prescribing for other health care facilities or drug therapies, since each PCT will decide which health care will be rationed (Burke, 2002). Other criticism came from the pharmaceutical industry claiming that there is a lack of transparency concerning how NICE reached its conclusions and professional groups doubting the independence of NICE from the government and the pharmaceutical industry (Dent and Sadler, 2002; Pharmaceutical Journal, 2002).

The National Service Frameworks (NSFs), which are developed in collaboration with external reference groups including service users, are publications which outline national standards of health service provision including the drug therapy of common conditions (Department of Health, 2001a). They describe what care should be provided, the time scale in which improvements have to be implemented and the performance measures against which the standard of care should be compared. There

have been concerns about how the standards set out in the documents can be implemented within the funding and staff resources available (Pharmaceutical Journal, 2001a).

1.5.3.3 Local guidelines and formularies

Apart from national guidelines, each local health care provider such as a PCT defines guidelines and standards regulating access to the medicines locally. The majority of PCGs planned to introduce prescribing guidelines and many wanted to produce a formulary (Cantrill and Leese, 2001). At present, in the majority of PCTs there is no formulary in use. This is likely to be related to the fact that apart from GPs' budget restriction and financial rewards for practices with cost-effective prescribing, the PCG/PCTs boards have only limited legal influence on the prescribing pattern of their local GPs (Ashworth *et al*, 2002).

It is noteworthy that the majority of PCGs established a prescribing committee, which advises the board – the governing body of the PCG/PCT - about the allocation of funding regarding drug treatment (see Diagram 1.5.1) (Dixon, 2001). A prescribing advisor - a pharmacist or a GP - leads the prescribing committee, organises and manages its work. Although the PCT board has the decision-making authority, in a survey among 72 PCGs in England the prescribing committee was seen as the most influential group determining prescribing policies and targets (Cantrill and Leese, 2001).

To reduce differences between neighbouring PCTs and authorities and to reduce their workload in producing review and recommendation about newly marketed drugs and specialised services other advisory groups were founded at regional level (e.g. New Drug Groups (NDG) and the Regional Specialist Commissioning Groups) (Pharmaceutical Journal, 2000).

In contrast to primary care, in secondary care hospital formularies have been used for decades to promote rational drug use and to limit expenditure (Bochner *et al*, 1994; Khan, 2002). Normally, only the drugs included in the formulary can be used in the hospital, where the Drug and Therapeutic Committees (DTCs) decide about the

inclusion of drugs into the formulary (Khan, 2002). In the current trend of merging the management of primary and secondary care, the influence of the DTCs might decrease since decisions about funding are made on a PCT rather than at the DTC level.

In summary, various mechanisms have been used to regulate access to medicines: excluding medicines from reimbursement, reducing the demand, increasing the efficiency and reducing the number of patients eligible for access to treatment. Over the last years guidelines have been used increasingly as a tool to regulate access.

1.6 Information basis for decision-making about access to treatment

The previous part described each of the main decision-making committees in the NHS and their role related to decisions about access to medicines. In the following part some aspects of the process of decision-making will be highlighted. In particular, the question of the type of information used as a basis for decision-making and the techniques used to gather and to analyse the evidence used to inform the decision-maker will be addressed. Table 1.6.1 gives an overview of the common types of information inputs into decision-making (Mitton *et al*, 2003).

Table 1.6.1: Common types of information inputs (adapted from Mitton *et al*, 2003)

Information inputs	Example
Research data used from pre-licensing trials	Randomised controlled trials (RCTs) designed for the requirement of the licensing process
Empirical research of the technology under trial conditions	Case control study of the clinical effect of the technology
Outcome data on the use of the technology in practice	Drug utilisation
Health technology assessment (HTA)	Technology assessment review Health technology assessment systematic review
Needs assessment	Epidemiological studies in the prevalence and incidence of a disease
Budget information	Total budget available
Guidelines and policies	National standards such as National Service Frameworks (NSFs)
Submission from stakeholders	Patient organisations' statement
Formal ranking system	Jadad scale (Jadad *et al*, 1996)

1.6.1 Research data from pre-licensing trials

One of the information inputs into decision-making are the results of research carried out prior to the licensing of the medicine. In order to understand the nature of the research data and its limitations it is necessary to say something about the licensing process in general. The research before market authorisation aims to provide data about the safety, efficacy and quality of the product. The process of data gathering for the application of market approval can be divided into four parts, in which the drug is tested in animals and later, in an increasing number of healthy volunteers or patients (see Table 1.6.2) (Salt, 2003). Whereas the first two phases are mainly used to determine safety and dosage, the next two aim to assess the efficacy and adverse effects of the substance.

Table 1.6.2: Drug testing before market authorisation

Phase	Subjects	Purpose
Pre-clinical/pre-human testing	Laboratory and animal studies	Assessment of safety and biological activity
Phase I	20-100 healthy volunteers	Assessment of safety and determination of dosage
Phase II	100-300 patient volunteers	Evaluation of efficacy and side effects
Phase III	1,000-3,000 patient volunteers	Evaluation of efficacy and monitoring adverse effects

The agencies approving a substance for marketing have the task to protect the population from harmful drugs and at the same time to make useful therapeutic agents available as soon as possible, which is in line with the interest of the applicant from the pharmaceutical industry. Both the number of individuals on which the drug is tested and the time available for the tests are an important limitation of the data.

The studies in phase II and III with the purpose of determining the efficacy of the drug – the effect of the drug under ideal condition – commonly compare the new substance against a placebo. Efficacy includes the size of effect and its probability (the likelihood of the effect occurring). The definition of the desired size of effect, which a substance needs to show in order to obtain marketing approval, may be difficult (Koopmans, 2002). This is related to the fact that it is commonly determined by using so-called surrogate end points, which are intermediate outcomes that are believed to result in the desired effect later on. For instance, if a drug is intended for the treatment of arrhythmias, the suppression of the arrhythmias as a surrogate marker is used instead

of survival, based on the assumption that suppressing the arrhythmias will eventually result in a survival improvement (Fleming *et al*, 1996). Importantly, the use of surrogate end points is one important limitation of the data used to obtain market approval, because they sometimes fail to predict the desired health outcome (Psaty *et al*, 1999). For example, trial data showed that the suppression of arrhythmias resulted in higher mortality. Hence, the drug may have obtained market authorisation but does not show the assumed long-term benefit.

Another limitation is the assessment of safety. Only the side effects which are occurring with a certain probability can be detected due to the limited number of volunteers on which the drug has been tested. Ferner (1996) calculated that a side effect that may affect around 1 in 500 patients could be missed out assuming that the drug has commonly been tested in about 1,500 volunteers. Hence, the data gathered for the application of the market authorisation is limited in its predictability regarding effectiveness and safety in a large population.

1.6.2 Data from health technology assessments

At the time of the market authorisation, the only data published or partly available are often gained from phase I, II or III clinical trials, which are designed for the requirement of the licensing process. Other primary research, which is carried out independently from the licensing procedure, is commonly sponsored by the applicant or later the holder of the market authorisation, who is prepared to fund the expensive research. Most clinical trials are limited to six months, which means that only short-term effects of the drugs can be investigated (Psaty *et al*, 1999). This is in contrast to the demands of the policy-makers who aim to determine long-term effects of new innovations.

In the past individual experience in using the technology and clinical trials were regarded as the primary information sources for decision-making, sufficient to make a judgement about the benefit of the intervention (Bloom, 1986). This has changed dramatically over the last twenty years. Nowadays, secondary research is one of the most important information inputs in the decision-making. One argument was that in order to understand the impact of a new medicine or any other technology,

comprehensive secondary research needs to be carried out gathering and appraising or evaluating primary sources of information about the short and long-term consequences (Banta, 2003). Another argument was based on the recognition of the increasing number of complex technologies with unknown beneficial and harmful effects and constantly rising costs (van den Heuvel *et al*, 1997). Starting in the US in the beginning of the 1970s, health technology assessments (HTAs) were used to inform policy-makers (Stevens *et al*, 2003). These assessments included the evaluation of primary research data of the clinical effects of the technology as well as economic evaluations on the likely cost impact of the technology on the current funding structure. The advantage of the assessment was thought to be the inclusion of a broad range of factors such as the organisation, management and delivery as well as social and ethical aspects possibly influencing the effect of the technology in practice in addition to the clinical ones (Fulop *et al*, 2003).

This development went along with two other important movements of gathering mainly clinical research data and evaluating its use. One of the movements is the Cochrane collaboration, going back to Archie Cochrane's work on effectiveness and effect in health care (Cochrane, 1972). The Cochrane collaboration argues, "the only way to build an adequate understanding of the effectiveness of different interventions is through systematically locating and synthesizing the available evidence from research" (Chalmers, 1992). The Cochrane collaboration establishes literature reviews of a wide variety of health interventions according to a certain set of rules. To determine the effect of an intervention the randomised controlled trial (RCT) was advocated as the primary tool. Evidence-based medicine (EBM) is the other movement, which aims to affect medical practice by integrating individual clinical experience with external clinical evidence from systematic search and appraisal of the evidence (Sackett *et al*, 1996). This systematic research for instance could include a Cochrane review to inform clinical practice.

All of the three movements – using HTAs, the Cochrane collaboration and EBM - have in common the systematic search and assessment of the information according to certain defined rules. For instance the Jadad Quality Score is used to accredit trials with certain points according to their trial design and data analysis (Jadad *et al*, 1996). The lower the total number of points allocated the higher the likelihood of bias in the

RCT and the more uncertain the predicted outcome of the trial. The form of information is ordered in a hierarchy where meta-analysis and RCTs are given higher priority over case control studies or case reports as it is thought that they have a higher certainty in predicting the effect of the intervention.

1.7 Empirical studies investigating authoritative decision-making about access to medicines

Since there is no consensus about the principles which guide the decisions about access to medicines, the study of the actual process becomes relevant in various ways. Which are the principles guiding decision-makers and in what situations are they relevant and why not in others? What other influencing factors guide policy decisions about access to medicines? What are the most valuable information sources for making decisions in practice?

A systematic search of MEDLINE, EMBASE, PubMed, International Pharmaceutical Abstracts (IPA) and Pharmline as well as search of two large library collections – the King's Fund library and the British Library - holding relevant health policy literature has been performed. A range of key and title words were used: "medicines", "drugs", "new product" "formulary" in combination with "decision-making", "health policy", "access", "rationing", "priority setting" or single terms such as "drug and therapeutic committee" or only "committee" depending on the scope of the database. Empirical research of the last twenty-three years investigating actual or perceived decision-making process about access to medicines within a health care system was included. Excluded were studies which used hypothetical decision-making scenarios or investigated evaluation of the decision-making after an intervention such as the educational training of the decision-makers or the introduction of a certain decision-making framework. Studies only focusing on the use of economic data in the decision-making process were also excluded.

In addition, a hand search of journals relevant to health policy was carried out such as *Health Policy, Politics and Policy, Health Economics, International Journal of*

Technology Assessment in Health Care, *Journal of Politics, Policy and Law* and *Journal of Health Service Research and Policy*. The issues of the last five years were searched.

In total, 30 empirical studies of institutional decision-making about access to medicines meeting the inclusion criteria were identified. The majority of studies have been carried out in the US investigating the structure, organisation and process of decision-making in Pharmacy and Therapeutic (P & T) Committees of various institutions. As in the UK, in other countries the use of formularies determining the drugs which are provided within hospitals, has also been well established for decades (Summers and Szeinback, 1993; Weeks and Brooks, 1995; Shalansky *et al*, 2003). Hence, the number of studies focusing on hospital formulary committees exceeds the ones investigating other institutions. Over recent years Maintenance Care Organisations (MCO), that means health insurance organisations, and other long-term care providers in the US have adopted the use of formularies. Only the drugs included in the formulary are fully or partly reimbursed (Sheperd and Salzman, 1994). Despite the number of studies investigating the structure and the organisation of the committees in various institutions, only some explore the interplay of decision-makers, their interests and the weighting of criteria determining the decision. The following part summarises the findings of these studies carried out in different countries using various methodologies. Studies of decision-making about inclusion of a drug in a hospital formulary or in another health care institution will be discussed, followed by research into decision-making about access to medicines in HAs or provincial committees not necessarily using a formulary.

Most of the studies in the field of hospital formulary management focus on economic aspects such as cost containment strategies rather than exploring the actual entire decision-making process. As mentioned before, these studies were excluded. The majority of studies included in the literature review used surveys to analyse the structure and functions of the committees. For example, Manneback *et al* (1999) in their survey of 187 hospitals found that the majority of US hospital formulary committees had a high percentage of clinicians as their members with an average of around 70% of the vote. The main activity of the committees was to review or approve new drugs (one third of their time) with a mean of 18 drugs approved and 16 drugs

excluded per year. As in the US, also in Australia and in the UK a physician chaired the majority of the committees (Weeks and Brooks, 1996; Leach and Leach, 1994).

Apart from the description of the structure, functions and organisation of the committees the surveys asked the participants on what criteria decisions for acceptance of new drugs on the formulary were based and the relative priority given to these criteria. The majority of the studies uniformly report that the decision-maker regard efficacy, safety, cost containment and treatment alternatives as the predominant factors on which the decisions should be based (Collier and Foster, 1985; Segal and Pathak, 1988; Joshi *et al*, 1994; Weeks and Brooks, 1996; Lyles *et al* 1997). In addition, some studies investigated the sources of information used for making formulary decisions (Luce and Brown, 1995; Formulary, 1995; Lyles *et al*, 1997; Shalansky *et al*, 2003). Data from peer review journals including RCTs, regional guidelines and decisions at peer hospitals and local opinion leaders ranked highest (Formulary, 1995; Lyles *et al*, 1997; Shalansky *et al*, 2003).

Some studies show a change in the pattern of criteria importance over time. For example, two large US surveys among P & T committee members from a variety of institutions were carried out in 1995 and 2001, where data were analysed from about 300 returned questionnaires in each case (Formulary, 1995; Campbell and Sprague, 2001). The majority of responders in both surveys were members of hospital P & T committees; others were from MCO, long-term care providers, government agencies or other health care providers. Interestingly, whereas in 1995 14% of the respondents stated that they tried to use quality of life information or that they plan to use this information, this changed significantly six years later. In 2001 36% of the respondents stated that the impact of the drug on patient's quality of life would always be taken into account.

Although the studies found a high degree of similarity of the main criteria used for decision-making about acceptance on the formulary, there were large discrepancies between the drugs included in the formularies of different organisations (Rucker and Visconti, 1976; Manneback *et al*, 1999). Some studies explained this difference was attributable to the case differences in each of the hospitals (Manneback *et al*, 1999), the variation in the committee composition with its political dynamic (Rucker and

Schiff, 1990) or the difference in considering factors such as the attributes of the products, for example user convenience (Sheperd and Salzman, 1994). Interestingly, in relation to a report from 1985 about the activity of a drug and therapeutic committee in one UK health authority (HA) admitted that the committee "sometimes included a drug because of its traditional use and popularity, rather than because there was published evidence for its efficacy" (Collier and Foster, 1985). Similarly, in an interview with three members of a P & T committee of a large teaching hospital in the US they admitted that the members of the committee were sometimes found to be susceptible to the pharmaceutical advertisement (Quintiliani *et al*, 1990). The case examples given during the interview highlighted that de facto in some cases the cost considerations overruled the safety of patients in relation to side effects and patient compliance, even though safety aspects were stated to be more important than cost implications.

None of these studies explored the actual instead of the perceived importance of each of the criteria in determining the decision which drug go on the formulary and the process of decision-making in depth. One recent study investigated the relationship between certain organisational features of HMOs and their decisions regarding seven innovative drugs (Dranove *et al*, 2003). The authors concluded that the main determinants for the formulary adoption decisions were the size of the committee (large committees adopted fewer drugs), the incentives of the pharmaceutical director and the frequency of visits by the pharmaceutical company representative. More innovations were accepted in the cases where pharmaceutical companies representatives made more visits (compared to other HMOs which were visited less frequently) and where the pharmacy directors of the committees are rewarded according to the medical instead of the pharmacy costs.

Only two studies were identified which used observational methods to further investigate the decision-making process in formulary committees and the interaction of the decision-maker in more detail. In a Canadian study Martin *et al* (2003) combined document analysis of the minutes of drug and therapeutic committee meetings of three hospitals of the same network with the analysis of 18 interviews with committee members and observation of three meetings to describe the decision-making process. They identified that a cluster of factors was influencing the decision where costs were not the determining, but a relevant factor. Evaluating the process by using the

framework of accountability (Daniels and Sabin, 1998) as a standard showed that lay members should be included in the committee and that the transparency of the committee could be improved by publication of the committee minutes describing the final decisions and the reasons for those decisions.

Whereas Martin *et al* (2003) concentrate more on the evaluation of the decision-making process and neglect the detailed description of how each of the factors are weighed in the decision-making process, an observational study from the UK gives an interesting insight into this process (Jenkings and Barber, 2003). They observed and tape-recorded four drug and therapeutic committee meetings from two general hospitals respectively and found that evidence from formal outcome data of clinical trials were complemented with various other types of evidence in the decision-making process. These were for example the pharmaceutical companies' activities, the decisions of other drug and therapeutic committees, the clinicians' excitement and the personality of the applicant. All of them had considerable weight in the final judgement.

Other studies retrieved from the literature review investigate decision-making about access to medicines other than in the context of formulary committees. One of the first empirical studies about decision-making in the newly structured English HAs after they became responsible for the provision of health care within their region was carried out by Ham (1993). The interviews of professionals from six HAs showed that due to the difficulties of comparing health services between different medical specialities it was preferable to analyse priorities within a service area. There were attempts to involve the public in decision-making and to rank services according to priority points allocated. The main principles for the allocating of points varied between the HAs. Whereas need was the main determining factor in one HA, in another it was a mix of utility, improvement of the quality of service and principles according with local views. Importantly, the interviewees highlighted that there was an absence of information to guide them in making priority decisions. The study was carried out at a time when decision-making about access to health care was new for policy-makers on a regional level, and they were likely to have little or no experience in making those decisions.

Five years later, Hope *et al* (1998) reported that there is still a lack of guidance for regional policy-makers in making decisions about access to health care. They reported about their experience concerning decision-making in one HA in England, where a special committee advised on rationing decisions using three main criteria determining the decisions: effectiveness, equity and patient choice. The last of these criteria aims to ensure that the patients can choose between treatment options of similar efficiency. Whereas Ham (1993) reported about the difficulty involved in comparing between interventions provided in different health service areas, the HA in Hope *et al* (1998) suggested comparing interventions which have similar effect, for instance life-extending interventions in oncology and neurology without curative effects. Although the report uses the decisions about access to two drug treatments (riluzole and isotretinoin) as an example, it gives no details about the factors other than effectiveness, equity and patient choice, which potentially influenced decision-making.

Foy *et al* (1999) reported their experience of priority setting for oncology treatment in a consortium of six HAs in the United Kingdom. They described that the quality and amount of evidence about clinical outcomes of treatment were the main influential factors on reimbursement. They concluded that there was a certain cut-off point under which the available information about effectiveness of information was regarded as insufficient to justify funding. This threshold was influenced by the values placed on the treatment outcomes and certain political and financial considerations. The data of the study are limited to oncology treatments and decisions made within one financial year.

This is distinctive from the data gained by Martin *et al* (2001) and Singer *et al* (2000) who observed priority setting committees deciding about oncology and cardiology treatment in Canada's publicly funded health care system. In addition, they conducted interviews with the members of each committee. They found that there were a number of factors influencing the decisions. The patient's benefit as the main determining factor was combined with others such as the presence or absence of alternative treatments, the total population of patients affected or the total cost of the system and the quality of the evidence. In contrast to previous proposed trade-offs, they argued that the factors were interrelated and contributed to the complexity of the decisions.

Another empirical study (McDonald *et al*, 2001) investigated the perception of 21 pharmaceutical advisors on the entry of new drugs in England. In using semi-structured telephone interviews the researchers found a discrepancy between the goals of the decision-making as stated by the advisors and the actual management, where cost containment was the main influencing factor for the decisions. Success of the strategy of cost containment was assessed by determining the volume of prescriptions. A participant observation confirmed that an explicit and systematic approach to allocation decisions was hindered by the decision-makers' immediate self-interest and a lack of public perspective (McDonald, 2002). Immediate, measurable objectives were preferred over long-term goals.

In summary, the majority of studies used surveys to investigate primarily the function and organisation of the formulary committee and included questions about the perceived importance of certain factors determining the adoption of drugs to the formulary. It is striking that despite the similarity in the declared importance of efficacy and cost impact of the decision as determinants for the acceptance of the drug, the decisions between the committees varied considerably. The observational studies carried out illustrate that there are several organisational and political factors, which reveal a much more complex picture of decision-making about access to treatment.

Before discussing the implication of previous work on the design of the present study, the theoretical and empirical background of individual decision-making about medication will be addressed.

CHAPTER 2: THEORETICAL AND EMPIRICAL WORK ON INDIVIDUAL DECISION-MAKING

2.1 Introduction

The following chapter will address the individual decision-making about medicines between health care professionals (HCP) and individual patients. Although other HCPs are involved in the care of a patient, primarily the role of the physician as the HCP treating the patients will be analysed. In investigating informed consent (IC) for medicines the role of nurses as HCPs frequently involved in medication administration will also be discussed. However, focusing on decision-making about access and choices, the physician has traditionally been the professional in charge of the prescribing task. Prescribing a medicine for the patient can be understood as giving the patient access to the medicine. The patient-physician decision-making is a way of deciding about access to health care on an individual level. It means that the physician can be seen as gatekeeper in a unique position to decide who is given access, to which type of care and to how much.

Regarding the decision-making models, which will be discussed in the following, it needs to be pointed out that all of them have been developed in European or North American medical culture. Hence, they evolved from a specific traditional value concept, which may limit their application to other cultures.

There has been an increasing demand for patients' involvement in decision-making regarding their care, fostered by recent policy movement nationally and internationally (Department of Health, 2000a; World Health Organisation, 1994). This chapter presents various forms of physician-patient decision-making, in particular models where the patient has a role in decision-making. One of the oldest and most explicit ways to implement patients' involvement has been the requirement to obtain IC, which has been used traditionally in research and for invasive procedures. Currently, there is - apart from the legal requirement and political enforcement - also a strong moral argument to use IC for all health care interventions (Sharpe and Faden, 1998). In this chapter the concept of IC including its theory and application will be described. The

focus will be on the use of IC in therapy, in particular for medicines, compared to its application in research.

This will be followed by a discussion of other decision-making models and concepts of patient-doctor interactions, which have been developed more recently than the concept of IC. In addition, an overview will be given of the findings of empirical studies, which investigated the extent of the actual and perceived decision-making about medicines in a patient-physician consultation, the patient preferences of their role in decision-making, the potential barriers to participation and its impact on treatment outcomes. Finally, the chapter will close with the implication of the previous studies in the field of health policy and individual clinical decision-making for the design of the research presented in this thesis.

2.2 Theory and empirical studies of IC

This part discusses some of the philosophical and practical conditions for IC, its limitations and the empirical literature about IC, focusing on its use for non-invasive therapy with medicines.

2.2.1 Theory of IC

Consent stands out from other decision-making models in the fact that it offers protection for HCPs against litigation and is supported by strong ethical arguments from recent authors (Beauchamp and Childress, 1994; Sharpe and Faden, 1998). This contributed to the fact that today IC is one of the best-developed and most practiced models of patient-physician decision-making about health care. In the following its historical development, the ethical arguments and its implication for practice will be addressed.

2.2.1.1 Historical development

In their well-known work "The theory and history of informed consent" Faden and Beauchamp (1986) saw IC as a creation of law rather than ethics. Case law shaped the preconditions, which need to be met in order that the patient gives his or her valid

authorisation to the health care intervention: the person needs to have the decision-making capacity and understand the relevant information disclosed, to act without influence of another party which would determine the outcome of the choice and to give an intended authorisation (Faden and Beauchamp, 1986). The consent can be expressed in written, verbal or non-verbal form.

Beauchamp and Childress (1994) distinguish between three different forms of non-verbal consent: implied, tacit and presumed consent. Implied consent is inferred from the action of the patient, for instance the patient raises his or her arm to receive an injection. Tacit consent is expressed by omission of refusal or protest. However, both forms of consent are only valid if all the criteria mentioned above are met, in particular the patient needs to be informed about the consequences of his or her permission. Some authors warned that non-verbal consent is open to misinterpretation as it is assumed that the patient – understanding the consequences of his or her action – gave permission (Kennedy and Grubb, 2000). Only the protest of the patient is a clear sign of his or her disapproval. Implied and tacit consent needs to be distinct from presumed consent, which does not depend on the patient's words or action and is 'presumed' either on the basis of the knowledge about an individual patient preferences or on the grounds of human good. Commonly, 'presumed' consent applies to emergency situations where it is assumed that the patient wishes to be treated though the patient could not express his or her will (Childress, 1982).

Early court cases focused on the aspect of battery, which means touching the patient without his or her permission, which was seen as violation of the right of privacy. In order to obtain the valid permission of the patient before touching, the patient needed to be informed about the nature of the intervention. It was not until 1956 that the term "informed consent" was used instead of consent. In this case the Californian court argued that, in order to give permission to the proposed health care intervention, the patient need to receive sufficient information. The case highlighted the physician's duty to disclose information not only about the nature of the intervention, also about risks and alternatives. However, it was the Canterbury versus Spence case in 1972, that the US court explicitly stated that the physician would neglect his or her duty if s/he would not disclose all the information required to enable the patient to make an "intelligent decision" (Canterbury versus Spence, cited in Faden and Beauchamp,

1986). The protection of the patient's right for self-determination became the core function of IC. Battery and negligence are still the guiding notions of English case law regarding IC (Department of Health, 2001b).

For many years, the 'professional standard' guided the judgement of British courts (O'Brien, 1986; General Medical Council, 1998). The professional standard means that in case of a court appeal a physician would decide if the information disclosed by his or her colleague was adequate. It has been argued that the legal duty to disclose all relevant information according to a 'professional standard' is not necessarily safeguarding the moral duty to enable an individual patient to decide about their health care (Braddock, 1998). Therefore, the 'patient standard' or recently the 'subjective standard' has been suggested as the only valid way to decide about the appropriateness of the information disclosed, where the information is targeted toward the need of the individual patient, not a hypothetical "reasonable" patient (Skene and Smallwood, 2002; Marks, 2003).

There is some agreement about the circumstances in which the legal requirement to obtain IC can be overridden (Faden and Beauchamp, 1986): Public health emergencies, medical emergencies, incompetent patient, patient waiver of the right of IC and the therapeutic privilege. In the case of patient waiver the patient has explicitly expressed the wish that the physician makes the decision for him or her. The therapeutic privilege means that the physician withholds information in order to protect the patient from potentially harmful effects of this information.

However, there are problems with the definition's interpretation and debate about when these exceptions apply in practice. For example, debate arises about a valid test in order to define whether a patient is competent or not. A publication of the British Medical Association and the Law Society (1995) gives advice on the assessment of competence. Some patients may be competent to give consent in some situations but not in others (Department of Health, 2001b). According to the Mental Health Act 1983 the refusal of consent by patients with mental disorders can be overridden (Oates, 2000). In cases where patients are incompetent to give consent it should be assured if possible that the paternalistic action is consistent with their preferences and values. This is usually done by asking relatives, or if unknown, the HCP to decide what is

assumed to be in the individual's best interests, which is called the "Principle of the best interest" (Oates, 2000). This is also the principle governing the decision in the case of a child. In case law the ability of "sufficient understanding and intelligence" determines the capacity to give consent at all ages.

The therapeutic privilege also creates controversy. It is questionable how the potential harm can be measured in an objective way and psychological damage predicted (Faden and Beauchamp, 1986). The authors argue that in all these exceptional cases, where no consent is obtained and the choice of the patient is overridden, other values must be weighed higher in order to justify this action.

Advance directives are different from IC in the way that they state permission or treatment refusal for specific situation, which may happen in the future. For example, it is possible, for a patient recently diagnosed with cancer and competent, to refuse all life-prolonging treatment in advance. If a situation arises, in which this patient is incompetent, the prior refusal of treatment has to be respected (Department of Health, 2001b).

2.2.1.2 Ethical argument

The moral dimension of consent was not realised until about 1970 where the self-determination of the patient was acknowledged as a core function of IC. Before, consent was a legal rather than moral requirement. The concept of IC, in the sense of the right for self-determination, was seen as incompatible with good patient care. In order to understand the development of the concepts of IC and other models of patient-doctor interaction it is helpful to highlight some of the historical changes in the way medical decision-making is viewed.

For centuries, medicine was practised in the belief that the individual as a patient is exempt from the duty to take responsibility for their action (Jonsen, 1998b). In addition, it was argued that the disclosed information about the condition of the patient and the proposed treatment could unnecessarily harm the patient; caring meant that the physician would decide in the best interest of the patient, largely without disclosing

any information, which was thought to generate fear or anxiety in the patient (Pelligrino, 1992).

In the middle of the last century historical events resulted in a new perspective of medical decision-making. This was partly triggered through the crimes committed by physicians carrying out research on patients during the Nazi regime in Germany, which resulted into the Nuremberg code requiring, among other things, IC before the patient undergoes any research carried out during care (Shuster, 1997). In addition, the technical advance of medicine was perceived as a threat to patients, where they were exposed to unknown risks and largely seen as subjects (Illich, 1976). This resulted in questioning the overall benefits of medical interventions. It was found that the views about the benefit of the intervention might differ between the physician and the patient, which is regarded as the premise for IC (Faden and Beauchamp, 1986). Following on from this it was argued that in addition to the physician, the patient also needs to decide about the value of every health care intervention for therapeutic as well as for research purposes (Sharpe and Faden, 1998). The patient's right of self-determination, which is grounded in the view of the patient as a person, became the central justification for IC. Instead being a subject either in research or in therapy, there was a call to protect the individual rights of the patient as a person, with the physician's duties of loyalty, fidelity and mutuality (Ramsey, 1970).

2.2.2 IC for medicines

The change in the purpose of IC, from protecting the patients' privacy and protection from interference in their bodily integrity towards enabling the individual to make an informed decision, meant that there was a strong ethical argument to obtain IC for *all* kinds of health care interventions, including writing prescriptions and administration of medicines (Ubel, 1996). Despite the strong ethical argument, there is considerable variation for which medicines in clinical therapy a formal process of obtaining IC is used. In the following section it will be argued that the application of obtaining IC for medicines is patchy and depends on local circumstances. In recent years, the professional and governmental agencies have addressed this issue and published nationwide guidelines defining an ethical standard regarding IC for all health care intervention.

There are some groups of therapeutic medicines, for which the use of written IC has been recommended and which have mostly been discussed in relation to IC. These are chemotherapy and antipsychotics (Lilleyman, 2002; Brabbins *et al*, 1996). Apart from therapeutic medicines, written IC has been increasingly used for diagnostic agents (e.g. radiological contrast substances), vaccines and anaesthetics (Hopper and Matthews, 1993; Charles and Lewis, 1994, Watkins *et al*, 2001). Additionally, there are companies who recommend the prescriber to obtain written IC for some of their products, for instance for the use of roaccutane in female patients of childbearing age (Roche Pharmaceuticals, 2001). All of these drug groups have potential serious or irreversible side effects; some are also associated with visible changes (e.g. alopecia with chemotherapy) or personality influencing effects (e.g. with antipsychotic drugs). However, there is considerable variation for which medicines a formal process of IC is used, depending on local policies rather than national or internationally agreed conventions (Rich, 2001). Hence, for medicines with similar severity of side effects such as amiodarone or warfarin no formal process is commonly used. That means patients who take such medicines give their *implied* consent *provided that they have been adequately informed and understand the consequences.*

Commonly, the responsibility to inform the patient about the medicines and their effects is shared between the pharmaceutical companies, regulatory agencies and the HCPs prescribe, dispense or administrate the medicines (Herxheimer, 2002). Despite the patient information leaflets ("labels") – issued by the holder of the market authorisation - giving detailed information about benefits as well as all potential side effects of the medicines, they do not provide information about treatment alternatives which is one requirement of IC. Therefore, it is the HCPs' responsibility to inform the patient about the treatment alternatives as well as to ensure that the patient is competent, has understood the facts disclosed and acts voluntarily.

A formal process of IC has been specifically advocated for the administration of medicines used outside their licensed indication (off-label use) or for unlicensed medicines where safety, efficacy and quality have not been approved by the governmental agencies. In other words, these medicines may be associated with increased harm, for which the manufacturer is not liable. In addition, there is usually an absence of a printed patient information leaflet informing the patient about potential

effects of the medicine compared to licensed medicines where the manufacturer is legally required to provide this information. Instead, the full liability for any harm lies with the prescribing or administrating HCP who has the duty to inform the patient and to obtain IC (Nursing and Midwifery Council, 2002). Recently, Wright (2002) pointed out that the unlicensed administration of medicines includes the opening of capsules and crushing of tablets.

In the last five years national HCP organisations (e.g. the General Medical Council (1998), the Nursing and Midwifery Council (2002)) and the Department of Health have issued standards on the use of IC. For instance, the former United Kingdom Central Council for Nursing issued guidance on the administration of medicines, which specifically addresses the question of IC for medicines (UKCC, 2001). It stresses that patients have to give IC for every medicine administered by nursing staff. The guidance continues, "For that agreement to be effective, the patient or client must have been given adequate information about the nature, purpose, associated risks and alternatives to the proposed medication". (The position of the UKCC was adapted by the Nursing and Midwifery Council in 2002).

In this context, the disguised administration of medicines and its ethical and legal implications has been widely discussed (Cayton, 2000; Dewing, 2002). The UKCC took the position that this is regarded as deception and only acceptable in cases where the patient is not able to give consent and the action is discussed and agreed by all the HCPs caring for the patient. In addition, "such treatment must be necessary in order to save life or to prevent a deterioration or ensure an improvement in the patient's or client's physical or mental health" (UKCC, 2001).

Nursing prescribing guidelines specifically refer to the use of IC and stresses, "Ensure you have discussed alternatives with your patient and what would happen if they did not have any treatment" (Lewis and Allen, 2002).

2.2.3 Empirical studies of IC

The application of IC for all kinds of health care interventions raised the question of how well this is implemented into practice. The criteria of IC – competence, non-

coercing, information disclosure, understanding and authorisation – are standards against which practice can be evaluated. In order to investigate how well the standards are applied regarding the prescription and administration of medicines a literature search was performed. Databases including MEDLINE, EMBASE, PubMed, International Pharmaceutical Abstracts (IPA), Pharmline and Zetoc were searched using the keyword or title word "informed consent" and "consent" either alone or in combination with either "medicines" or "drugs" for a period between 1966 and 2003. If the database did not go back that far it was searched for the maximum period of time. A hand search was conducted of the journal *Patient Education and Counselling* for the last ten years as this periodical was of particular relevance to the study area. The catalogue of the University of London Library and the British Library were searched for theses and conference abstracts using the keyword or subject word "informed consent" and "consent". Included were studies which specifically referred to the concept of IC in relation to routine therapy with medication, and which were carried out in countries with a relatively similar culture compared to the UK (such as Europe, Northern America, Australia and New Zealand). It was the aim of the review to study IC in competent adults and exclude aspects of IC related to decision-making capacity. Therefore, research studying the assessment of capacity and related issues, IC in children and the problem of patients whose condition is believed to affect their ability to give IC, such as psychiatric patients or Alzheimer's disease patients, were excluded from the review.

The empirical studies of IC fall generally in five main categories:

- assessment of the capacity to give IC (this category of studies was excluded for the reasons mentioned above),
- information disclosure,
- patients' recall and understanding of information disclosed after obtaining IC,
- impact of IC on the patient, and
- policies and procedures of obtaining IC (for instance the procedures for which the use of written IC has been made obligatory by the health care provider).

Some studies address more than one of the categories.

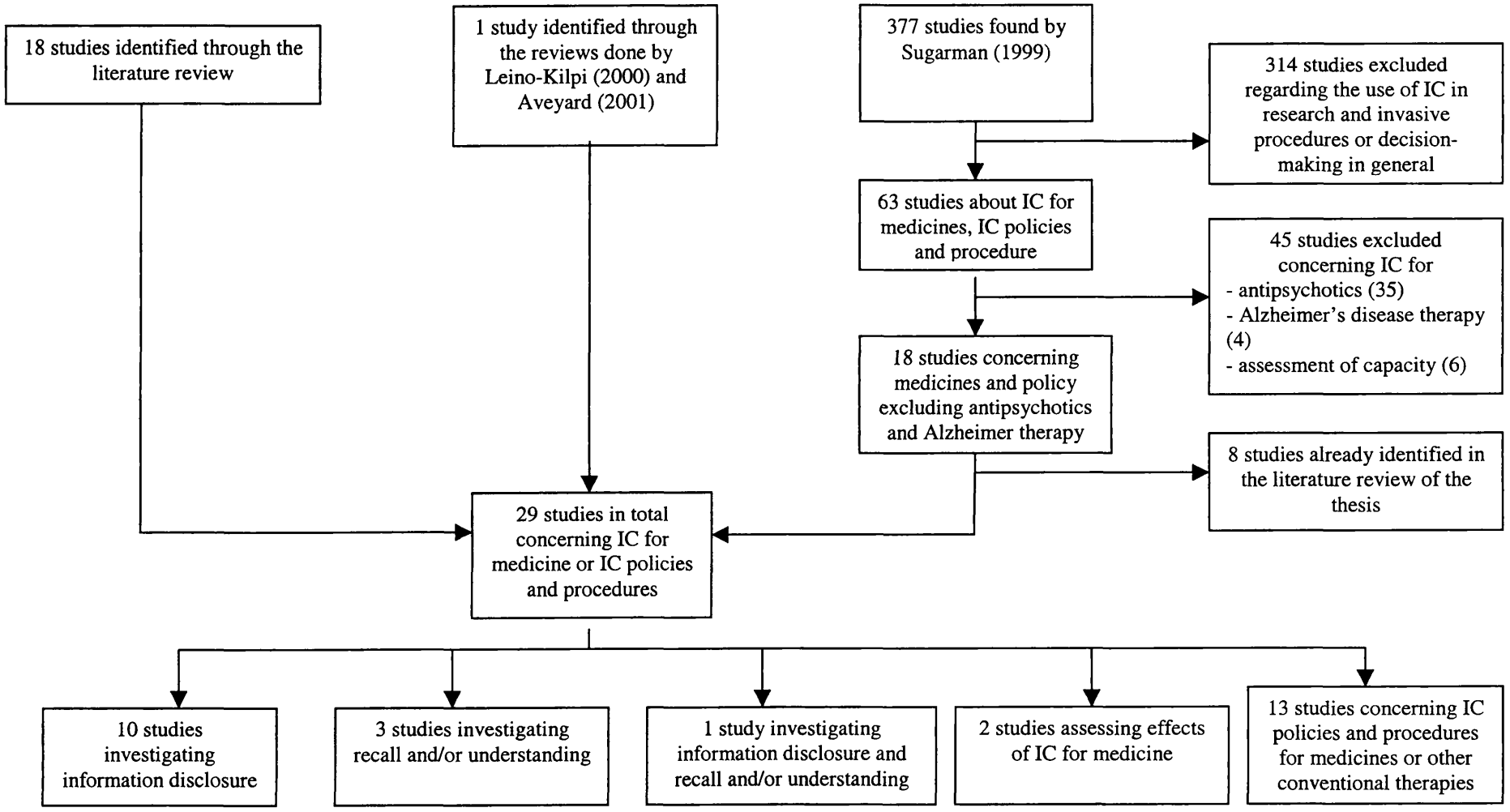
Two literature reviews from the US on empirical studies of IC (Kaufman, 1983; Sugarman *et al*, 1999) and one from a European group with nursing research

background (Leino-Kilpi, 2000) have been identified. Whereas the review by Kaufman (1983) included patient decision-making in addition to IC, Sugarman *et al* (1999) and Leino-Kilpi (2000) concentrated on empirical studies of IC. In addition, Aveyard (2001) performed a literature search of empirical studies of IC prior to nursing care procedures. All reviews were searched for empirical studies complementing the literature search performed for this thesis which, in contrast to the other reviews, focused on conventional therapy regarding medication. Diagram 2.2.1 summarises the number and categories of publications identified. The review by Sugarman *et al* (1999) has been used to illustrate the proportion of studies on each study area. The Diagram 2.2.1 shows that the large majority of studies investigate IC in a research context, a finding that was confirmed by the other reviews as well as the review performed for this thesis. For example, no studies were found regarding IC prior to routine nursing care procedures (Aveyard, 2001). Studies concerning IC for conventional therapy are mainly concerned with the use of written IC for invasive procedures such as surgery or diagnostic interventions compared to the use of IC in non-invasive care.

A considerable number of studies were carried out in the beginning of the 1980s, at the time of the President's Commission for the Study of the Ethical Problems in Medicine and Biomedical and Behavioral Research (PCEMR) (1982), which commissioned several studies on IC. For example, two large surveys were carried out including 805 physicians and 1,257 members of the general public concerning their views on IC and its use (Harris *et al*, 1982). The findings showed that only around one quarter of the physicians thought IC entails the patient giving permission or agreeing to something. For most IC meant disclosure of information about benefits and risks. The public and physicians' perception deviated regarding the common procedure of information disclosure. Whereas 93% of the physicians reported that they would always disclose the risks of the therapy, only 68% of the public thought so, although 94% of them wanted to be told everything.

About ten years later, another survey of 1,000 members of the general public in Canada (Singer *et al*, 1993) showed similarly that 97% of the participants thought that the physician should disclose all the information before they undergo treatment. However, 33% believed that certain information should be withheld from patients upon families' wishes.

Diagram 2.2.1: Empirical studies of informed consent (IC) for medicines in routine therapy, IC policies and procedures



Most of the studies investigating IC for routine therapy with medicines focus on the aspect of information disclosure and understanding. The majority of those used questionnaires to either test the patients' recall and understanding of information disclosed (Muss *et al*, 1979; Cassileth *et al*, 1980a; Dodd and Mood, 1981; Olver *et al*, 1995) or the patients' or physicians' view on the information disclosure practice (Williamson, 1980; Faden *et al*, 1981; Shriner *et al*, 1992; Singer *et al*, 1993; Scott *et al*, 2003). Regarding the assessment of the patients' recall, a study by Olver *et al* (1995) for example examined the extent of comprehension and recall of information about the benefits and risks of conventional chemotherapy in 100 patients within 28 days after signing the consent form via questionnaire. They found that 26 patients did not know the goal of the therapy. Regarding the consent form only 34 understood the purpose of it. This is similar to the findings of Cassileth *et al* (1980a) and Muss *et al* (1979) who found that patients had difficulties understanding and recalling the information or had not read the consent form carefully. In addition, around one third of the patients did not understand the purpose of the treatment, for instance 35% of oncology patients thought wrongly that their treatment was curative instead of palliative (Muss *et al*, 1979). Some authors have called for more caution in using patients' recall as a method to assess the understanding of information disclosed (Sulmasy *et al*, 1994; Doyal, 2001). They argue that it is very likely that patients will be unable to remember details of the information disclosed and that the amount of information recalled may not reflect their actual understanding. The literature review showed that the time between the patient signing the IC and the assessment of the recall varied significantly from one day (Cassileth *et al*, 1980a) up to two years (Muss *et al*, 1979).

Other studies investigated the patients' and HCPs' views on information disclosure regarding IC in clinical practice. For example, Scott *et al* (2003) asked residents and nurses in Scottish nursing homes about the use of IC for routine health care interventions such as medication. They found a large discrepancy between the two groups of 101 residents and 160 nurses participating in the study. Whereas only 2% of the elderly stated that they had always received information about the risks of their medicine treatment, 29% nurses believed they would always provide information. Four percent of the elderly thought that they had always been informed about changes in their medication, whilst 25% of nurses stated they would always do so. Williamson

(1980), Faden *et al* (1981) and Shriner *et al* (1991) asked physicians about the disclosure practice in their clinical practice. Shriner *et al* (1991) found a considerable difference among 48 dermatologists about which information they would disclose regarding four hypothetical scenarios. Williamson (1980) reported that oncologists disclosed more information about surgery than medication and the study by Faden *et al* (1981) revealed that neurologists believed that they disclosed only what they regarded as the most common side effects of antiepileptic drugs.

As seen above, many earlier studies focused on information recall after providing written information (Muss *et al*, 1979; Cassileth *et al*, 1980a; Olver *et al*, 1995) or physicians' perception of information disclosure (Williamson, 1980, Faden et al, 1981; Shriner et al, 1991). The results show that there is a lack of patients' awareness for potential side effects. In addition, a considerable number of patients wrongly assumed that the administration of drugs would have therapeutic effects instead of being palliative. However, the studies do not record how much verbal information was actually disclosed during the patient-doctor encounter.

A few studies used observational methods (tape or observer) to record the amount of information actually disclosed (Lidz *et al*, 1983; Lidz *et al*, 1985; Wu and Pearlman, 1988; Katz *et al*, 1992; Braddock *et al*; 1999; Gattelari *et al*, 2002). In the 1980s Lidz *et al* (1983 and 1985) and Wu and Pearlman (1988) observed IC for routine health care interventions including medication administration on surgical and cardiology hospital wards. The results showed that there was a lack of information disclosure about side effects and treatment alternatives. Treatment alternatives were only mentioned in 12% of 172 observed procedures regarding the care of 25 patients (Wu and Pearlman, 1988). Besides the lack of information, Lidz *et al* (1983) found in their observation of 101 hospital inpatients common barriers to IC such as the physicians' attitude that they thought only one treatment was indicated and therefore offered no alternatives. Physicians' disclosure about non-invasive procedures in the sense of IC was rare (Lidz *et al*, 1985). Although relatively more information was given about invasive procedures, the information did not allow the patient to make an informed decision. Katz *et al* (1992) audiotaped 45 consultations in a rheumatology outpatient clinic and investigated IC for routinely prescribed medicines. They reported that physicians prescribing non-steroidal anti-inflammatory drugs, usually disclosed between one or

two side effects per encounter. Braddock *et al* (1999) analysed 1057 audio-taped primary care and outpatient consultations regarding elements of informed decision-making, which varied according to the decision complexity. They reported that only 9% of the decisions met the definition of completeness and in about 80% of the decisions an eliciting of the patient preferences was absent. All of these studies were carried out in the United States. An Australian study analysing audio-tapes of oncology outpatient consultations showed, similar to the one of Braddock, that in only 29% of cases the patient was explicitly invited to make a treatment decision and in only 10% the patient's understanding was assessed (Gattelari *et al*, 2002).

Another area of empirical research of IC examined the effects of obtaining IC on the patients. Although many authors hypothesised that IC would increase the patients' anxiety and/or the number of patients refusing treatment, this has not been confirmed (Faden and Beauchamp, 1980; Quaid *et al*, 1990). Faden and Beauchamp (1980) examined the effect of disclosed information about contraceptive methods including contraceptive medicines on the choice of 406 patients and on their anxiety to use a particular method and confidence to choose one. They concluded that the vast majority of patients had already decided on a particular method before the information disclosure and that the information did neither increase the patient's anxiety nor confuse them. However, patients may have been partly familiar with contraceptive methods prior to the study and disclosing information about unknown medicines and interventions may result in different findings. Instead of relying only on patients' self-report another study additionally used physicians' assessment of serum drug concentration to assess the effect of IC and information disclosure on adherence (Quaid *et al*, 1990). They investigated the influence of two different types of consent forms – one form enclosing more detailed information about side effects and associated risks than the other form - on giving permission to treatment and adherence to take an antiepileptic drug in routine therapy. There was no evidence of decreased adherence in subjects who received a consent form with more detailed information compared to the control group.

Five studies used semi-structured interviews or focus groups to investigate the patients' and HCPs' understandings and views of IC for routine health care interventions in more detail (Lidz *et al*, 1983; Davies, 1988; Eijkman and Goedhart,

1996; Barnes, 1998; Aveyard, 2002). In addition to observation of inpatients in a teaching hospital in the United States Lidz *et al* (1983) performed in-depth interviews with 43 cardiology and 58 surgery in- and outpatients of a university teaching hospital. The results showed that patients often did not know who the responsible decision-maker was and that the decision-making was mainly assumed to be the physicians' task. This was similar to the findings of Barnes (1998) where many of the 31 oncology patients with different ethnic backgrounds accepted the passive role and expected the oncologist to decide about treatment. Whereas Lidz *et al* (1983) and Barnes (1998) focus primarily on the patients' views of decision-making another US study explored the perspective of nurses only, investigating their approach towards IC obtained by physicians for medical treatment (Davies, 1988). The participating 27 nurses saw themselves as patient's advocate, co-ordinator and facilitator as well as watchdog (Davies, 1988). The study is limited in that nurses did not carry the main responsibility to obtain IC. A Dutch study used focus groups to explore the opinion of 34 dentists towards the introduction of a new legislation concerning IC in dentistry, which found that many dentists feared increased paper work and greater time investment per patient as well as loss of income (Eijkman and Goedhart, 1996).

Only one of the five studies using interviews or focus groups was conducted in the UK (Aveyard, 2002). It investigated the perception of nurses who obtained consent prior to nursing care procedures and gives valuable insight into their understanding of IC. In addition to six focus groups 100 critical incidents were explored through 30 in-depth interviews to study the meaning of implied consent prior to nursing care procedures. The majority of nurses assumed the patient's consent to the health care procedures. Since there was an absence of information disclosure prior to the procedures the author concluded that the nurses' understanding of implied consent was similar to compliance.

Some studies combined questionnaires with an analysis of other recorded information to assess the quality of the IC process for routine interventions. No studies were found which investigated the quality of obtaining written IC exclusively for medicines such as chemotherapy. However, the following studies have been included in the literature review since they give some information about the common practice of obtaining written IC for routine therapy in general as well as HCPs' knowledge about IC.

For example, a recent study by Woodward and Roberts (2001) examined the incidence records of a district general hospital and written complaints to see how many were related to IC. In a period of three years 74 incidences related to consent had been reported. For instance, complaints relating to IC were made in 0.3% of all operations carried out between 1999-2000. The most frequent type of failure was an incorrect treatment procedure written on the consent form. Other complaints related to lack of information given about risks and complications of treatment. In addition, Woodward and Roberts (2001) sent out questionnaires to physicians of different grades testing their knowledge of IC. They concluded that all of the physicians lacked essential knowledge about consent. For example, 43% of senior doctors were unable to define the term "valid consent". Most of the junior doctors had the opinion that they were not adequately prepared for obtaining consent. Ferguson (2001) did an audit of medical records in a NHS district general hospital to investigate the number of times informed consent was obtained and the grade of medical professionals obtaining it. They found junior doctors obtained consent from 82 out of 100 patients undergoing surgical interventions. The inadequate training of junior house officers recruited from different regions in the UK has also been described by other authors (Richardson and Jones, 1996; Paice *et al*, 2001). Studies from other countries report similar results about gaps in the knowledge of the physicians (Harris; 1982; Schouten, 2001).

No research was found which examined the readability of consent forms for routine health care interventions; all studies looked at consent forms used in clinical trials. One study compared the reading comprehension level of 100 consecutive patients in an oncology outpatient clinic and compared this with their findings of the readability of consent forms. They concluded that only 34% of the patients in the study had a reading comprehension level which was required for the comprehension of the consent forms (Jubilier *et al*, 1994).

In summary, most empirical studies about IC for routine therapy focused on the practice of information disclosure and the assessment of the patients' recall and/or understanding and used quantitative methods. In terms of trends in the design of study and aspects investigated, more recent studies use a combination of methods (e.g. document analysis combined with surveys) or the analysis of audio-tapes rather than relying on the results of surveys only. Due to the development of increased patient

involvement into medical decision-making some of the studies conducted in more recent years investigated the aspect of informed decision-making in addition to IC, in particular the aspect of offering patients the choice between various treatment alternatives (Braddock *et al*, 1999; Gatterlari *et al*, 2002).

One of the recent publications about the theories of consent might help to understand why the quantitative assessment of the patient's knowledge and understanding dominates in the research literature about IC (Alderson and Goodey, 1998). The authors argue that this is the result of a positivist understanding of IC, in which the factual information given to the patient and the quality of the information presentation are the core issues. Measuring the amount and quality of information provides an indication of the delivery in practice. The positivist approach to consent set such high standards that it is regarded as very unlikely that patients are able to give IC. Other theories such as the constructed consent, which emphasise the complexity and ambiguity of consent, or the post-modern view, which perceives the different treatment options as more important than the actual decision, are neglected. According to the post modern view the offering of treatment choices is regarded as the core issue to enable patient self-determination.

The practical limitations of IC in routine therapy concerning treatment with medicines reported in the studies were the patients' lack of intellectual capacity, emotional ability or desire to make an informed decision, as well as a lack of training of doctors in obtaining IC. Due to these limitations a considerable body of literature questions the practicality of IC in medical therapy and suggests a more flexible approach (Ubel and Loewenstein, 1997; Hansson, 1998; Clarke, 2003;).

Overall, there is a paucity of studies investigating the patients' and the HCPs' views on IC for medicines, its benefits, risks and limitations in various health care settings. Only one UK study has been found which investigated the HCPs' understanding of IC for a routine procedure in depth (Aveyard, 2001). An exploration of the patients' and HCPs' perception could help to understand why IC is not realised in practice.

2.3 Other models of treatment decision-making

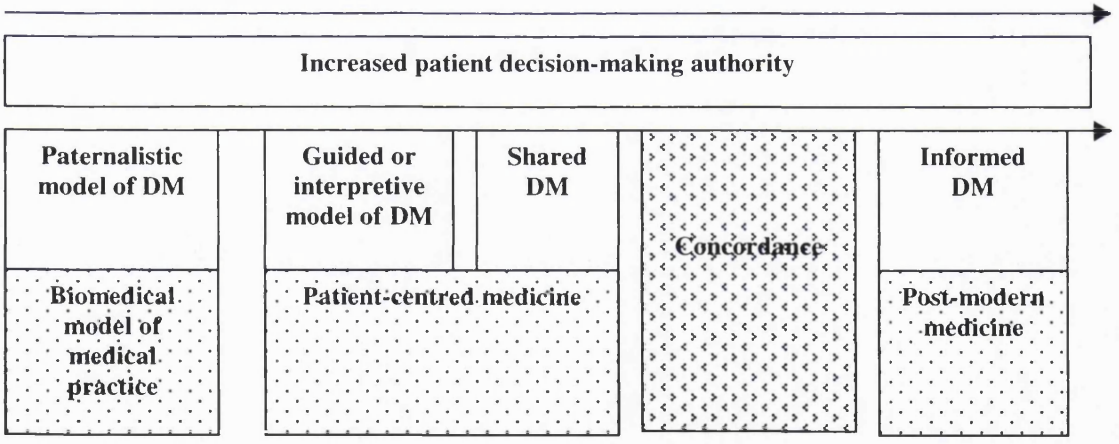
In this section some of the most frequently discussed decision-making models developed over the last decade will be described. This will be followed by a summary of the empirical literature about the decision-making concerning medication treatment made within the physician-patient consultation. It will close with the implications of previous theoretical and empirical work for this thesis.

The interest in research about doctor-patient interaction increased over the last few decades and with it the body of literature about decision-making on medicines. As described before, the notion of the right of the patient for self-determination resulted in a change of the professional-lay relationship and the view on medical decision-making. The majority of work about the decision-making models has been written during the last decade. However, one very early scientific paper from the 1950s introduced two new models of decision-making besides the paternalistic model, the most common of patient-physician interaction: the guided model and the mutual participation model (Szasz and Hollender, 1956). Interestingly, there is not only a difference in how the patient and the physician interact in the decision-making. The authors also point out that the definitions of "therapeutic outcome" and "health" are different in each of the three models. Whereas in the paternalistic (or so-called active-passive) model and in the guidance-co-operation model the physician defines and evaluates therapeutic results, in the mutual participation model treatment outcomes are defined in agreement with the patient. The role each party prefers to take is related to the individual's perception and beliefs about health and illness. Besides the movement towards more patient autonomy, the change in the concept of health and illness may be another driving factor for the search of new decision-making models (Sullivan, 2003).

Apart from IC, which was established into medical practice especially for research and invasive therapies, new models of doctor-patient interaction and day-to-day decision-making were suggested, in which the locus of decision-making authority shifted from the physician increasingly towards the patient. Today, some of the most frequently cited models of treatment decision-making are the paternalistic, the interpretive, the shared (or deliberative) and the informed (Emanuel and Emanuel, 1992; Charles *et al*, 1997 and 1999a,b). All the decision-making models vary in the role that the physician

and the patient take in the final selection of treatment (see Diagram 2.3.1). In the paternalist model, practised over centuries, the physician chooses the treatment after evaluating the information about the disease of the patient, the treatment options and the probabilities of outcomes. Another model is the interpretive (Emanuel and Emanuel, 1992). Although the physician decides about a treatment plan, he/she would take the value and preferences of the patient into consideration. Shared decision-making means that at least two participants – the physician and the patient – are being involved, both parties (physicians and patients) take steps to participate in the process of treatment decision-making, they share information and a treatment decision is made to which both parties agree. Only in the informed decision-making model the patient decides on his or her own after the physician has disclosed the information about benefits, risks and alternatives of the treatment options.

Diagram 2.3.1: Different decision-making models in relation to decision-making authority



IC, which has not been included in Diagram 2.3.1, is most similar to informed decision-making model as in both cases the patient has the final decision-making authority. However, in contrast to informed decision-making, IC is a form of legal authorisation for which certain criteria need to be met before the patient's authorisation is valid (such as capacity, understanding etc, which have been mentioned above). In addition, in informed decision-making the physician presents all treatment options

available to the patient from which the patient decides, whereas IC does not require that the patient selects one treatment out of a range of options. Usually, the physician asks for the permission for one certain health care intervention while informing about others.

Some of the decision-making models rest in a wider concept of physician and patient interaction and include factors unrelated to decision-making. For instance, shared decision-making rest in the concept of patient-centred medicine. In addition to decision-making, patient-centred medicine also describes the way in which physicians should practice medicine (Weston and Brown, 1995). It is based on the theory that incorporating patient's subjective experience of the disease into the clinical practice is an essential element of medical practice (Laine and Davidoff, 1996). Six interactive components of the patient-centred process have been defined (see Table 2.3.1). The third element of patient-centred medicine, which is called "finding common ground regarding management", refers to decision-making between physician and patient. It suggests the use of shared decision-making or interpretive decision-making according to the patient preference. In a recent publication Michie *et al* (2003) added that, apart from the skill to elicit the patients' beliefs, the ability to activate the chronically ill patients to take control in the consultation and/or in the management of their illness might be of great importance for the outcome of the patient-centred approach.

Table 2.3.1: Elements of patient-centred medicine (according to Weston and Brown, 1995)

Elements
• Exploring both the disease and the illness experience • Understanding the whole person • Finding common ground regarding management: problems and priorities, goals of treatment, roles of doctor and patient in management • Incorporating prevention and health promotion • Enhancing the patient-doctor relationship • Being realistic

Similarly, the model of concordance promotes a way of physician-patient encounters rather than just decision-making about treatment (Royal Pharmaceutical Society of Great Britain and Merck Sharp & Dohme, 1997). It is similar to patient-centred medicine in the assumption that the patient's experiences of the illness need to be incorporated in their care. According to the definition given in the foundation

document concordance is defined as "an agreement reached after negotiation between a patient and a healthcare professional that respects the beliefs and wishes of the patient in determining whether, when, and how medicines are to be taken" (Royal Pharmaceutical Society of Great Britain and Merck Sharp & Dohme, 1997). The authors regard the concept as "a new approach to the prescribing and taking of medicines". This suggests that the concept is only focusing on the part of the consultation which deals with prescribing and taking of medicines. However, the authors wished to see concordance as "an integral aspect and not a detachable component of the whole process of clinical care." Besides including aspects other than decision-making the concept of concordance is distinct from shared decision-making in the fact that the patient's view should have precedence and that the patient can delegate the decision-making authority to the physician (Pollock *et al*, 2002). (In contrast, delegating the decision-making authority to another party is not defined as shared decision-making.)

Most authors discussing the models have acknowledged that in practice rarely only one model is used in its ideal form (Charles *et al*, 1999b). There are several forms between the ideal models in which decisions can be made. In addition, elements from various models can be used in one decision-making process. Which model is used depends on the clinical circumstances and on the preference of the patient and the physician. In an emergency situation the paternalistic model has been advocated. There is no agreement about which model is the most appropriate in a given clinical situation.

2.4 Empirical studies about patient participation in decision-making during the doctor-patient consultation

There is a large body of social science and medical literature concerning patient-doctor communication. One part is empirical literature about decision-making regarding medicines in the patient-physician consultation and in particular the role of the patient in decision-making about medication therapy. The following section will give an overview of the findings of empirical studies investigating actual and perceived decision-making about medication in the patient-doctor consultations, in particular

studies which investigate the inclusion of the patients' perspective and preferences in decisions concerning their medicines.

The databases MEDLINE, EMBASE, PubMed, International Pharmaceutical Abstracts (IPA), PsycINFO and Pharmline were searched by using the key or title words "decision-making", "shared decision-making", "patient participation", "physician-patient relations" either alone or in combination with "prescription", medicines", "drugs", "hospital". The function 'related articles' in PubMed was used to search for more empirical work as well as the thesaurus, the subheadings or keywords of the databases to identify relevant literature. A hand search was conducted of the journals *Social Science and Medicine*, *Health Expectations*, *Journal Patient Education and Counselling, Medical Decision Making* and *Quality and Safety in Health Care* for the last ten years or since they were published. Mainly studies carried out in the UK will be discussed as it has been highlighted that the concept of participation is viewed differently depending on the cultural background and health care setting (Brody, 1980).

Before summarising the findings of the studies it is worth mentioning some aspects of research regarding patient participation in decisions about their medication within the patient-doctor consultation and the difficulties in searching for relevant literature. The term 'patient participation' or 'patient involvement' is very loosely defined and represents a continuum regarding firstly, the kind and extent to which the patient is involved in his or her care and secondly, the stage in the care, in which the patient is involved. For instance, involvement can range from receiving information about potential side effects of the treatment to actively deciding between treatment options. In terms of stages, the patient can be involved in the phases of the diagnosis or at the time of management of the treatment at home. This absence of a more specific terminology and the enormous number of studies exploring various forms of 'patient participation' make it difficult to narrow the number of relevant studies. In addition, the studies use very different methods to determine the extent of participation and its effects, which hampers a comparison between studies. Furthermore, the study results are often inconclusive and suggest a very complex relationship between variables. It is therefore necessary to carefully analyse the criteria of assessing the actual, perceived or preferred degree of participation in decision-making, or the aspects of cognitive,

affective and behavioural measure chosen in order to detect the assumptions built into them or the way, in which the authors conceptualise 'patient participation'.

A considerable number of early communication studies focused on the information exchange between doctor and patient (Brearly, 1990). Ong *et al* (1995) pointed out that sharing of information is not the same as participation in decision-making. However, the degree of information provided to the patient is often used to indicate how far the patient is able to participate. The elements of discussion of patient preference and negotiation about treatment choice are commonly neglected (Charavel *et al*, 2001). This is probably related firstly, the historical background of research into doctor-patient communication and secondly, to the difficulties in assessing these elements.

Historically, doctor-patient communication has been studied extensively in primary care in Britain during the sixties, seventies and beginning of the eighties (Balint, 1964; Byrne and Long, 1976; Tuckett *et al*, 1985). Although these studies do not focus on decision-making about medication, they established and promoted a patient-centred consultation style, which contributed to the later development of the decision-making models such as the interpretative, deliberative and shared decision-making. Byrne and Long (1976) for example distinguished between doctor-centred and patient-centred behaviour, where patient-centred behaviour included items such as answering the patient's questions, seeking, accepting and using the patient's ideas. They developed a checklist, which can be used to assess the consultation style of a physician. However, these studies investigate more the doctors' behaviour and do not assess the extent to which the patients have influence on the final treatment selection.

This review focuses on studies which investigated the patient participation in decision-making about medication treatment in the physician-patient consultation. Excluded were studies which investigated the patient participation in a wider sense than decision-making, for example the patient participation in other aspects of their care or the patient-centeredness model, which embraces more aspects than the process of decision-making as discussed before. The review focuses on three aspects: the actual process of decision-making about medicines in the physician-patient consultation, the perceived role of the patient in the process and the patients' and physicians' concept of decision-making. Additionally, studies assessing the patient preferences of their role in

decision-making, potential barriers to patient participation as well as research investigating the influence of actual or perceived patient participation in decision-making on outcomes will be discussed.

2.4.1 Empirical studies of actual patient-doctor decision-making regarding medication therapy

Observational studies of decision-making about medicines in the primary care consultation in the UK have shown that the physician decides about the course of treatment in most consultations. Video-tapes of 261 consultations from a purposive sample of 40 primary care physicians in Oxford showed that in only 15% the physicians asked the patients about their opinion regarding their medicines and the majority of the patients were passive and did not seeking information about their treatment, contributing to a lack of patient participation in decision-making (Makoul *et al*, 1995). Stevenson *et al* (2000a) used the first two characteristics of shared decision-making – the exchange of information about and the view of medicines – to analyse 63 audio-taped primary care consultations in two regions in England. On one hand, the study revealed that the majority of patients did not tell the physician whether they preferred a different medicine than previously or whether they did not want a prescription. On the other hand, in the majority of consultations the physicians did not ask the patients about their preferences regarding medicine treatment. The authors concluded that there was no evidence for shared decision-making regarding prescribing decisions. These findings were confirmed by Elwyn *et al* (2001a) who purposely selected ten primary care consultations from clinicians trained in communication skills to study the elements of shared decision-making in depth. The element of eliciting the patients' view about treatment alternatives and their preferred role in the decision-making process was absent. From various elements of shared decision-making the least time was dedicated to information provision about treatment choices, which the authors regarded as the crucial element to enable shared decision-making.

Barry *et al* (2000) analysing 35 primary care consultations reported that the style of communication between the physician and patient and the decision-making differed according to the problem with which the patient presented in the consultation. When

patients suffered from a psychological problem it was more likely that physicians took the individual experience of the patients into account compared with patients presenting with a physical complaint. In the latter the physician tended to use a technical language and control the consultation, which suggested that acknowledgement of the patient's individuality was less important and had a smaller impact on the process of decision-making.

A Swedish study used audio-tapes of 51 primary care and outpatient consultations of 11 physicians from five different clinics to analyse the doctor-patient communication and the process of decision-making about antihypertensive drugs (Kjellgren *et al*, 2000). Although in 14 consultations a drug dosage was changed or a new medicine prescribed, this was discussed in only four of these cases. Interestingly, one of the most frequently introduced topics by the patient was drugs, mostly related to their effects or side effects, and followed by the quantity and the choice of the drug. However, in almost all cases the physician decided about the medication and dominated the communication, in which the risks related to hypertension were mentioned in only one fifth of consultations.

Findings from an observational study of 1,057 primary care consultations in the US shows similarly, that the amount of information provided during the consultation was not enough to make an informed choice (Braddock *et al*, 1999). As discussed in the context of empirical studies investigating IC, treatment alternatives were only mentioned in 11%, the physicians asked the patients about their preferences in 21% of cases and the patients' understanding was only assessed in 1.5% of the cases. The low proportion of consultations in which patient's understanding had been confirmed was also found by Gattellari *et al* (2002) who studied audio-tapes of oncology outpatient clinic consultations in patients diagnosed with incurable cancer in Australia. However, the recordings showed that in contrast to the findings from the primary care consultations the number of patients who were informed about treatment alternatives such as chemotherapy (44%) or who were offered choices (30%) were significantly higher. This is likely to be related to the severity of diseases and the side effects of the treatments used. However, still around two thirds of patients were not offered treatment choices and more than half were not informed about alternatives.

Another part of the same Australian research project included only patients in the early stage of the disease receiving adjuvant therapy, where 59% of the 101 patients were offered an explicit choice of treatment options including chemotherapy, out of which 53% accepted the recommendation of the physician (Leighl *et al*, 2001). In contrast, the rest of the patients (41%) were not offered a choice, 95% of them accepted the recommendation. It was found that a patient's age of less than 60 years and the female gender of the patient were predictors for more detailed information disclosed. That the majority of patients followed the recommendation of the physician was also found in an observational study of consultations of bowel cancer patients in the UK (Sanders and Skevington, 2003). It was noted that only in the 10 out of 55 consultations where the physician did not give a recommendation about a preferred treatment option, did the patient become more active in seeking information about the potential treatment alternatives and the pros and cons of each of them. The authors concluded that in these cases the patients felt the urge to be active and evaluate the options in order to be able to make a decision.

As seen so far, many studies focused on the amount of information exchanged between the physician and the patient rather than specific elements of a decision-making style. An exception is a study of 3,453 primary care consultations observed by 8 trained research nurses carried out in the US which focused on the investigation of a participatory decision-making style defined as offering treatment choices and giving the patients control and responsibility over the decision-making. This included negotiation elements in the consultation such as "What do you think?", "What would work for you?", "How would you feel about doing it this way?". It was found that in only 25% of the consultations participatory decision-making occurred (Gotler *et al*, 2000).

Compared to primary care or outpatient clinics, the number of observational studies found which were carried out on hospital wards was low. In this context it is helpful to mention a large study by Silverman (1987), who did most of his work in paediatric outpatient clinics analysing over 1,000 audio-tapes. In the introduction to his book he wrote that for practical reasons such as resource intensive observations and the difficulty to tape-record the ward based conversations, he decided to audio-tape outpatient instead of inpatient consultations. Although his data did not include hospital

ward consultations, his extensive and detailed work give interesting insight into clinical decision-making of patients and medical specialists. He concluded that there are three main types of decision-making behaviours: (1) the 'doctor decision-making' where only the doctor decides about the course of action, (2) the 'persuasive' type and (3) the 'democratic' decision-making where in the former the doctor persuades the patient to choose the physician's preferred option, and the latter where both patient and doctor assess the options and decide about the course of action. He concluded from his findings that the nature of the medical condition determines the decision-making style chosen. For instance, if the patient is asymptomatic but an intervention is regarded as necessary the physician would use a persuasive style. On the contrary, if the patient were symptomatic the doctor would decide. A democratic consultation style would only be chosen if there is no urgency for the intervention.

One US study used an observational method to investigate physician-patient interaction on oncology wards (Blanchard *et al*, 1983). The researchers used a behaviour check-list to record their findings from 392 interactions observed. It was found that a conversation about test results with the patients occurred in 14% of the observations, the identification of future treatments, tests or the possibility of future procedures in 32%, and an explanation for reasons for treatment or non-treatment in 10%. Far less frequently was a detailed description of the future treatment given (5%) or the prognosis discussed (3%).

Overall, most studies have been conducted in primary care, investigating the type and amount of information provided by the physician regarding medication which was used as an indicator of how far the patient is able to make an informed choice or to participate in the decision-making. The results showed that the physicians almost exclusively made the decisions regarding medication.

2.4.2 Physicians' and patients' perception of the decision-making about medication therapy

One would expect that observational studies be more frequently used since they assess the actual communication between the physician and the patient. However, Makoul *et al* (1995) noted that compared to observational studies there is more communication

research, which relies on self-reported behaviour or perception of the information given, that means assessing communication indirectly. Surveys assessing the patients' and HCPs' perception of information provision in hospital are more frequent than the analysis of observational data. For example, a recent large survey carried out in hospitals in England including around 95,000 hospital inpatients found that 43% of hospital inpatients felt that they had only partly been informed about their medicines and one fourth felt that the doctors were talking about the patients without directly addressing the patient (Department of Health, 2003b). Around 46% of the respondents stated that they would like to be more involved in the decisions regarding their care and treatment.

The perception of patients concerning their role in decision-making about medication gives some clues to why patients were more passive and did not take control. For example, one important theme from a Canadian study involving 21 women deciding about adjuvant chemotherapy for ovarian cancer after surgery was the patients' feeling of "being overwhelmed" and not necessarily able to make a decision even if they wanted (Elit *et al*, 2003). The severity of illness, the unexpected diagnosis of cancer and the feeling of being pressured made it difficult to decide. Importantly, the interviewees perceived that there were no treatment alternatives. Only four patients stated that there was the option not to undergo chemotherapy and one thought that there was an alternative between drugs. Most of them mentioned their respect and trust in the oncologist to make the decision for them.

Although some of the bowel cancer patients interviewed by Sanders and Skevington (2003) were aware of the alternative not to undergo the treatment, for instance chemotherapy, they did not regard this as an actual option since they thought refusal would result in dying very quickly with no hope. Other important findings were that first, many of the 37 participants felt no ownership for the decision as they did not regard themselves as experts and second, that some participants who felt to have an active role understood 'active' as accepting or rejecting treatment rather than deciding between different options. This is very different from the results of a study among 39 patients diagnosed with HIV/AIDS (Marelich *et al*, 2002) where the large majority considered themselves as active in the sense of making decisions between different treatment options and even demanding specific treatment. This might be due to the

younger age of the participants (mean age was 40 years compared to 60 years of the oncology patients) or the different nature of the condition. Patients of younger age have been found to prefer a more active role in decision-making compared to older patients, as discussed below (Beisecker and Beisecker, 1990). However, similar to the finding of Elit *et al* (2003) participation in the decisions immediately after diagnosis was found to be difficult.

Apart from the younger age of the participants diagnosed with HIV/AIDS the different health care settings of the studies could be another reason that HCPs in primary care may be more likely to adopt shared decision-making than HCPs in a secondary care setting which the findings of interviews with 12 twelve pre-registration house officers spending their training in both settings suggest (Thistlethwaite, 2002). However, even in a primary care setting findings of a survey in 13 GP practices in England involving 171 patients showed that patients felt that in nearly half of the consultations (47%) the decisions had only been made by the doctor (Ford *et al*, 2003). Thirty-nine percent of the decisions were seen as shared and in 14% patients believed that they made their own decisions.

As seen so far, the rationale behind some of the oncology patients not to make decisions about the selection of their medicine treatment was the lack of awareness of treatment alternatives, the lack of ownership for the decisions or the emotional ability in a situation of recent diagnosis. The latter was also experienced by HIV/AIDS patients in primary care immediately after they were informed about their diagnosis (Marelich *et al*, 2002).

Beside studies investigating the patients' perception of decision-making about medication other research explored the concept of GPs and hospital doctors regarding patient participation about decision-making (Elwyn *et al*, 1999; Stevenson, 2001 and 2003; Rogers, 2002; Ford *et al*, 2003). The results of focus groups involving a total of 39 GP registrars from Wales using shared decision-making as a framework showed that the participants thought sharing the process of decision-making is only possible in a few consultations (Elwyn *et al*, 1999). These were cases where there are different treatment options and where the patient was able and willing to participate in decision-making. The participants admitted that they did not disclose all the options available,

in particular the choice of not initiating treatment and that they usually "friendly persuaded" the patient. Some physicians thought that shared decision-making would "unburden" the doctor, especially in situations where there was uncertainty about what was seen as the best treatment option. Others warned that shared decision-making could be reduced to a rhetorical question of what the patient prefers. The participants assumed that they were able to judge the patient preference in decision-making or that they were in agreement with the patient, which was often not the case (Stevenson, 2003). Similarly, Britten *et al* (2003) reported that only in 53% of the time the doctor was right when asked whether the patient wanted a prescription or not. A prescription was defined as wanted if the patient stated in a pre-consultation questionnaire that they would like one.

The findings of the nine face-to-face interviews conducted by Stevenson (2001) in addition to a focus group with GPs in England showed that GPs assumed to know the amount of information the patient wishes to receive about oral steroids and tended to provide information only upon request. However, her sample size was small and the study only focused on one chemical class of drugs.

Stevenson *et al* (2000a) conducted feed-back sessions with GPs about their consultation style after an audio-tapes analysis of their consultations. The GPs believed that the hospital based education and the patients who expected their problem to be solved would encourage a paternalistic consultation style. In addition, although no evidence of a shared decision-making style was found in the majority of the consultations analysed, the GPs assumed that they would practice it (Stevenson, 2003). This finding was also reported by Towle and Godolphin (1999), who interviewed five GPs beside four patients and three patient educators.

Instead of shared decision-making Rogers (2002) investigated the views of physicians towards patient autonomy. She interviewed 21 purposively sampled Australian GPs regarding three aspects of lower back pain management in their daily practice: narcotics, x-ray and complementary medicines. Depending on the aspect of management, the patient autonomy was acknowledged or discouraged. In contrast to complementary medicines, the GPs felt most strongly about restricting patient autonomy regarding narcotics with the argument to prevent the patients from harm.

This was inconsistent with their attitude towards x-rays where they responded more to the demand of the patients for more x-rays, even though it was known to be harmful. The author explained that with the absence of professional and legal regulations limiting the number of x-rays and with the fear of the GPs loosing their patients if they did not respond to the patient's demand. Since the study was carried out in Australia some issues may be less relevant for the practice in Britain such as the fee-for-service and the fear of loosing the patient. However, Bradley (1992) reported similarly, that the prescription of medicines was driven by the fear of damaging the physician-patient relationship.

Ford *et al* (2003) conducted interviews with a purposive sample of 11 GPs, 10 hospital doctors, five practice nurses, 11 academics and eight lay people in the UK to explore the concept of evidence-based patient choice consultations. They reported that regarding the step of decision-making the doctors' opinions ranged from the need to 'steer' the patient towards the most appropriate option to the ones who thought that the patient should determine the treatment chosen.

In summary, the majority of research investigating the views of physicians' perception of decision-making was carried out in primary care. GPs tended to rely on their judgement regarding the amount of information that the patients wish to receive and the extent to which they wanted to be involved. A considerable number of GPs thought that they would practice shared decision-making, which was in contrast to the findings of the observational studies.

2.4.3 Patient preference for participation in decision-making

Since all studies showed an absence of patient participation in decision-making of the selection of the medication, the question arises if patients themselves would want to participate and to what extent they would preferred an involvement. Most studies have been conducted in the US and have used surveys. Hence, they do not explore the reasons of the participants' views held in depth, and it is uncertain how far the results can be applied to a different cultural setting and a country with a public funded health care system.

The surveys distinguish between the patients' preference for involvement by receiving information and the preference for making a decision regarding their treatment. In general, despite the overwhelming majority of patients wanting all the information about their treatment, fewer patients wanted to make a treatment decision (Blanchard *et al*, 1988; Ende *et al*, 1989; Beisecker and Beisecker, 1990; Degner and Sloan, 1992; Vick and Scott, 1998). Some of the studies - particularly the early ones - used very broad and less specific questions to quantitatively assess the desire of patients to make a treatment decision. For instance, the patients had the alternative to decide between "I prefer participation in the decisions" or " I prefer leaving the decisions to physicians" (Cassileth *et al*, 1980b; Blanchard *et al*, 1988). Alternatively, a study used one statement "I prefer to leave decisions about my medical care up to my doctor" and classifying 'strongly agree' or 'agree' as passive and 'disagree' and 'strongly disagree' as active (Arora and McHorney, 2000). Other studies used more specific statements and distinguished not only between the passive and the active, but also gave the option of a collaborative role (Degner and Sloan, 1992).

Deber *et al* (1996) highlighted the fact that the questions in previous surveys were commonly not specific enough to distinguish between the patient's desire to take part in the selection of the desired treatment outcomes (so-called 'decision-making') and the actual process of identifying the most suitable treatment ('problem-solving'). In their survey among 300 patients undergoing an angiogram at a day clinic or short-term stay at hospital they found that the majority of patients wanted to be involved in the 'decision-making' (the selection of the treatment outcomes). However, the patients preferred the physician to do the 'problem-solving'.

A review of research investigating patient participation reported that the majority of studies used hypothetical scenarios and recruited patients with life-threatening conditions such as oncology patients or patients undergoing invasive procedures (Guadagnoli and Ward, 1998). Only some focused on patients with chronic conditions and investigated their actual preferences for immediate care (Strull *et al*, 1984; Arora and McHorney, 2000;).

Many of the previously discussed studies investigated the relationship between the severity of the conditions, age, degree of education and the desire for participation.

Overall, most studies concluded that the more severe the condition the more the patient prefers to delegate the decision either to the HCP or another patient proxy (Blanchard *et al*, 1988; Ende *et al*, 1989; Degner and Sloan, 1992). In general, increasing age and lower educational status went along with a lower desire to participate in the decision (Cassileth *et al*, 1980b; Blanchard *et al*, 1988; Bradley *et al*, 1996). Comparing different types of medical decisions varying in their significance, such as the initiation of radiotherapy or open heart surgery with the increase of the insulin dose, it was found that the patients wanted more input in the former (Mansell *et al*, 2000). The study also suggested that prior experience of the illness increased the desire of the patients to participate. However, a review of surveys investigating patient preferences for involvement concluded that demographic and situational variables explain only 20% of the variability in preferences (Benbassat *et al*, 1998). They argued that in order to involve the patients according to their preferences the physician has to explicitly elicit the patient preferences.

Instead of questionnaires individual interviews have been used to explore the decision-making role preference of 32 asthma patients from one secondary and four primary care sites, in particular the aspect of the rationale for their preferences (Caress *et al*, 2002). Thirteen out of 32 (41%) preferred the doctor to make the decision but strongly considered their option (semi-passive) and one patient thought that only the doctor should make a decision (passive). Whereas some responders felt unable to participate due to a perceived lack of knowledge, others decided deliberately to entrust the decision to the doctor. The chronic nature of the disease was believed to enable the patient to make decisions over time. The responders expressed the desire to have some level of control and a disliked being treated as purely passive recipients, therefore a semi-passive instead of a passive role was preferred.

2.4.4 Barriers to patient participation in decision-making

The barriers to patient participation in decision-making overlap with the one previously mentioned in relation to IC. In primary care and outpatient clinics, observational studies found that the barriers to participation were related to the attitude of the physician and the patient, the lack of communication to clarify and prevent

misunderstandings, and the limited resources, particularly time (Beisecker and Beisecker, 1990; Makoul *et al*, 1995; Stevenson *et al*, 2000a; Caress *et al*, 2002).

Especially for secondary care barriers such as the large number of decisions, the involvement of several decision-makers and the limited decision-making capacity of the patients due to their clinical condition and emotional status were reported (Lidz *et al*, 1983, Degner *et al*, 1997).

2.4.5 Relationship between patient participation in decision-making and treatment outcomes

One important reason to promote patient participation in decision-making was the hypothesis that it will result in better treatment outcomes first, through tailoring the treatment according to the patient preference and second, due to the belief that the patient involvement itself has positive effects on treatment outcomes (Coulter, 1997). Although it is not the aim of this literature review to describe these studies in depth, the main findings will be summarised and some of the methodological difficulties related to the investigation of patient participation in decision-making discussed.

To date there is still controversy over the question of whether there is benefit and if so, what kind and extent of benefit can be expected from patient participation in their treatment decisions regarding their medicines. One explanation for the ongoing debate is the difficulty to assess and measure the benefits related to the form of decision-making between the HCP and the patient (Degner *et al*, 1997). Most studies used affective, behavioural or cognitive outcome measures elicited by patient self-reporting such as satisfaction, adherence, anxiety, coping style and knowledge about the treatment as outcome measures. Clinical markers of the disease progression, for example function of daily living, which are either assessed by the patient or the HCP, or the number of health consultations have been used less frequently.

Guadagnoli and Ward (1998) did a review of studies investigating the influence of patient participation on outcomes. They found that the study results were inconclusive and argued that the variations in the study findings are probably related to the quality of their design. Many studies have a small sample size and are limited to one health

care setting. Perhaps the most significant limitation results from the lack of a uniform definition of 'participation' or 'involvement in decision-making' and how it is determined. Some of the methodological difficulties will be highlighted below.

It is worth mentioning two frequently cited studies, which are some of the first studies using patients' self-reports of health status (Greenfield *et al*, 1985) or HCP assessment of clinical markers (Kaplan *et al*, 1989) to assess the outcome of outpatient clinic consultations where the patient had a more active role. In contrast to many previous studies the authors specifically related the term 'patient participation' to the influence of the patient on the decisions made during the consultation rather than other aspects of patient participation in their care. For instance Greenfield *et al* (1985) defined patient involvement "within the context of the physician-patient interaction because it is there that the patient can have the *greatest impact on medical decisions* and course of treatment" (italics not in the original). In both studies the patients of the intervention groups – suffering from chronic conditions - were educated about their condition and possible treatment alternatives before the consultation with the aim of enabling them to take increasing part in the discussion and negotiation with the physician. The two studies used audio-tapes for the assessment of the patient role during consultation. The findings of the studies suggest that increased involvement of the patients resulted in health improvement reported by the patients (Greenfield *et al*, 1985) or by the HCPs using clinical markers such as blood pressure or glucose level (Kaplan *et al*, 1989). However, examining the way in which these studies assessed patient participation shows that the measures chosen do not necessarily reflect the full meaning of patient participation. Greenfield *et al* (1985) for instance assessed the degree of participation by measuring the patient utterance per time during the consultation beside other criteria such as the number of patient's questions, controlling attempts and the ratio of controlling utterance by the patient to the physician information provision. However, the frequency and length of utterance may not reflect a meaningful contribution. In addition, the number of controlling attempts of the patients indicates more their ability to lead the conversation not necessarily the influence on the treatment decision made.

More recent studies conducted in oncology, respiratory medicine and endocrinology relied on patient self-report to assess their involvement in decision-making compared to audio-tapes (Adams *et al*, 2001; Gattelari *et al*, 2001; Heisler *et al*, 2002; Golin *et*

al, 2002; Gattelari *et al*, 2002). In contrast to Greenfield *et al* (1985) and Kaplan *et al* (1989), these studies did not conduct an educational intervention to enable the patient to play an active role and only one of the studies assessed the direct impact on a health outcome (health related quality of life) (Adams *et al*, 2001). Whereas on one hand, the study results suggest that the patients' perception of participation in the decision increase their satisfaction (Gatterlari *et al*, 2001; Adams *et al*, 2001; Golin *et al*, 2002), self-management (Heisler *et al*, 2002) and health related quality of life (Adams *et al*, 2001), on the other hand, it was found that increased participation resulted in increased anxiety of patients (Gattelari *et al*, 2002). However, as pointed out above a comparison between studies is limited due to their different methods of assessment and underlying concept of patient participation. For instance, whereas Adams *et al* (2001) asked patients about the physicians' facilitation of the participation including the opportunity to have control and responsibility over the treatment, Gatterlari *et al* (2002) asked the patients for their perception of their involvement in the decision only. Asking patients to assess the physicians' facilitation of patient participation may more reflect the physician's communication skills rather the perceived degree of patient's impact on the decisions made. The studies were carried out in different medical specialities (respiratory medicine, endocrinology and oncology), which are distinct in the significance and gravity of the decisions made. It is likely that the decision about asthma or endocrinology treatment will have a lower significance and gravity for the patient than a decision regarding oncology treatment and accordingly, the degree of anxiety is different.

Another study where 359 patients were randomised to receive either a directive or shared consultation style (eliciting patient's views on diagnosis and treatment) found that patients who received a directive consultation style were statistically significantly more satisfied with the consultation compared to patients receiving a shared consultation style (Savage and Armstrong, 1990). The consultations were audio-taped and checked for consistence of the physician's adherence to the consultation styles required. Satisfaction was assessed by asking patients for the quality of the explanation, the GP's perceived understanding and the feeling of being greatly helped. The authors argued that a directive consultation style was especially satisfactory if patients presented with a physical problem or received a prescription. Hence a directive consultation style may be beneficial for these patient groups. No difference

was found for longer consultations or where patients had psychological or chronic problems or received advice.

In summary, the studies investigating the influence of the actual or perceived patient participation in the treatment decision on affective, cognitive or behavioural outcomes suggest that patients' satisfaction may be enhanced with increased perceived participation. However, the effects on their health and other treatment related outcomes are still unclear and may depend on multiple factors. In addition, the studies and their findings illustrate the difficulty in using appropriate measures, which meaningfully reflect the theoretical concept of patient participation in decision-making proposed by the models, for example shared decision-making.

2.5 Implications for the present study

In the first chapter and within this chapter the theoretical and empirical considerations of health policy and individual, clinical decision-making have been explored. The remaining part of the chapter will highlight the implications of the previous work on the design of the research conducted in this thesis.

Over the last forty years IC has been established as a legal and moral requirement prior to all health care interventions (Doyal, 2001). However, there is an absence of studies investigating IC in clinical practice including medicines compared to its use in research. Previous studies have focused on completeness of information disclosure and recall and neglected a more fundamental approach. The HCPs' and patients' views on the concept of consent and its use in pharmacotherapy are largely unknown. Results of the findings could clarify its acceptability and feasibility in practice.

The concept of IC has been criticised as neglecting the importance of implementing the patient preferences in the range of choices offered and the treatment recommendation given. This had led to the question of what role patients prefer in treatment decision-making and how far patients would like to be involved. So far most studies have used questionnaires and hypothetical scenarios to quantitatively assess the patient's desire to become involved in decision-making. Additionally, there is a paucity of studies

exploring patients' attitude to decision-making, other than in situations of invasive procedure or in oncology. The reasons why patients prefer either to be involved in decision-making or not are still largely unclear (Robinson and Thomson, 2001; Caress *et al*, 2002). Since many of the studies investigating patient preferences were done in the United States, there is uncertainty as to how far the results can be applied to a different cultural setting and a country with a publicly funded health care system.

Studies using observational methods, including the analysis of audio- and video-tapes of consultations to investigate the doctor-patient interaction showed that most patients are uninformed about treatment alternatives and pros and cons of the proposed treatment. The lack of information limits or denies the patients' opportunity of becoming involved in the treatment decisions, make an informed decision and give their IC. There is a lack of studies investigating the patients' and physicians' concepts of involvement and their perceived risks and benefits. Additionally, their reasoning for specific preferences of involvement in their actual context in which one option is preferred needs to be investigated. Elwyn *et al* (1999) highlighted this when they wrote "observational studies alone will not capture the process that takes place 'in the patient's head', where ultimately, decision-making occurs."

Most of the British studies have been conducted in primary care, where the patient is usually more familiar with the general practitioner (GP). However, regarding decision-making about treatment the use of medicines with potentially serious side effects are initiated in a secondary care setting by specialists, either on hospital wards or in outpatient clinics. For example, treatment with warfarin, amiodarone or digoxin is usually initiated while the patient is hospitalised. On one hand, it can be argued that due to the significance of these decisions it is even more important that patients become involved in treatment decisions in secondary care. On the other hand, treatment in secondary care is often very complex and potentially need to be initiated very urgently, which would give the patient less time for consideration. Acute illness, severity of the condition, strange environment and unknown staff can be other obstacles for effective communication.

Although each of the models discussed above captures important aspects of the patient-physician relationship, the models neglect the situational factors of decision-

making such as the various health care settings with their different constraints. It is unclear if and how patients and physicians perceive these factors. Are patients aware of their choices and how important are these for the patient? Do physicians feel in conflict when they know that they are restricted by national or local policy and how do they react to it? How do doctors see their responsibility to make decisions about access to medicines? Do they need to balance the patient's best interest with other constraints?

There is a need to investigate the views of patients and doctors towards choices between treatment alternatives in common prescribing decisions in secondary care, their preferences of the role in decision-making, their reasoning behind actions and choices and the trade-off and weighing-up made between competing values. The results can increase the understanding of patient and doctor preferences and the boundaries of theoretical decision-making models in practice.

In this thesis the research of individual decision-making about medicines will be completed with studies of the health policy decision-making level, where decisions about access to medicines are a precondition for the choice made on an individual level. As discussed in the previous chapter, the main body of literature concerning decision-making about resource allocation covers a range of disciplines: economics, philosophy and medical sciences, although a multidisciplinary approach is rare. Regarding choice behaviour and decision-making theories there is a significant body of psychology and social science literature. Only some of the theories have been applied to health policy decision-making. In health policy for a long time the economic perspective has dominated (Light and Hughes, 2001). Most of the literature concerns the theoretical aspects of health care allocation as described above by using the philosophical work as an example. How useful these theoretical models are in practice is largely unclear. The examples of Oregon, the Netherlands and New Zealand show that there are several limitations of the theoretical models developed. Empirical studies into the factors influencing the decision-making process and investigating limitations of the theoretical models are sparse (Jan *et al*, 2003). However, it has been argued that it is only possible to investigate the values involved in decision-making by examining the decisions made and the actions taken (Neuberger, 2002). Furthermore, through empirical research it is possible to identify the areas which are most relevant for

decision-makers. As described before, only some of the empirical studies have been carried out in the UK, and have focused on one medical speciality or one group of policy-makers. Research needs to investigate a wide range of possible scenarios to reflect the diverse nature of health care provided for very distinct user needs. Additionally, considering scientific, political and ethical aspects would enable an examination of the complexity of influencing factors. Ideally, it should include a variety of information sources to capture the different aspects in decision-making (Hasman, 2003).

Finally, although both health policy decisions about medicines and prescribing decisions involve choice, it is interesting that the above two sets of literature seem to exist in isolation. This guided the research carried out in this thesis towards searching for common and distinct elements that could be drawn out.

The research presented in this thesis has therefore three key aims:

- First, to investigate authoritative decision-making in the NHS about the access to health care including the process, the rationales and the influencing factors.
- Second, to investigate in secondary care the HCPs' and the patients' views on decision-making about choices of medicines within the medical consultation, including the use of IC for medicines.
- Third, building onto the findings from the first two aims, to seek similarities and differences between the factors found to contribute to each type of decision-making about choices investigated before.

The following two chapters will describe and discuss the aims, objectives, methodology and the results of the studies of health policy decision-making regarding access to medicines. The fifth chapter will present the methodology and the results of the study of individual decision-making regarding prescribing decisions. Finally, the sixth chapter will draw out the similarities and differences between these decision-making levels and will present the implications for practice.

PART B: EMPIRICAL STUDIES OF HEATLH POLICY DECISION-MAKING

Part B addresses the first aim of the thesis: the investigation of authoritative decision-making about the access to medicines. In particular, it presents results of two empirical studies investigating decision-making of national and regional bodies of the English National Health Service (NHS) about providing access to medicines by using three drug therapies as an example.

One of the studies was part of a European research project about ethical aspects of health policy in various countries. Instead of investigating all authoritative decisions made in the past or in a certain time frame, which is timely and resource intensive, the research team in the Netherlands coordinating the project chose four health technologies as an example to investigate health policy decision-making in five European countries: Italy, the Netherlands, Poland, Sweden and the UK. Two of the health care facilities chosen are medicines: sildenafil (Viagra®) and rivastigmine (Exelon ®), one is a chemical class of medicines, HMG-CoA reductase inhibitors ('statins'), which lower cholesterol blood levels (see table B.1). In addition to the drug therapies, lung transplantation was chosen as a contrasting example. However, due to the scope of this thesis with its focus on drug therapy, the results of the investigation regarding lung transplantation will be not presented.

Table B.1: Comparison of some characteristics of the three drug therapies

Variables	Sildenafil	Rivastigmine	Statins
Kind of treatment	Treatment	Alleviation of symptoms	Prevention
Estimated number of patients	2 million	250 thousand	5 million
Age range of the majority of patients	> 40 – 70	> 75	> 50
Main outcome measures	Patient's satisfaction with sexual intercourse	Cognitive function and overall health status	Mortality and morbidity from CHD
Year of market authorisation in the United Kingdom	1998	1998	1989 (licensing of simvastatin as the first statin
Cost per year per patient	~ £ 240	~ £ 800	~ £ 660

The three drug therapies chosen differ in some key characteristics: the kind of treatment intervention offered (alleviation of symptoms, treatment, prevention), the prevalence of the disease in the population, age of the majority of patients affected, the

measurement of effectiveness, the time since their market authorisation in the UK. The different characteristics were thought to provide a variety of scenarios indicative for many other health policy decisions.

The two empirical studies are distinct in their methodology: the first study, a detailed document analysis, was the starting point for the investigations about authoritative decision-making in the NHS about medicines and within the scope of the European project. Assuming that the document analysis would give the official side of decision-making and giving less insight into the process of decision-making and detailed reasoning for deciding in certain ways, a second empirical study was carried out, which was independent from the European project. It used in-depth interviews with policy-makers and stakeholders to explore the views and perceptions of decision-makers and their personal reasoning, which might not be in agreement with the official account. Chapter 3 will focus on the document analysis; Chapter 4 will describe the methodology and results of the in-depth interviews.

CHAPTER 3: DOCUMENT ANALYSIS OF HEALTH POLICY DECISION-MAKING ABOUT ACCESS TO THREE DRUG THERAPIES

Chapter 3 describes and discusses the methods and results of the detailed document analysis of health policy decision-making about access to medicines in the NHS by using three drug therapies as an example. The document analysis had four main objectives:

- to gather information about the outcomes of the health policy decisions about access to the three drug therapies concerned and of the authoritative bodies which were involved;
- to investigate the decision-making process including the information sources used to make the decisions;
- to search for the influencing factors and rationales used to justify the decisions;
- to analyse the arguments used by stakeholders in the debate about access to the three drug therapies.

Before going into the details of the results and the discussion, the study method will be described.

3.1 Document analysis

Document analysis is a research method widely used in history and social science as documents are often viewed as giving an account of events as well as being realities in themselves (May, 1997). Regarding policy research, documents can present the legitimisation used by various policy-makers and institutional bodies (Silverman, 1993). The method was chosen to provide an official account of the decision-makers and different interest groups or health policy analysts and their reasoning and arguments used. In addition, the documents were used to identify the authoritative bodies involved in the decision and the data sources informing the decision-makers. As May (1997) discusses, there are different traditions of document analysis. One tradition is the quantitative content analysis in which the researcher is concerned with a manifest and surface meaning. Another is the interpretative tradition which has been chosen for the present study. According to this qualitative method of analysis the documents are searched for a deeper level of meaning, the perspective of the writer(s)

and the context in which it was written. In respect to the present study it allowed obtaining the view of the health policy makers and stakeholders, the process of its production as well as the social context in which the health policy documents were written. Hence, the information about the chain of historical events relevant for the decision was also included in the analysis.

As some authors have pointed out the method of document analysis is limited in only providing information, which was intended for publication (Jupp and Norris, 1993; Hammersley and Atkins, 1995).

3.1.1 Types of documents

Documents are not necessarily printed information and can include photographs for examples. However, for the present study, only written records have been used. After identification of the main policy-makers and stakeholders involved in national and regional policy-making, their main publication sources were searched. Various types of official documents have been included in the analysis: Administrative and government committee reports and minutes of committee meetings, parliamentary discussions, printed mass media reports and press releases and publication of pharmaceutical companies, patient organisations as well as professional bodies and medical publication about the disease, its prevalence and treatment alternatives.

3.1.2 Sources of data search

The websites of the Department of Health and Her Majesty's Stationary Office (HMSO) were used to obtain government documents including committee reports of the Standing Medical Advisory Committee (SMAC), the Health Service Circulars (HSC) and the National Service Frameworks (NSFs). The websites of the National Institute for Clinical Excellence (NICE), the NHS Research and Development Directorate and the Wessex Institute were also used to retrieve press releases and guidelines concerning the drugs under consideration.

Additionally, databases, library catalogues and hand searches were carried out to identify publications from scientific journals in the field of medicine, health care

research and political science to gather a more reflective and interpretative account of the health policy decision-making. Medical, pharmacy related or social science related databases including Medline, EMBASE, International Pharmaceutical Abstracts (IPA), Pharmline, PubMed and International Bibliography of Social Sciences were searched for publications with the title or key words of the three drug therapies under consideration. They were used as single terms or used in combination with terms such as "policy", "health policy", "health care policy", "decision-making", "United Kingdom", "priority setting" and "rationing". The same search terms were used for the catalogues of two of the main libraries for the holdings of literature on UK health policy - the British Library and the King's Fund library. If the title or the abstract were found of relevance the entire publication was obtained.

A hand search using the same search strategy as described above was carried out for journals of special interest for the subject being researched such as *Health Economics*, *Health Policy*, *International Journal of Technology Assessment in Health Care*, *Journal of Health Service Research and Policy, Journal of Politics*, *Policy and Law* and *Politics and Policy.*

To obtain the media reports the newspaper archives of six main British newspapers – *The Times/ Sunday Times*, *The Independent*, *The Observer*, *The Guardian*, *The Daily Telegraph*, *The Financial Times* - were searched by using the generic name of the drug as well as the product name. Additionally, the name of the conditions or common terms used in connection with the condition such as "Alzheimer's", "Alzheimer's disease", "impotence" and "cholesterol" were chosen as keywords. The *Proquest Newspaper* database, the *British Humanities Index* and databases of each of the newspapers available on CD supported the search.

Websites of patients' and professional organisations of the conditions concerned such as the Alzheimer's Society or the British Heart Association as well as the pharmaceutical companies holding the marketing authorisation for the drugs concerned were searched for press releases and publications about the reimbursement decisions of the drugs and product information. In cases where document titles were displayed or retrieved from other information sources but not fully published, they were requested from the organisations or the companies.

3.1.3 Time period searched

In case of rivastigmine and sildenafil all the databases and search engines were searched from 1998 onwards, the year of their licensing. For statins medical databases and search engines were searched from 1989 onwards, the licensing year of simvastatin as the first statin on the UK market. The hand search of selected journals included the last five years of publications.

3.1.4 Data analysis

The documents were systematically searched for information about the outcome of the health policy decisions; the authoritative bodies which were involved; the information sources they used and the rationales to justify the decision or the position of the author(s). In particular, the speeches of members of the opposition were searched for aspects, which might have been left out in the government accounts about the decisions made. The media publications were used to obtain a variety of opinions and arguments for and against reimbursement that partly reflected public opinion. Press releases of pharmaceutical companies, patients' organisations and professional bodies were analysed regarding their argumentative speech. The latter type of documents were treated as accounts of a group perspective in a particular setting which could reveal aspects of the process of making decisions and interaction of decision-makers and stakeholders.

While the documents are located within a wider social and political context it was important to bear in mind the intention of the writer. The documents were scrutinised for what reasoning and arguments the authors used. In addition, a comparison between the different documents allowed the identification of gaps in each of them and the examination of their intended meaning. As May (1997) described a critical document analysis in the sense of investigating the content, the intended and received meaning can provide useful information about the interests and characterisation of certain events.

3.2 Results of the document analysis

The presentation of the results for each of the three drug therapies will follow a similar structure: firstly, an introduction will be given in order to provide the reader with a picture of the knowledge background of the decision-makers and the stakeholders. In this introduction, the drug properties, the condition for which the drug is used and the treatment alternatives available at the time of the decision will be highlighted. Secondly, the contents of the decisions about access to treatment, as well as the legitimisation used by the regional or national decision-making bodies, will be given. The presentation of the results will focus on these two aspects. Lastly, the main contributions of the stakeholders to the discussion about access to treatment will be described.

3.2.1 Rivastigmine (Exelon ®)

3.2.1.1 Introduction

Rivastigmine (Exelon ®) was licensed in 1998 in the UK for the treatment of patients suffering from mild to moderate Alzheimer disease (AD). As the second kind of its drug from the group of acetylcholinesterase inhibitors (AChEIs), it inhibits the breakdown of the neurotransmitter acetylcholine (ACh) which has important cognitive and memory functions (Novartis, 2001). Its concentration in the brain of an AD patient is lower compared to other individuals. Similar to donepezil and galantamine, the other AChEIs currently licensed, it shows dose related side effects such as nausea, vomiting, diarrhoea and bradycardia, which have been reported in 14 to 50% of patients (Lamb and Goa, 2001). Due to the lack of comparative studies between the three AChEIs, decisions about prescribing one over another AChEI are guided by dosing frequency, co-morbidity and costs (per month £1,248 for donepezil, £821 for rivastigmine and £1,049 for galantamine by using the highest strength of the product) (Clegg *et al*, 2000).

Alzheimer's disease (AD), the most common cause of dementia, is characterised by the loss of memory, cognitive impairment, depression, functional and behaviour changes and an average life expectancy of ten years after diagnosis (Mayeux and Sano,

1999). Basic activities of daily living such as washing, dressing and eating become increasingly difficult as the disease progresses and dependence on carer grows. Before the licensing of AChEIs, such as rivastigmine, drug therapy was limited to the treatment of depression and behaviour changes such as overactivity and aggressiveness which did not influence the progression of the disease.

3.2.1.2 National regulations

At present, the most important document regulating the availability of rivastigmine in the NHS is the NICE guidance published in February 2001 (NICE, 2001). The guidelines refer to all AChEIs and specify the criteria which entitle the patient to receive NHS treatment with rivastigmine (see Table 3.2.1).

Table 3.2.1: National guidelines about the use of acetylcholinesterase inhibitors (AChEIs)

Report/ Title	Date	Recommendation
Standing Medical Advisory Committee (SMAC) "The use of donepezil for Alzheimer's disease"	May 1998	Treatment should be initiated by a specialist and only continued if there is evidence of its benefit. No specific recommendations were given when to initiate therapy.
Development and Evaluation Committee (DEC) "Rivastigmine in the treatment of senile dementia of the Alzheimer type (SDAT)"	Dec 1998	Treatment should be made available to people with mild to moderate SDAT (Mini Mental State Examination 10-26).
National Institute for Clinical Excellence (NICE) "Guidance on the use of donepezil, rivastgmine and galantamine for the treatment of Alzheimer's disease"	Jan 2001	▪ Specialist clinic has to confirm the diagnosis of AD ▪ Patients are eligible for treatment if they have a score of 12 or more in the MMSE ▪ Carer's view on the patient's condition has to be considered ▪ Patient likelihood to adhere to treatment has to be assessed (no standard test, individual clinical judgement) ▪ Patient must benefit from treatment: improvement or no deterioration in MMSE score as well as evidence of global improvement on the basis of behavioural and /or functional assessment ▪ Patient has to be reassessed every six month
Department of Health (2001c) "National Service Framework for older people"	Mar 2001	▪ Reference to the NICE guidelines

Although there had been other reports published about the use of AChEIs before 2001, they were not obligatory for regional decision-makers. Nevertheless, they possibly

influenced the design of the NICE guidelines. The main recommendations of these reports are summarised in Table 3.2.1.

The following section describes the NICE guidelines in more detail. The NICE guidelines recommend that the GP should refer a patient with possible Alzheimer's disease (AD) to a specialist for the confirmation of the diagnosis and the decision about treatment with an AChEI. The specialist should include tests of cognitive, global and behavioural functioning and of activities of daily living (ADL) in the assessment of the patient. The decision to initiate treatment with an AChEI should be based on the outcome of the tests as well as on the subjective assessment of the patient and the likelihood of adherence with treatment. Additionally, the carer's view of the patient's condition should be taken into account before initiation of the treatment.

In justifying the decision to provide treatment under certain conditions NICE referred to the results of the health technology assessment (HTA) commissioned by the institute (Clegg *et al*, 2000). The HTA included four randomised controlled trials (RCTs), which investigated the efficacy of rivastigmine by using three different types of outcomes: cognitive function, global outcome and quality of life. The RCTs lasted for a maximum of 6 months and showed mixed results. Two RCTs found improvement in more than one outcome measure, and two found improvement in one of the measures used (see Table 3.2.2).

Table 3.2.2: Outcomes and results of four RCTs assessed in the HTA

Trial	Global outcome	Cognitive function	Quality of life
Agid *et al* (1998)	+[1)]	-[2)]	Not assessed
Corey-Bloom (1998)	++[3)]	++	++
Foretter *et al* (1999)	+	-	Not assessed
Rösler *et al* (1999)	+	++	-

[1)] + statistically significant improvement against placebo, [2)] - no statistically significant difference against placebo, [3)] ++ statistically significant improvement against placebo only in the higher dose

Although the improvement in global and cognitive function was *statistically* significant between placebo and rivastigmine, the authors of the HTA saw the extent of improvement on the measurement scales not necessarily as *clinically* significant. In the

following, the assessment of cognitive function is used as an example for the difficulties to measure the effect of rivastigmine on AD patients.

One difficulty in the assessment was the size of cognitive improvement. Two studies (Corey-Bloom *et al*, 1998) found a statistical improvement in cognitive outcome between placebo and rivastigmine of an average of 3.78-point improvement on the 70-point Alzheimer's Disease Assessment Scale - cognitive subscale (ADAS-cog). However, the authors of the HTA pointed out that only a seven-point improvement on the ADAS-cog was thought to be clinically significant according to the literature (Clegg *et al*, 2000). Another difficulty was the probability of the effect of rivastigmine. One of the RCTs (Rösler *et al*, 1999) found an improvement of more than four points on the ADAS-cog for 24% of patients in the high dose treatment group compared to 15% of patients in the placebo group. This means that 13 patients need to be treated (NNT=13) to achieve an improvement in one of them by more than four points on the ADAS-cog. The authors of the HTA concluded that there was a high placebo effect.

Considering the fact that only two studies assessed the effect of rivastigmine on the AD patients' quality of life, the authors commented: "The limited use of quality of life measures, whether directed at the person with Alzheimer's disease or their carers, is thought to reflect their relative infancy and the difficulty in developing a valid and reliable measure" (Clegg *et al*, 2000).

In addition to the clinical trials, the HTAs included four economic studies, of which one was found to be out of date, one conducted in the US, one in Canada and only one in the UK. The UK study calculated that significant cost savings for the treatment with rivastigmine for mild to moderate AD are present from 6 months to 3 years of treatment.

Despite these uncertainties in terms of the clinical effects and the limited data on potential cost-savings of rivastigmine, NICE decided to fund treatment for patients who fulfilled certain criteria. It justified its decision on the basis of data about cost-effectiveness suggesting a delay of patients' referral into a nursing home. "A delay of 12 weeks at £370 per week would yield a cost saving of about £4,500" (NICE, 2001). In terms of the group of AD patients eligible for treatment NICE argued that since

clinical trials included only patients with a Mini Mental State Examination (MMSE) score of >10, clinical effectiveness had not been shown for a score of <10. Hence, drugs could only be cost-effective if the MMSE score was above 10 and "at levels somewhere above this boundary, although exactly where this would occur is not currently known" (NICE, 2001). NICE arbitrarily defined that only patients with a MMSE score above 12 should receive treatment and that the score should improve or at least not decrease during therapy.

3.2.1.3 Local regulations

Before 2001, in the absence of the NICE guidelines, rivastigmine was available on private prescription. Access within the NHS varied. A survey among 135 health authority prescribing advisors, which was conduced one year before the publication of the NICE guidance, found that only 46 out of 95 participating health authorities (HAs) provided funding for the treatment with AChEIs (Taylor *et al*, 2001). The local guidelines of the PCTs and the hospital's drug and therapeutic committees should now be in line with the NICE recommendations but the data suggest that even a year after the publication of the NICE guidelines only three quarters of HAs were funding AChEIs (Shubra and Taylor, 2002).

3.2.1.4 Debate of stakeholders about access to rivastigmine

Professional debate and the pharmaceutical industry

One of the first professional guidelines on the use of AChEIs was an interim statement of the Royal College of Psychiatrists (1997), which was similar to the recommendations of a group of British psychiatrists from the London area (Lovestone, 1997). Although this was before the licensing of rivastigmine, earlier professional consensus on the use of the drug class of AChEIs and its place in therapy might have influenced the NICE guidelines. Their recommendations regarding diagnosis by a specialists and the use of the MMSE to assess cognitive function at baseline and to monitor progression of the disease during the treatment were later adapted in the NICE guidelines. In contrast to NICE, the professional statement mentions the uncertainty about the point at which treatment should be discontinued, and hence, no specific MMSE score is associated with it (Kelly *et al*, 1997; Lovestone *et al*, 1997).

The pharmaceutical industry promoted the use of AChEIs in a large nation-wide campaign with frequent advertisements in professional journals such as "Mum has AD but she knew that I was calling today". This kind of advertisement was criticised by medical professionals who argued that the evidence on efficacy was not strong enough to make this kind of claim (Greenhalgh, 1997; Stein *et al*, 1997). This is in line with the overall scepticism of many professionals about the clinical relevance of the functional and cognitive improvement shown by the RCTs (Bayer, 1999; Drug and Therapeutic Bulletin, 2000).

Patient organisations

The British Alzheimer's Society has published a document appraising the use of AChEIs in AD (Alzheimer's Society, 2000). The document was submitted to NICE - before its publication on the use of AChEIs - summarising the views of the Society that the drugs should be available for all patients in the NHS who might benefit from them. The main arguments of the Society were the inappropriate choice of clinical outcome measures used in many RCTs, which had resulted in the underestimation of their benefit. Instead of cognitive function and global outcome, quality of life (assessed by self-report of patients or carers) should be used along with other patient-defined outcomes of treatment. The Society's publication backed their arguments with the results of a member survey in which improvements in mood, increased confidence and reduction in fear and distress were reported more frequently and hence, thought to be more valued than the less frequently mentioned cognitive function. The survey included 2000 responders (84% of them were carers); 1180 (59%) said that they or the person they cared for had received AChEIs for the treatment of AD. Although around three quarters (73%) of the responders thought that the drugs improved or at least prevented the deterioration of the condition, 10% were unsure and 17% said that it showed no improvement. In terms of style of the report it mainly uses quotations from patients and carers to illustrate their points and strengthen its viewpoint.

According to the Society most reports about financial implication for the health service overestimated the costs. The Alzheimer's Society (2000) reported that only 10% of patients suffering from AD would be considered for treatment with AChEIs. In general, economic studies on the cost of AD would rely on very crude estimations

because they often extrapolate findings from a short period of study or pool data from patient groups with very different health care needs. For instance, data from patients with moderate AD and patients needing long-term care in the later stage of the disease were analysed together.

Media reports

The media reporting about the use of new drugs for the treatment of AD accused health authorities (HA) of delaying the funding of rivastigmine in arguing that they wanted to wait for the decision of NICE before they decided about funding. (Pilling and Timmins, 2001). Elderly patients, especially those suffering from dementia, were seen as disadvantaged and vulnerable, "not likely 'to make a fuss' and not a strong interest group" (Bryan, 1998). With the progression of the disease most of them become incompetent in making decisions about their care and become dependent on relatives and carers. "The longer the patients retain their independence the longer they retain their dignity" was used as an argument for the promotion of reimbursement of drugs by the NHS (Stuttaford, 1998).

Another ethical issue involved in the decision about access- the discontinuation of treatment - had also been discussed in the media. In a situation where carers and relatives see the patient in "physical and intellectual humiliations" with no hope of cure it is likely that they wish the patient would have access to NHS funded treatment with rivastigmine. "[...] Relatives will press doctors to prescribe them 'just in case'" (Le Fanu, 2002). If treatment with rivastigmine does not show improvement and is accordingly stopped, it has been argued that this would be "unnecessarily cruel" and "denying a small glimmer of hope".

3.2.1.5 Summary of the debate about access to rivastigmine

Main issues in the debate about access to rivastigmine were the uncertainty about the clinical effect in practice, which was partly related to the absence of reliable and valid outcome measures to determine the impact of the treatment on the quality of life on patients and carers adequately.

Beside the interests of the patients, the carers of the patients were an important interest group due to the high burden of care. The debate also showed that the boundaries between health care and social care were contested.

The case of rivastigmine highlights the problems surrounding decision-making under conditions of uncertainty. The lack of knowledge about the aetiology and pathology of AD not only delays the development of effective treatments, it also makes it difficult to diagnose AD and identify suitable candidates for treatment. This has several consequences. First, it is difficult to make any robust cost calculation for the NHS in treating AD patients as the discussion of the Alzheimer's Society report highlighted. Second, in practice it will depend on the diagnostic skills of the GPs to refer patients to specialists and enable them to receive treatment. Third, the problem of forecasting the success of therapy of a NNT of 13 means that a larger number of patients, who will not benefit from treatment in terms of cognitive improvement, will be exposed to side effects. In addition, resources will be spent without providing improvement of the patient's cognitive function. Whereas the case of rivastigmine highlights the difficulty to determine the benefit of treatment, the case of statins, which will be discussed next, showed another focus of the debate.

3.2.2 HMG-CoA reductase inhibitors (statins)

3.2.2.1 Introduction

Coronary heart disease (CHD) is one of the priority areas for British health policy (National Service Frameworks, 2000). According to epidemiological data 28% of men and 18% of women die due to CHD in Britain (Ebrahim *et al*, 1999). More than 1.4 million people suffer from angina and 300,000 people have a heart attack annually (Department of Health, 2000b). Lipid regulating drugs aim to reduce the progression of coronary arteriosclerosis and heart disease, which can lead to myocardial infarction, angina or heart failure. One class of drugs, which influences the synthesis or absorption of cholesterol and fat, inhibits the HMG-CoA reductase, an enzyme essential for the synthesis of cholesterol. In 2003 there were four HMG-CoA reductase inhibitors - also called 'statins' - licensed in the UK (simvastatin, atorvastatin, pravastatin and fluvastatin). Three of the statins were among the 25 top sold drugs in the UK in 2000 (Association of British Pharmaceutical Industry, 2002).

Side effects with an incidence of less than 1% such as myopathy (sudden unexplained muscle soreness or weakness) and rhabdomyolysis (acute fulminating, potentially fatal condition) are dose related and increase significantly with concomitant use of drugs which inhibit the isoenyme metabolising statins (Schachter, 2001; Davidson, 2001). However, with the awareness of potentially hazard drug interactions and with cautious dosing these side effects can be avoided. Reversible and dose related elevation of liver enzymes with a level three times greater then the norm occurred in 2 to 5% of patients taking lovastatin, the first statin marketed in the US in 1987 (Tolman, 2002). Since then, all product information leaflets of statins advise to monitor liver enzymes within twelve weeks of therapy induction.

Treatment alternatives can be divided into two groups: therapy similar to statins, which regulate serum lipid levels, and other CHD risk reducing therapies. Although other cholesterol lowering drug classes were clinically effective in individuals with established CHD, the benefit was less significant than with statins and, most importantly, an increased rate of mortality was found for their use in primary prevention (Smith *et al*, 1993). Dietary changes are the most frequently suggested alternative. However, compared to a reduction of the cholesterol level by 25% with statins, the extent of reduction by using dietary change was found to be modest (2-9%)

(Ebrahim *et al*, 1999). Other therapies aim to reduce CHD by targeting other risk factors associated with it such as hypertension, smoking, obesity or diabetes. Although treatment for hypertension does not require blood tests necessary for the statin therapy, the intervention is seen as less effective in terms of prevented cardiovascular events. Because cholesterol level is an independent risk factor, its reduction means a further decrease of morbidity and mortality (British Cardiac Society, the British Hyperlipidaemia Association, the British Hypertension Society, endorsed by the British Diabetic Association, 1998).

3.2.2.2 National regulations of access to treatment with statins

The Standing Medical Advisory Committee guidelines

Probably the most important governmental document on the use of statins was the Standing Medical Advisory Committee (SMAC) recommendations in 1997 (see Table 3.2.3). It was announced around two years after the publication of the first two large RCTs investigating the use of statins in primary and secondary prevention (Scandinavian Simvastatin Study Group, 1994; Sheperd *et al*, 1995). By the time of the SMAC publication the expenditure on cholesterol-lowering drugs had resulted in nearly a six-fold increase, from £20 million in 1992 to over £113 million in 1997 (NHS Centre for Reviews and Dissemination, University of York, 1998).

The SMAC concluded that treatment with statins results in a reduction of morbidity and mortality of CHD in patients with established CHD (secondary prevention) as well as in people without symptoms but at certain risk to develop the disease in the future (primary prevention). Although there is a treatment benefit in both risk groups, the guidelines recommended that treatment should be targeted at those patients with a cholesterol level of 5.5mmol/l and who have signs of CHD or in individuals who have an annual risk of 3 % or more of developing CHD. The committee stated two arguments for these recommendations. The first argument was the absence of data about long-term safety. It was argued, "the balance of benefit and risk has not been determined for treatment of longer duration. This is unlikely to be important when treating those at high risk of major coronary events – we suggest 3% per year or more – but is more significant when considering treatment of people with a relatively low

CHD risk" (SMAC, 1997). The second argument for the choice of the risk level of 3% was that cost-effectiveness was judged to be low.

Table 3.2.3: Key events related to the governmental decisions about advising on the use of statins

Date	Event
1989	Simvastatin launched in the UK.
1990	Pravastatin launched in the UK.
1993	Fluvastatin launched in the UK.
1996	NHS Executive South and West, Research & Development report (1996) "Who benefits from statins? Revisiting the evidence from a purchasing perspective?" Recommendation only for treatment in secondary prevention, but stressing importance of further studies on cost impact in the NHS.
1997	Standing Medical Advisory Committee (SMAC) (1997) "The use of statins" Recommendation of statin use in secondary prevention; in primary prevention statins are only recommended if annual risk of CHD > 3%.
1997	Atorvastatin launched in the UK.
1998	British Cardiac Society, British Hyperlipidaemia Association, British Hypertension Society, endorsed by the British Diabetic Association (1998) "Joint British recommendations on prevention of coronary heart disease in clinical practice" Recommended for statin treatment in secondary and primary prevention (>2% annual risk of cardiovascular risk), however acknowledged that due to cost implication primary prevention only possible for patients >3% annual cardiovascular risk.
1998	NHS Centre for Reviews and Dissemination (1998) "Cholesterol and coronary heart disease: screening and treatment." No definition of a specific threshold for asymptomatic people was given in the report because of the absence of a consensus on their use in this patient group.
1999	Ebrahim *et al* (1999) for the NHS Research and Development HTA programme "Health Technology Assessment – What role for statins? A review and economic model" Recommendation to use statins for secondary prevention; recommendation for primary prevention for a baseline risk of >3%.
2000	National Service Frameworks (2000) "National Service Frameworks on coronary heart disease" Treatment recommendation for secondary prevention, primary prevention only if >3% per year and total cholesterol between 5-7.9 mmol/l.

The committee estimated the percentage of the British population, which would be eligible for treatment under the defined criteria, as follows:

- for secondary prevention: 4.8% aged 35-69 (5.9% men and 3.6% women);

- for primary prevention: 3.4% aged 35-69 (5.7% of men and 0.4% women).

It was recommended that before treatment with statins is considered life-style changes should be implemented if necessary. It was also recommended to use the Sheffield table based on the Framingham data to estimate the risk of CHD development. The Framingham data were gained from a large, prospective, epidemiological study in the US, gathering data about life-style and the prevalence and mortality from CHD.

Two other important documents prior to the publication of the SMAC guidelines, the NHS Executive South and West Research and Development report (1996) and a publication by the Trent Institute for Health Service Research (Working Group on Acute Purchasing, 1996), also recommended the provision of treatment in secondary prevention (see Table 3.2.3). In terms of primary prevention no consensus was found and local decision-making was recommended. Two years after the publication of the SMAC guidelines a HTA was published, which was in line with the SMAC guidelines recommending. It recommended funding of treatment for individuals with an annual CHD risk of 3% (Ebrahim *et al*, 1999).

National Service Framework

The more recently published National Service Framework on CHD (National Service Frameworks, 2000) includes the current guidelines on statins treatment. The guidelines refer to the SMAC guidelines and do not change the eligibility criteria in principle (only the cholesterol level is defined >5mmol/l instead of >5.5mmol/l). It argues: "This NSF sets a practical threshold of risk that will target resources at those in greatest need." It continues in stating that the treatment benefit of individuals, the total number of people eligible for treatment, cost-effectiveness and total resource implication of the policy all influenced the choice of this threshold. However, whereas the SMAC found little evidence to support that use of statins in older people the NSF includes people up to the age of 75 for primary prevention.

The NSF based its rationale for the treatment recommendation on the data of three large RCTs (Scandinavian Simvastatin Study Group, 1994; Sheperd *et al*, 1995; Sacks *et al* 1996). Two of them had already been published at the time of the SMAC guidelines. Regarding cost-effectiveness the framework refers to work done by Pickin

et al (1999) which concludes that on the ground of current prices statin treatment is cost effective for secondary prevention and only for primary prevention in the case of an annual CHD risk of > 3%. If treatment would be made available for all who could benefit (>1.5%of annual CHD risk) 90% of the current drug cost expenditure in the community have to be spent on statins. The NSF refers to another review, which argued that, beside cost-effectiveness, treatment of a large population could result in the potential stigmatisation of people as "being ill" who are otherwise feeling healthy (NHS Centre for Reviews and Dissemination, 1998).

Although the document discusses treatment alternatives to lower cholesterol such as hypertension and smoking cessation it states that the cholesterol level is an independent risk factor and that treatment should be considered in addition to other interventions.

3.2.2.3 Debate of stakeholders about the use of statins

Medical professional bodies

One year after the publication of the SMAC guidelines a group of several large professional organisations issued joint recommendations on the prevention of CHD including statin therapy (British Cardiac Society, the British Hyperlipidaemia Association, the British Hypertension Society, endorsed by the British Diabetic Association, 1998). They clearly stated that treatment with statins in primary prevention was beneficial at a level of 1.5% or higher annual risk of developing CHD, although they acknowledged that "the cost for medical service would be considerable". Other professionals criticised more vehemently the large discrepancy between the British national guidelines and the recommendations of professional bodies such as the European Arteriosclerosis Society or the European Society of Hypertension, which set the treatment threshold by 1.5-2% of annual CHD risk (Reynolds, 1997).

At the time of marketing of the first statins there was considerable professional debate about the potential risks of a low cholesterol level causing cancer or suicide (Smith *et al*, 1992). Alarmed by the publication of reports about an increase of mortality with the use of other types of cholesterol-lowering drugs some British physicians called for cautious use including the newly marketed simvastatin (Mulldoch, 1990). However,

this was later rejected by referring to study results which indicated that low cholesterol was secondary to the underlying illnesses such as infectious disease, cancer or alcoholism resulting in death (Jacobs *et al*, 1997). No support was found for a potential direct association between a low blood cholesterol level and an increased risk of violent death (Stamler *et al*, 1999).

The cost implications of statin therapy were another issue in the professional debate. Beside the national recommendation on the use of statins there had been several other publications – some by professional bodies - on the use and the estimated costs of statin therapy (Faculty of Public Health Medicine of the Royal College of Physician, 1997). Whereas treatment in secondary prevention was regarded as cost-effective, the decision about treatment in primary prevention was seen as "a matter of policy not an argument about evidence" (Fahey, 1998). It was estimated that for an average Health Authority to treat all individuals eligible according to the SMAC guidelines, an expenditure of £8 million would be required, which was around 20% of their drug expenditure at the time (Freemantle *et al*, 1997). The professional discussion reflected the uncertainty about how to evaluate the 'prospective need' of statistical people as compared to the immediate needs of real people.

Pharmaceutical industry

Some research initiatives partly funded by the pharmaceutical industry coincided with the debate about recommendation of long-term treatment with statins and an increased interest in aspects around adherence to treatment (Royal Pharmaceutical Society of Great Britain and Merck Sharp & Dohme, 1997). That adherence to statins in long-term treatment is of relevance has been shown by studies which reported a decline of more than 25% in the first 6 months after the original prescription (Brenner *et al*, 2002). Comparing different risk groups, the results indicated that after two years of follow-up 40% of patients with a history of an acute coronary event, 36% with a known coronary disease and 25% without detectable manifestation of CHD were adherent (Jackevicius *et al*, 2002). Non-adherence has been discussed as the cause of lower effectiveness than that calculated from clinical study data and resulting in disinvestment (Cleemput and Kesteloot, 2002).

Another company introduced a so-called 'outcome guarantee' for the use of its statin (Pharmaceutical Journal, 2001b). If the target cholesterol blood level is not achieved the company would calculate rebate and a payment would be made to the regional health care provider.

Public and media

There is little information about the extent to which individual patients demand treatment with cholesterol lowering drugs. This might be due to the unawareness of the public about the treatment options available and of the impact of non-treatment on the risk of developing CHD (McConnachie *et al*, 2001). In a British survey eight out of 10 did not know nor were worried about their cholesterol level (Feinmann, 2001). In contrast, in the United States the direct-to-consumer advertising of prescription-only medicines had major influence on the awareness of patients for specific medicines including statins (Holmer, 2002).

In the British media the potential cost implications of statin therapy were called "another unexploded bomb that cannot be defused by talking of lifestyle drugs" such as sildenafil (Stuttaford, 1999). Referring to the increased risk of CHD for people in socio-economic disadvantage groups, statins were regarded as having the potential to "reverse many of the disadvantages brought by poverty" (Stuttaford, 2000).

3.2.2.4 Summary of the debate about access to statin therapy

In contrast to rivastigmine statins are used in prevention. Furthermore, whereas the focus of the rivastigmine debate was around effectiveness in clinical practice, the financial impact of providing therapy for all individuals who could benefit was the main issue in the discussion about access to treatment with statins. The long-term safety was another important issue in the debate. Pressure from patients demanding access to treatment was not an issue in the debate. This is distinct from the decision-making about sildenafil, which is discussed in the following section.

3.2.3 Sildenafil (Viagra ®)

3.2.3.1 Introduction

Sildenafil (Viagra ®) was the first drug licensed specifically for the treatment of erectile dysfunction (ED), a condition which occurs in around 10% of men (around 2.3 million in the UK) (The Impotence Association, 2001). Whereas previously a psychogenic cause of ED was diagnosed in 90% of patients, studies from the past decade have shown that in the most sufferers an organic cause could also be attributed (Rosen, 1996). In 25% of cases ED was drug induced (Drug and Therapeutic Bulletin, 1998).

Five months after its licensing in the US in April 1998 sildenafil obtained its market authorisation in the UK and many other European countries (Food and Drug Administration, 1998; Committee for Proprietary Medicinal Products, 1998). Most of other medical interventions such as injection of a combination of drugs, vacuum pumps and surgery used in ED are invasive and/or more expensive than sildenafil (Wilt *et al*, 1999). Since sildenafil is administered orally, it is more user-friendly than many other therapeutic agents, which makes it suitable for use in patients in primary care (Bellinghiere *et al*, 2001). Most common side effects are headache, facial flushes and transient visual symptoms (Drug and Therapeutic Bulletin, 1998). Contraindications include the use of nitrates, which can cause severe hypotension when used together with sildenafil (Langtry and Markham, 1999).

3.2.3.2 National regulation of access to treatment with sildenafil

The next paragraph will briefly describe the historical events after licensing of sildenafil in Europe with a focus on the governmental statements regarding its use in the NHS (see Table 3.2.4). The chain of events illustrates the context in which the decisions were made.

Table 3.3.4: Reports and guidelines on the use of sildenafil

Report/ Guideline title	Date	Recommendation
"Prescribing of sildenafil (Viagra)" (DoH*, 1998a)	Sep 1998	Advice given to Health Authorities not to support provision of sildenafil at NHS expense.
"Advice from the SMAC** on the use of Viagra (sildenafil) in the treatment of impotence (erectile dysfunction) " (DoH, 1998b)	Nov 1998	▪ No medical reason was found why sildenafil should be not available on the NHS. ▪ Sildenafil should be prescribed by GPs and referral to a specialist only made where appropriate. ▪ Any decision should take into account the equity of access as well as the availability of resources. ▪ The judgement about reimbursement should be based on the predisposing condition, severity and the effect of the condition on the patient's life.
"Viagra ® – NHS prescription proposals announced" (DoH, 1999d)	Jan 1999	Groups of men who would be eligible for treatment according to the proposal: ▪ Male patients who had radical pelvic surgery or their prostate removed; ▪ Suffering from spinal injury; ▪ Diabetes; ▪ Men with multiple sclerosis; ▪ Men with single gene neurological disease; ▪ Men who are severely distressed by impotence after a special assessment in a hospital.
"Sildenafil (Viagra)" HSC+ 1999/042 (DoH, 1999e)	Feb 1999	▪ The government emphasised that the announcement made in September 1998 are guidance only
"Treatment for impotence" HSC 1999/115 (DoH, 1999f)	May 1999	▪ Final guidance on the use of sildenafil. ▪ During a consultation period of six weeks 861 responses were received. ▪ Extension of the condition for which sildenafil can be prescribed: inclusion of men suffering from prostate cancer, Parkinson's disease, spina bifida, polio, renal failure and men who were treated for ED before the 14 September 1998.
"Treatment for impotence" HSC 1999/148 (DoH, 1999g)	Jun 1999	▪ The government summarises final legislation which comes into force on 1 July 1999 and advising on the number of sildenafil prescribed for an individual patient (once a week).
"Treatment of impotence – patients with severe distress" HSC 1999/177 (DoH, 1999h)	Aug 1999	▪ Definition of criteria which should guide the assessment of patients who are severe distressed.
"No change for guidelines on GP prescribing of NHS impotence treatment" (DoH, 2001d)	Oct 2001	▪ Confirmation of the current guidelines on use of sildenafil in the NHS which will be unchanged. ▪ Expenditure for the treatment of ED has increased over the last years and is at £25 million per year. ▪ From 1st of November new ED function treatment Uprima ® is, as sildenafil, included in Schedule 11 for restricted use.

* DoH: Department of Health, ** SMAC: Standing Medical Advisory Committee, + HSC: Health Service Circular

The first UK governmental announcement was made in September 1998, a day before sildenafil obtained its marketing authority across Europe (Department of Health,

1998a). The government sent out an interim message and advised all health authorities and GPs "not to support the provision of sildenafil at NHS expense to patients requiring treatment for ED, other than in exceptional circumstances". It was stressed that the final decision about use of the sildenafil in the NHS would be made "within the next weeks".

In August 1998 the Standing Medical Advisory Committee (SMAC) was asked by the government to give an interim advice on the use of sildenafil in the NHS (Standing Medical Advisory Committee, 1998). The recommendations of SMAC were sent to the government in November 1998. The committee saw no clinical reason why sildenafil should be not used for the treatment of ED within the NHS. The committee suggested that in the light of limited resources it "could be successfully targeted at men with the greatest clinical need". Suggested criteria to evaluate the clinical need were predisposing condition, severity and the effect of ED on the patient's life.

It was noted that the effectiveness of sildenafil varies between different predisposing conditions with less effect following prostatectomy. In terms of severity it was emphasised that the assessment depends on patient self-report. The committee decided not to recommend eligibility criteria regarding severity based on the number of failed intercourse over a certain amount of time. This was because the committee thought patients would be tempted to quote that figure in order to have free access to treatment. No medical reason was found to limit the amount of sildenafil used by an individual in a certain period of time other than the restriction imposed by the licensing authority to once a day. Instead, the SMAC proposed to use the result of a survey that reported that the frequency of sexual intercourse in the group of 40 to 60 years old British men was once a week, as a guide to limit the amount of sildenafil prescribed. The committee summarised that "any decision should take into account equity of access as well as availability of resources".

Finally, in January 1999 the government announced its proposal for the use of sildenafil with a six-week consultation period (Department of Health, 1999d). It was proposed that sildenafil and other types of ED treatments should be restricted to six conditions causing ED (radical pelvic surgery or prostate removal, spinal cord injury, diabetes, multiple sclerosis and single gene neurological disease). If ED is causing

severe distress the general practitioner should refer the patient to a specialist. In a later document the diagnosis of severe distress was defined as "significant disruption to normal social and occupational activity, marked effect on mood, behaviour, social and environmental awareness and marked effect on interpersonal relationships" (Department of Health 1999h).

The final decision of the government regarding the use of sildenafil in the NHS was published in May after the consultation period in which 861 replies were received (Department of Health, 1999f). Most of the replies agreed with some form of restriction but did not support the defined criteria set up by the government. In the absence of any proposed alternatives on how to restrict the access to sildenafil the government decided not to change its first proposal. However, it announced that some additional conditions would be included such as men suffering from prostate cancer, renal failure treated with transplant or dialysis, polio, spina bifida, Parkinson's disease or severe pelvic injury. The restriction on sildenafil and other forms of ED treatments came into force on 1 July 1999 under an amendment of Schedule 11 of the NHS (General Medical Service) Regulations 1992. As mentioned in Chapter 1 (Section 1.5.1) Schedule 11, the so-called "grey list", lists those medicines which can only be prescribed on NHS expense for certain indications.

Nearly two years later the Health Minister announced that the restriction of sildenafil and other ED therapies would continue to be in place (Department of Health, 2001d). Newly marketed therapies of ED such as sublingual apomorphine (Uprima ®) would also included in Schedule 11 of the NHS (General Medical Service) Regulations 1992.

3.2.3.3 Reaction to the governmental decision about access to sildenafil

The governmental statements published in 1998 and 1999 produced great public attention since it was the first time that a British government restricted nationally the use of a drug immediately after market authorisation. The statement caused significant confusion. Some Health Authorities believed the guidelines to be mandatory rather than advisory and banned the use of sildenafil (Henderson, 1998).

Whilst the interim guidance from 1998 was in place, it was criticised that other "more expensive, and eye-watering, anti-impotence treatments such as injections into the penis and pushing pellets into the urethra" were funded whereas "a safe, less expensive and non-invasive treatment was excluded" (Shallcross, 1999). The Drug and Therapeutic Bulletin (DTB) similarly argued in the same way that if other treatments are funded there was no rational argument against availability of sildenafil in the NHS (Drug and Therapeutic Bulletin, 1998).

The main arguments used against the final governmental action were not the restriction of availability of sildenafil per se. It was more the fact that the decision was inconsistent with some of the main principles of the British health care system such as equity and treatment based on the need of each individual patient. For instance the criteria of eligibility for treatment excluded patients suffering from ED caused by hypertension who might have the same need for treatment as a patient with ED due to one of the eligible conditions. In addition, treatment of patients with physical disorders received priority (access to treatment in primary care) over the ED treatment of psychogenetic origin (access to treatment only after referral to a specialist). The defined criteria did not promote the treatment on the basis of medical need (Bosanquet, 1999).

Since the governmental decision was delayed and frustration about the inaction of the government grew, the General Practitioners Committee (GPC) published its own internal guidance. It stressed the importance of a thorough assessment of the patient to rule out undetected, underlying, potentially severe conditions and to consider the use of resources. After this assessment sildenafil should be offered if appropriate. Regarding the proposal of the government in January 1999 the British Medical Association stated, "it is wholly unethical to distinguish between patients according to the cause of their erectile dysfunction" (British Medical Association, 1999).

After the governmental proposal in January 1999 the opposition accused the government in parliament of defining the eligibility criteria so that only 15% of patients suffering from ED would be eligible for treatment with the aim to keep expenses at a certain level (Commons Hansard, 1999a). "For the first time, he (the Secretary of State) has proposed making a drug or treatment available to some people

only, on a basis of cost rather than need" (Commons Hansard, 1999a). Referral to a specialist as a gatekeeper for the diagnosis of severe distress caused by ED and following treatment was regarded as protection against abuse. However, sildenafil was regarded as a drug particularly suitable to be prescribed by GPs since they know the medical history of the patient and are able to follow-up the patient (Select Committee Report, 1999). In contrast to other European countries, the opposition characterised the role of the British government as ambivalent in promoting new investments of the pharmaceutical industry and providing and securing jobs, while on the other hand it sought to ration access to health care (Commons Hansard, 1999b).

The final governmental decision to restrict the use according to underlying condition was also criticised by the Impotence Association (The Impotence Association, 2001). The defined criteria were seen as arbitrary and the Association demanded, "All impotence sufferers, regardless of cause, should have equal access to impotence treatments within the NHS primary care system".

The criticism led to the company's successful challenge of the interim guidance in the High Court (Dyer, 1999). The duty of the physician to use their clinical judgement in caring for their patients was restricted by the interim guidance, which was against English law. The interim guidance was also in conflict with European law, which stated that any restriction of a drug has to be justified on the basis of "objective and verifiable criteria".

Others warrant that a possible consequence of restricting the use of sildenafil may be an increase in the number of patients needing treatment for depression and social counselling (Biggs and Mackenzie, 2000). That would mean spending of resources in social care instead of health care and failing to achieve cost savings.

3.2.3.4 Media reports

Since the time of its launch in the US sildenafil has received enormous media interest (Hall, 1998). In Britain the overall tone in the media was that some kind of rationing was necessary (Boseley, 1999). However, the existence of rationing should be made explicit and with public involvement. It was argued that excluding patients on the basis

of the cause of ED would mean that only patients who can afford it would receive treatment for this indication. This would be in contrast to the principles of the current health care system delivering equal and free access at the point of delivery (Halpern, 1999).

3.2.3.5 Other reports about access and use of sildenafil

Many reported 'abuse' of sildenafil for various reasons such as healthy men who believe it would increase their sexual desire or their sexual performance and men who suffer from ED caused by alcohol or illegal drugs (Downie *et al*, 2000). Another group reported to take sildenafil outside its licensed indication were women who suffered from sexual disorders who assumed that the drug would treat their dysfunction (Aldridge and Measham, 1999).

A British survey amongst nightclub customers found that 3% had used sildenafil as a recreational drug bought from friends, dealers, sex shops or the Internet (Aldridge and Measham, 1999). Widely offered sales over the Internet with large advertising campaigns have promoted easy assess (Armstrong *et al*, 1999). Despite potential abuse the embarrassment to talk face-to-face with a physician might be a contributing factor to prefer to order via Internet and bypassing the physician.

3.2.3.6 Summary of the debate about access to sildenafil

In many ways the decision about sildenafil was exceptional. The marketing of the drug in the US received high media attention world wide and created debate about sexual disorders. Additionally, it triggered a unique British debate about the explicit rationing of health care and the purpose of the NHS. The government became involved in the decision about reimbursement of an individual medicine and implemented legal changes. The parliamentary debate shows many characteristic features of policy-making in the NHS. For example, the role of the government in encouraging investment of the pharmaceutical industry was seen in conflict with achieving cost containment of NHS expenses. The British government has been criticised for the lack of transparency in the decision process, its delay as well as for its arbitrary exclusion of patients with ED based on their underlying conditions. Many professionals and

members of the public perceived the way of rationing as "muddling through" and called for necessary changes in rationing decisions in the future.

3.3 Discussion of the results of the document analysis

The results of the document analysis presented in this chapter give insight into various aspects of decision-making about access to drug therapies in the NHS. In addition to the description of the decision-making bodies, the outcome of the decisions and their main information sources, it was possible to identify several rationales (including social and ethical issues) which were used as legitimisation of the decisions. In addition, the analysis revealed influencing factors and several key arguments used in the debate about access to the three drug therapies. Table 3.3.1 gives an overview of these aspects identified.

The document analysis shows that access to the three drug therapies was regulated by national guidelines defining the patient groups eligible for treatment. The criteria defining access to treatment are clinical or diagnostic markers as well as the aetiology of the condition, which give the impression of objectivity and accuracy. However, as it will be discussed below, a close analysis of the access criteria shows that they have significant limitations. Furthermore, the results of the document analysis showed that the evidence which was used as the information basis for the decisions, was far weaker and open for individual interpretation than the guidelines and the defined access criteria suggest. In other words, some of the medical and financial effects such as benefit of the therapy in clinical practice, safety and cost implications were largely unknown. Apart from benefit, safety and cost considerations, responsibility or solidarity were powerful arguments in the debate about access to treatment.

Table 3.3.1: Summary of aspects of decision-making about the three drug therapies

Issues of debate	Statins	Sildenafil	Rivastigmine
Outcome measures chosen to assess effectiveness	Reduction of CHD morbidity and mortality in the population	Satisfaction of the patient with the medicine	Cognitive and global outcome and partly quality of life
Number needed to treat (NNT)	25/14 (to prevent one death/ non-fatal myocardial infarction) in secondary prevention (Scandinavian Simvastatin Study Group, 1994) 143/50 in primary prevention (Shepherd *et al*, 1995)	3/2 (actual intercourse/ global improvement in erections) Moore *et al*, 2002)	13/ 4 (improved of 4 points on the ADAS cog / prevent decline of at least 7 points on the ADAS cog) (Livingston and Katona, 2000)
Issues related to safety	Limited data about long-term safety	Pharmacological interaction with nitrates	Individual reports about severe side effects
Social impact of the therapy	Medicalisation of adults who are feeling well	Abuse potential, 'life-style' drug	Delay of institutionalisation would result in potential savings of public expenditure but higher burden for individual carer(s)
Costs of therapy	Costs limit access	Costs limit access	Costs limit access as well as the establishment of diagnostic and treatment monitoring services
Treatment alternatives	Alternative therapies less attractive or effective and some cholesterol lowering therapies were found to increase mortality	Alternatives more expensive, inconvenient or unpleasant to use	No other treatment alternative other than drugs from the same class (e.g. donepezil, galantamine)
Questions regarding evidence base and knowledge	Long-term safety	Safety to give access without prescription	Effectiveness in clinical practice, timing of discontinuation
Access criteria	Risk of developing CHD	Cause of ED	Degree of cognitive function, effectiveness of treatment and adherence to treatment
Access according to severity of the condition	More severe cases are prioritised: patients already diagnosed with CHD	Only marginally: patients suffering from severe depression due to ED are prioritised. However, access to treatment of ED of physical origin is not provided on the basis of the severity of the condition	Not relevant

Table 3.3.1 (continued): Summary of aspects of decision-making about the three drug therapies

Issues of debate	Statins	Sildenafil	Rivastigmine
Access based on the ability to benefit	Access based on the ability to benefit in terms of the absolute risk reduction.	Not based on outcome benefit.	Access based on the ability to benefit in terms of cognitive function, i.e. absence of deterioration.
Patient/public demand potentially influencing access	Less patient demand	Influenced access and restriction	Elderly patients less demanding, more relatives and carers who demand for them
Authorising agency	NSF	Governmental decision	NICE guidance (NSFs referring to NICE guidance)
Official legitimating of gatekeeper function	Safety and tailoring access to patients most in need	Protection against use for non-medical purposes, safety	Need to monitor therapy
Potentially important driving factors for the decision	Safeguarding cost containment	Safeguarding cost containment	Safeguarding cost containment, solidarity with the patients and carers
Individual versus social responsibility of funding treatment	Partly social responsibility	Individual responsibility	Social responsibility, support of carers in particular
Dominating interest groups	Physicians	Patients	Relatives and patient organisations

3.3.1 Access criteria

Instead of providing access to all patients who can potentially benefit from treatment, access to all three drug therapies is limited to a certain patient group. In the case of rivastigmine the diagnostic tool of the MMSE is used to define access. Access to statins is based on the prediction of the annual risk of developing CHD and in the case of sildenafil access is defined by the underlying cause of ED. The MMSE, the prediction of the annual risk of CHD and the aetiology of ED have in common that they are easy to obtain in clinic practice. For instance, it takes around 10 minutes to complete the MMSE and does not require special training of the HCP performing the test (Burns *et al*, 2002). The prediction of the CHD is based on factors such as blood pressure, smoking habits and diabetes, which are assessed routinely in primary care. The aetiology of the ED such as multiple sclerosis, Parkinson's disease and diabetes is usually known to the GP of the patient suffering from ED. Although the measures give the impression of certainty and accuracy, the analysis of the measures and that of the their social implications on access show several limitations, which will now be discussed in more detail.

3.3.1.1 The MMSE score

In the case of the MMSE, access is based on a proxy measure for the patients' abilities in daily life. The MMSE was developed to fulfil two roles: first, as a brief screening test to assess quantitatively the severity of cognitive impairment, and second, to assess patients over time (Tombaugh and McIntyre, 1992). The instrument consists of 30 items, where 30 points is the highest possible value and is equivalent to no cognitive impairment (Folstein *et al*, 1975). The test assesses immediate and delayed recall, constructional ability, language, attention and concentration as well as orientation to time and place (Salmon *et al*, 1990). It has been argued that the diagnostic use of the MMSE does need to take into account age, language and educational variation. Study results that also cultural background could influence the outcome are inconsistent (Murden *et al,* 1991). It has been suggested that the MMSE be only recommended for individuals who are fluent in English and have an 8^{th} grade of education or more. NICE acknowledged the limitation of the MMSE test regarding the first language of the patient in stating "it is not without problems and reliance on this assessment is

difficult in people whose AD is complicated by dysphasia and whose first language is other than English" (NICE, 2001).

However, further limitations of the measurement have not been acknowledged in the guidelines such as inter-rater variations or limited sensitivity below 15 on the MMSE scale, which can potentially result in exclusion of patients who could benefit from treatment with rivastigmine. For instance, even if the MMSE is used by the same clinician, statistically significant variations between measurements have been found (Doraiswamy and Kaiser, 2000). For a group of 1648 AD patients assessed on two visits separated by 1-2 weeks a standard deviation of 2.1 points on the MMSE scale of 0 to 30 was found, which could not be explained by learning effect of the individuals or clinical improvement. This means that a patient assessed by a HCP may be excluded from access to rivastigmine treatment on the basis of the MMSE result below 12 whereas some weeks later the same patient with a result above 12 would be eligible to receive treatment according to the NICE guidelines. Since significant variations in annual score and measurement errors were found it has been suggested that only variation of more than three points on the MMSE scale are clinically significant and that measurements need to be done over at least three years to be meaningful (Clark *et al.*, 1999). Other scales, for example the Dementia Rating Scale (DRS), have shown more response to small changes in severely cognitive impaired patients (Galasko *et al*, 1991; Salmon *et al*, 1990). Overall, the MMSE score might give the illusion of an exact and reproducible measurement.

3.3.1.2 The assessment of annual risk to develop coronary heart disease (CHD)

Regarding the insufficient sensitivity of the MMSE there is some parallel to the use of measurement tool to define access to statin treatment, which can potentially results in unequal access. For example, there is evidence that older patients, smokers and patients with South Asian origin are less likely to receive statins (Reid *et al*, 2002; Patel *et al*, 2002). Additionally, studies reported a considerable difference in prescribing patterns between various GPs as well as differences in prescribing between socio-economic groups (Baxter *et al*, 1998; Bradshaw and Walker, 1997). It is possible, for example, that those in lower socio-economic groups might be doubly disadvantaged here – both because the general guidelines may not be sufficiently

sensitive to their degree of relative risk, and because GPs who work with them are placing other needs first. This creates questions on how sensitive the current access criteria are to identify those most in need.

Currently, there is controversy about the most precise tool to assess CHD in individuals in Britain. One commonly used source for the prediction of developing CHD is the Sheffield table, which is based on the Framingham function derived from large epidemiological data gathered in the US. On one hand, several authors have discussed the limitations of the Framingham formula to establish the individual risk to develop CHD, in particular, that the formula is less precise to predict the risk for individuals with Asian ethnic background and with family hypercholesterolemia (Colhoun and Fuller, 1999; British Cardiac Society, the British Hyperlipidaemia Association, the British Hypertension Society, endorsed by the British Diabetic Association, 1998; Cappuccino *et al*, 2002). On the other hand, a recent paper highlights that the risk of developing CHD in British men will be overestimated if based on the Framingham function (Brindle *et al*, 2003).

That older people were found less likely to receive statins might be due to the absence of RCTs investigating the effectiveness of statins in older people. In the NSFs treatment is now recommended up to an age of 75 years since a large RCT showed beneficial effects in patient groups, which were previously underrepresented such as patients aged 75 of years, women and individuals with diabetes (Heart Protection Study Collaborative Group, 2002)

3.3.2 Uncertainties in the debate about access

Despite the apparent certainty and objectivity of the access criteria a critical analysis reveals that there are significant uncertainties related to the evidence which were not acknowledged in the guidelines. For example, in the centre of the discussion about the funding of treatment with rivastigmine there was the debate about a reliable and valid measurement to assess treatment outcomes. Most of the RCTs were used in the licensing process, which indicates that the policy-makers were largely relying on data that were used for the application to obtain market authorisation. Hence, the choice of the trial design was shaped by the requirements of the licensing agencies compared to

the ones of policy-makers. Since policy-makers required data about effectiveness of the therapy, they questioned the clinical meaning of the outcome measures and were unsure about how to judge the impact of treatment on patients, carers and society in terms of quality of life and cost implications.

In contrast to rivastigmine, in the case of statins the uncertainty was related to the lack of data about long-term safety. That the debate about side effects might be more vivid than for other drugs is probably related to their use in prevention, where the benefit/risk ratio is considered to be small. Additionally, statins are drugs used long-term and exposure to side effects is potentially for decades, concerning a large group of the population. Part of the medical profession were worried about the potential side effects of statins and policy-makers used this as an argument to restrict their use to individuals with higher risk of CHD and a greater expected total benefit. There is still ongoing debate over the use of statins, which will potentially influence future policy decisions about access to treatment with statins. One of the adverse events – rhabdomyolysis – will be used to illustrate the ongoing debate about the safety of statins. As with other drugs, safety cannot be assured.

For instance, the withdrawal of cerivastatin from the market in 2001 - due to the drug induced rhabdomyolysis resulting in renal failure - created a new debate over the safety of statins. The case of cerivastatin showed that even if the molecular structure is similar between drugs their safety profile could be very different. In 12 out of 31 deaths reported to the FDA in connection with statins and rhabdomyolysis cerivastatin was used (Staffa *et al*, 2002). The FDA data indicated that the reporting rate for fatal rhabdomyolysis with cerivastatin monotherapy was 10 to 50 times higher than with other statins (1.9 death per million prescriptions) (Omar *et al*, 2001). In cases where cerivastatin was used outside its licensing in combination with gemfibrozil the rate was even higher. Nevertheless, it was concluded that the other statins – if used according to the manufacturers recommendations – are regarded as safe.

Apart from safety considerations much of the technical controversy about the availability and use of statins relates to the problems of evaluating its cost-effectiveness and also to the considerable variation in estimated cost-effectiveness between secondary and primary prevention. The high relevance of cost considerations

was similar to sildenafil, where the debate was dominated by the fear about future cost escalation. In addition, similar to rivastigmine, there was debate about the assessment of cost-effectiveness of sildenafil, first, because of problems in measuring and incorporating quality of life implications, and second, due to problems in drawing the boundaries of health care and the boundaries between health care and social care.

3.3.3 Solidarity, responsibility and demand

Apart from considerations about benefits, safety and cost implications of the therapies, solidarity and responsibility were powerful arguments in the debate. The decision about rivastigmine illustrates that the appeal of solidarity with the patients and with the carers was used to advocate access to treatment. The publications of the media have drawn the picture of neglected and vulnerable older people suffering without dignity, which was echoed by the Alzheimer's Society emphasising the importance of 'solidarity' with the patients as well as the carers. Giving access to treatment might bear some element of compassion with the carers and families and recognising the potential need for more social support since patients, carers and their families carry the high burden of care including the financial costs of it. However, a delaying referral to nursing homes might not necessarily be in the interest of carers and family.

The issue of private and state responsibility in providing access to health care was frequently discussed at the time of the decision about access to sildenafil where many argued that funding of treatment is private responsibility rather than duty of a publicly funded health service. Overall, the debate about access to treatment with sildenafil was regarded as a prototype of a rationing decision about "life-style" drugs, where the boundaries between pleasure and health care need were difficult to define. A "life-style" drug was regarded as personal choice where the purchase is the responsibility of the individual. As the SMAC (1998) pointed out, diagnosis, severity and treatment outcome is commonly based on patient self-report. This makes it difficult to distinguish between the use of the drug for the treatment of ED or other than therapeutic purposes, which lay outside the function of the health care system. The definition relates to the concept of health and its cultural and social perceptions. Previously, ED was seen as a sign of normal ageing and in the majority of cases it was untreated (Marumo and Murai, 2001). This has changed over the last decades and

authors claimed the "medicalisation" of the condition (Gilbert *et al*, 2000). The professional and the parliamentary discussion highlighted that government policy served to strongly underline very specific constructions of 'health service need', and to contrast this with other constructions of need, individual wants or lifestyle questions. It was debated how far the governmental guidelines discriminated against conditions of psychological origin and how much the current access criteria were driven by pragmatic considerations of easy diagnosis.

It is surprising that there is little public discussion about offering private treatment with statins. Although the government announced its plans to make statins available over the counter without prescription by spring 2004 (MHRA, 2003), there has been no wide public debate as with sildenafil. Perhaps an open debate is thought to result in public awareness of the limitations of the NHS and undermining the public confidence in the governmental decisions made. NICE has announced a review of the access criteria to statin treatment in the NHS within the next year, which might result in an extension of the current guidelines (NICE, 2003b).

As seen in the case of rivastigmine, carers and relatives were strong interest groups in demanding treatment. In the case of sildenafil, it was the patients who demanded treatment. In both cases the demand was perceived as a threat that there would be uncontrolled cost escalation. For example, in the case of rivastigmine the media predicted the dilemma of HCPs in deciding about discontinuation of treatment, especially as there was no other therapeutic alternative which could be provided.

3.3.4 Legitimisation of the decisions

Despite the uncertainties found in the data used as the information base for the decisions, one main justification of the decision-making bodies regarding access to the therapies was effectiveness (that means 'what works'). This is in line with the directive of the NHS Executive to ensure that decisions are driven by clinical effectiveness apart from consideration of cost-effectiveness (National Association of Health Authorities and Trusts, 1995). For example, the main legitimisation for the reimbursement of rivastigmine was that the therapy is effective in terms of delaying institutionalisation, which would result into cost savings. If the access provision on the basis of

effectiveness would extend a certain budget allocated need was used as another legitimisation for the decision about access to treatment. For instance, need was used to limited provision of statin treatment so that only individuals already diagnosed with CHD were given priority compared to individuals without a visible sign of CHD even though both patient groups could benefit from treatment. Hence, the way in which decisions were legitimised gave the impression that research evidence such as data about effectiveness was directly implemented into policy decisions without much adjustment. In addition, providing access to treatment on the basis of need would guarantee fairness.

However, the results of the document analysis show a discrepancy between the definition of access criteria and the direct application of evidence of effectiveness and need for treatment. In other words, the access criteria are not necessarily reflected in the legitimisation used for the decision made. These aspects became most apparent from the analysis of the documents gathered from stakeholders who commented and analysed the decisions and highlighted some of these inconsistencies. Sildenafil is an example where the discrepancy has been most overtly discussed and the access criteria most heavily criticised. The access criteria defined on the ground of aetiology of ED contradict the principle of giving priority to effective treatment and to patients most in need. In the case of statins it has been highlighted that the access criteria might favour certain groups of the population and is not sensitive enough to detect need for treatment. In case of rivastigmine it seems that the interpretation of evidence regarding the effectiveness of treatment was made in favour of a reimbursement whereas in fact the data did not necessarily support that conclusion. Other factors, such as the high burden of the disease for patients and carers or the absence of any treatment alternatives, might have influenced the decisions.

The reasons for the discrepancies cannot be answered from the findings of the document analysis. Therefore, a different qualitative research tool was used to investigate the decision-making about reimbursement in order to gain more insight into the interplay of different factors, which might determine the final decision. The method that was used and the results that were obtained will be described in Chapter 4.

CHAPTER 4: IN-DEPTH INTERVIEWS WITH POLICY-MAKERS AND STAKEHOLDERS

4.1 Introduction

This chapter presents the second empirical study investigating authoritative decision-making in the NHS about access to medicines. Whereas document analysis was used in the first empirical study described in the previous chapter, in-depth interviews were carried out in the second study. In building onto the results presented before, the objective of the second study was to explore in detail the views of the professionals involved in or influencing authoritative decisions concerning

- the process of decision-making,
- the rationales used for the decisions made,
- the influencing factors and
- the potential dilemmas and conflicts.

As in the previous chapter, before presenting the results and the discussion, the methods will be addressed.

4.2 Methods

4.2.1 In-depth interviews

In-depth interviews were used as the second research method in the present study. The use of in-depth interviews is a common research method in social science, history and increasingly health care research (Bowling, 1999a). In contrast to document analysis, which is limited to the presentation of information which is intended for publication, in-depth interviews allow the exploration of views, attitudes and experiences of participants in detail. Additionally, the method is used to investigate the interviewees' understanding, their constructed meaning of events and their experiences (Grbich, 1999). In contrast to structured interviews, where all the respondents are asked the same kind of questions to minimise deviation and increase reliability, the researchers' questions are open ended and flexible within a certain range, which allows clarification of uncertainties in the answers of the interviewees. Some authors differentiate between

"unstructured" and "semi-structured" in-depth interviews (Silverman, 1993), where the latter are interviews conducted with a more detailed topic guide than unstructured interviews. However, the difference between them is not clearly defined. For this study an interview topic guide was used which listed areas of interest and questions of relevance. However, its use was adapted to the setting, which means that all topics of interests were not necessarily explored in each interview.

Commonly, qualitative research uses small sample sizes (Bowling, 1999a). In contrast to quantitative research, the aim is to investigate only some cases in depth rather than many superficially. In addition, sampling is carried out to maximise the variety of views held by the participants in order to present the range of views held and not the quantity. Sampling is done until redundancy, which means until no new major themes are retrieved from the data. Since sampling is not random, the result has no generalisability in a statistical sense (Smith, 2002). Studying cases in depth may enhance their validity. However, it is important to be aware that the answers of the interviewees are subjective accounts, which are shaped by the situational factors of their production (Bury, 2001). The limitation of the method will be discussed in more detail in Section 4.4.1.

4.2.2 Sample and participant recruitment

It was intended that the sample would represent a variety of decision-makers and observers: first, those who advise and make decisions inside the NHS on a national and regional level (policy-makers), second, those inside and outside the NHS who represent interest groups (stakeholders). Both, the sample of policy makers and the sample of stakeholders, will be described in more detail.

From the document analysis, four main groups of policy-makers involved in the decisions about rivastigmine, sildenafil and statins were identified. The first group were pharmaceutical advisors either working as members of a PCT prescribing committee or a regional New Drugs Group (NDG) in primary care, or a formulary committee in secondary care. They were familiar with gathering clinical evidence about the efficacy and cost-effectiveness of treatment under consideration. The second group was medical professionals working in PCTs, secondary care or regional

specialist commissioning groups (RSCGs). A third group were lay members, defined as individuals who were not appointed due to their profession. The final group were policy-makers in the function of being chair of a committee deciding about access to medicines, either in a trust or on a regional level.

The main stakeholders identified were medical professionals, pharmaceutical company representatives and patient organisation representatives. Hospital clinicians in medicine for the elderly and in urology were also contacted since they were stakeholders regarding the access to treatment with rivastigmine and sildenafil in secondary care. Professionals working in the industry or in a patient organisation could give information about the role of pharmaceutical companies or patient organisations in promoting access to treatment.

And finally, in addition to policy makers and stakeholders, one expert in health policy was identified who could contribute with the knowledge about the structure of the NHS and the historical developments of policies.

The aim was to interview at least two participants from each of the groups identified, in order to represent some diversity. Since some of the participants were members of two groups, a sample size of 20 participants was chosen. The majority of the participants contacted were involved in regional/ local decision-making (e.g. PCT prescribing committees or hospital drug and therapeutic committees), as this is the level in the NHS where most decisions about health care provision are made. The others were involved in or influenced national policy decisions.

For some of the interviewees a snowball and for the rest a purposive sampling strategy was used. First, four professionals (three pharmaceutical advisors and one expert in health policy), who were known to the researcher and working in the London Region, were contacted. Further subjects were sampled by asking the first group of four participants about other professionals working in different committees who were involved in the decision-making process about the three drug therapies of interest. Additionally, the first group of participants was asked if they knew where decisions had been made differently compared to their committee, to gather a variety of views. A snowball approach was chosen for two reasons: First, the researchers did not know,

who were the decision-makers involved in the decision-making about the three drug therapies of interest. In asking the first four professionals with an expertise in the field of decision-making about drug treatment it was likely that further informants could be identified with particularly good knowledge about the areas of interest. Second, in April 2002 primary care groups (PCGs) became PCTs and many professionals were changing positions. It was difficult to access and recruit participants. Contact with advisors already known to the researchers was easier in the time available. The disadvantage of this sampling technique was that participants were all from a specific network, which potentially undermined the sample diversity (Bowling, 1999b).

A purposive sample was used to select stakeholders including clinicians in secondary care providing therapy with sildenafil, statins and rivastigmine, and professionals working with the companies holding the market authorisation for the drugs of interest. The clinicians contacted were all based at a teaching hospital linked to the research institution of the researcher. One clinician in medicine for the elderly was chosen since he was also chair of a committee deciding about the entry of new drugs into the hospital formulary; the second clinician was selected randomly from a list of consultants working in medicine for the elderly. Another clinician was recruited due to his work with patients suffering from Alzheimer's disease (AD), including monitoring of treatment with rivastigmine. So the clinicians who were recruited had very different tasks (e.g. chair of a DTC committee, work with patients suffering from AD). However, they were all from the same centre and speciality which may limit the variety of the sample. The public affairs offices of the pharmaceutical manufacturers of sildenafil, rivastigmine, simvastatin, atorvastatin and pravastatin were contacted and asked for a person who would have knowledge about the area of interest. From the group of statins, simvastatin, atorvastatin and pravastatin were selected since they were the most frequently sold statins in Britain (Association of the British Pharmaceutical Industry, 2000).

The first contact to the potential participant was made via letter (addressing letter see Appendix 4.2.1). After that, potential participants were phoned and a date for an interview agreed.

4.2.3 Interview topic guides

Based on the main findings from the document analysis (see Chapter 3) topic guides for each of the main interest groups were developed (see Appendix 4.2.2 to Appendix 4.2.5), which were used as reminder during the interviews.

In the first part of the interview the participants were asked about their professional background, their work related to decision-making about access to drug treatment and their role in the decision-making process. Using the decision-making process concerning rivastigmine, sildenafil or statins as an example, committee members were asked to explain how a decision about the access to the therapy was made, which rationales were used, which factors influenced the decision, what types of conflicts they experienced and how they were solved. Stakeholders were asked about their views of the health policy decisions concerning a drug or drug class related to their area of practice and the way in which they voiced their interests during the decision-making process. Probing was carried out by asking the interviewees about local policies and their response to national guidelines. Interviewees were also asked about their views of how decision-making had changed over the last five years, especially in the light of the establishment of NICE and Primary Care Trusts (PCTs). Finally, the interviews ended with asking them where they saw areas for improvements. Overall, the interview guides left scope for the interviewees to use examples of decisions beyond the three drug therapies to illustrate their points.

4.2.4 Data processing and organisation

All participants gave their permission to tape-record the interview, which was transcribed verbatim. In addition to the tape-recording field notes were made during the interviews and attached to the transcription document. They included the description of the interview location, the impression of the emotional state of the interviewee and the researcher, and particular circumstances of the interview such as time pressure for the interviewee or the frequency of interruption, which may have influenced the interview.

4.2.5 Data analysis

The analysis was started during the data collection. This was partly to explore aspects of interest and investigate them further in subsequent interviews and partly due to the time available for the study. The transcripts were coded line-by-line. Two research supervisors, one with a pharmaceutical and one with a philosophical background read the transcripts. Regular meetings with them allowed a comparison of the coding and a reflection on broader patterns identified during the analysis. Codes were labels, which were descriptive or interpretative and attached to a section of the text. A section could be either part of a sentence, a sentence or a complete paragraph. The codes allowed the data to be structured and categorised.

A variant of the grounded theory described by Glaser and Strauss (1968) was used to analyse the data. Grounded theory means the systematic discovery of a theory from the data using an inductive process (Glaser and Strauss, 1968). Researchers using the theory in its purest form derive all the explanation and theories from the data. However, in the present study a mixed and more pragmatic approach was used. Some of the codes, such as cost containment, were thought to be relevant from the outset of the research, since they had great relevance in the literature. However, many other categories were derived from the data, for example 'tactics to bypass guidelines'.

The sections of text labelled with one code were examined regarding similarities and differences between the issues expressed and for the particular language used by different responders. Glaser and Strauss (1968) described this as the "constant comparative method" where the views and experiences of the interviewees and, in a later stage of the analysis, the categories and concepts, are compared to explore similarities and potential differences. The transcripts were searched for an explanation of the views and the potential relationships between them and the position of the interviewees. Of particular interest were areas of conflicts and dilemmas evolving during the process of decision-making, as it was felt that the analysis of these cases could highlight some important aspects of decision-making, the limits of the decision rules and the design of the process.

The data analysis was supported by the software package "Qualitative solutions and research: Non-numerical unstructured data – Indexing, Searching and Theorising"

(QSR NUD*IST, version 4.0), which is a computer programme designed to aid handling of large quantities of unstructured data and support its categorisation, searching and comparison. The Index System of the software programme allowed division of the codes into categories and sub-categories allowed linking. For example, the cost impact of a treatment was a category and drug cost, service cost and other costs defined as the subcategories. If one category was found to include further aspects, more sub-categories were defined. They were implemented in the Index System as secondary and tertiary nodes. If it was found that two categories actually represented the same aspect they were merged into one. If a code was unrelated to others it was defined as free node. For example, the code 'common terminology of the participants' was defined as a free node. With more analysis of the data some free nodes were included in the Index System if they could subsequently be linked with previously defined codes.

Several text searches were carried out during the analysis. The software programme had some search functions, which enabled a text search by using single words or phrases. This function was used to test specific hypotheses about the data. For instance, a text search for the term "prevention" was carried out to investigate the relationship between the opinion held about funding of preventative treatment and the position of the interviewees.

4.2.6 Discussion of the quality of analysis

There are two opposing views on the assessment of the quality of qualitative research. Some argue that qualitative research should not be judged using assessment criteria used in quantitative research such as reliability, validity, generalisability (Mays and Pope, 2000). Other argued that certain criteria could be used although not all of them might be relevant (Miles and Huberman, 1994). In adapting the terminology used to assess quantitative research Miles and Huberman (1994) differentiate between internal and external reliability and between internal and external validity. Other researchers prefer to talk about credibility and transferability instead of internal and external validity, and dependability instead of reliability (Lincoln, 2002).

In the present study several methods were used to ensure the rigour of the qualitative research:

- The analysis was done in the same way for all the interviews to ensure thoroughness and consistency. If new codes were added all the previous interviews were re-read and coded accordingly.
- The results were presented to illustrate the complexity and richness of the data. Miles and Huberman (1994) use the expressions "context rich" and "thick" description.
- The researchers specifically searched for the diversity of views, especially negative cases, which were inconsistent with the emerging analysis. Seale (1999) highlighted the importance of "a commitment to examine negative cases". The different views were described in the results regardless of the frequency in which they were mentioned, to incorporate all findings and not only selected cases.
- In discussing coding and themes gained from the data with two other researchers experienced in qualitative research it was possible to reflect on the analysis taking and clarify ambiguity. Although the concept of inter-rater reliability is contested among qualitative researchers, some argue that it could increase confidence in the findings (Pope *et al*, 2000).
- The generation of themes, hypotheses and conclusions from the data has been described in detail to enable transparency for the reader and to show credibility and plausibility. Various authors have stressed this as an important part of qualitative research (Hammersley and Atkinson, 1995; Silverman, 1993).
- Some changes to the topic guide were made during the study. The changes was used to clarify responses of the interviewees and to explore themes emerging during data analysis in more detail (Smith, 2002) (see Appendices 4.2.2 to 4.2.5). For instance, during the first stage of analysis it was found that the interviewees mentioned that the interpretation of some outcome measures used in drug trials was difficult. Accordingly, the question about the potential difficulties in the interpretation of outcome results was added to the topic guide for subsequent interviews.
- The results were compared with previous studies in the field of health policy research to judge the relevance of the findings and set them into the context of current scientific knowledge.

4.3 Results

The results will be presented in four parts. The first part gives an overview of the participants' positions and their relationships to health policy decision-making. The three remaining parts describe the findings of the interview analysis.

4.3.1 Position of the participants and conduct of the interviews

Initially, thirty professionals were contacted. Ten of these did not take part. One pharmaceutical advisor did not want to take part because she had changed her job recently and was no longer involved in decision-making. Two consultants in urology did not participate due to their workload. Two other consultants in urology, four professionals from the industry and a chair of a drug and therapeutic committee (DTC) did not reply.

Twenty professionals were therefore interviewed; all were face to face. Table 4.3.1 gives an overview of the position of the participants and their relationship to authoritative decision-making about access to the three drug treatments studied. They were interviewed at their places of work in the absence of any third party. This was to ensure confidentiality and to minimise interruptions. All of the interviewees agreed to be tape-recorded. The interviews lasted between 50 and 90 minutes with the exception of one interview of 40 minutes. The average length was 60 minutes.

Table 4.3.1 Position of each of the participants

No	Position	Relation to health policy decision-making
1	Health policy analyst	Special knowledge and interest in health policy
2	Pharmaceutical advisor, PCT*	Member of the PCT prescribing committee
3	Medicine information provider for PCTs and regional new drugs group (NDG)	Regional involvement in providing reviews on newly market medicine
4	Regional pharmaceutical advisor	Regional involvement in prescribing committees advising about newly market medicines
5	Clinical governance pharmacist	Experience as a member of hospital formulary committees and as a member of a PCT prescribing committee
6	Pharmaceutical advisor, PCT	Member of a PCT prescribing committee
7	Clinician in medicine for the elderly	Chairman of a new drug panel in secondary care, individual clinical decisions about prescribing of statins
8	Chair of a regional specialist commissioning group	Decision-making facilitator for specialist services
9	Consultant in medicine for the elderly	Individual clinical decision about prescribing of statins in secondary care
10	Consultant in medicine for the elderly	Clinician in charge of a dementia clinic, prescribing decisions for rivastigmine
11	Chair of a PCT	Member of various committees of the PCT
12	Head of public affairs of a pharmaceutical company	Interest in the decisions made about access to erectile dysfunction treatment and statin treatment
13	Head of government relations of a pharmaceutical company	Interest in the decisions made about access to Alzheimer's disease treatment
14	Chief officer of a Community Health Council	Representation of the patient's perspective and public interests in the NHS
15	Chief executive of a charity	Charity activities in order to influence health policy and lay member of a national appraisal committee
16	Chief executive of a patient organisation	Patient organisation activities in order to influence health policy-making and representation of the lay perspective on a national appraisal committee
17	Lay representative	Member of a joint prescribing committee between primary and secondary care
18	Lay representative	Member of a prescribing committee
19	Head of public affairs of a national patient and carer organisation	Special knowledge of decision-making about sildenafil
20	Head of public affairs of a national patient and carer organisation	Special knowledge of decision-making about rivastigmine

Table legend: *PCT: Primary Care Trust

4.3.2 Overview of the findings of the interview analysis

From the data three main aspects of decision-making were identified: technical, political and ethical issues. The technical aspects included the scientific information about the treatment under consideration, the scientific analysis of the information retrieved and the use of outcome measures. The political aspects were defined as the interaction within and between different interest groups involved in the decision-making process, the perception of their role and the role of other interested parties. The ethical aspects concerned the values that the interviewees mentioned. Since reality cannot be classified in categories, these three aspects were interrelated and a clear separation was not possible. However, often one of the three aspects was in the foreground whereas the others stood more hidden. In the majority of cases the political and ethical aspects of decision-making were underlying the technical ones and were not overtly discussed. Since the technical aspects dominated in the discourse of the interviewees, this aspect will be addressed first, followed by the political and the ethical issues.

4.3.3 Technical aspects

4.3.3.1 Process of appraisal and presentation of evidence

All interviewees mentioned the appraisal of clinical and economic evidence as the core issue in deciding about funding. Expert members of the PCT prescribing committee or the Drug and Therapeutic Committee (DTC) provided the data review, which the policy-makers "trusted" in delivering an objective and independent evaluation of the evidence available. The account of the policy-makers suggests that due to this trust, the data review was scrutinised less than evidence from other sources.

> "Often there will be a summary produced by the prescribing adviser, who produces a summary of the view from the HA if you like. There have been ten trials showing this, ten trials show that, bloody, blah, so they would recommend or whatever. So we would get a recommendation from pharmacy. And that would be taken as what you are working form." (Lay member of a prescribing committee, interview 17, line 268-274)
> [For transcribing conventions see Appendix 4.3.1]

It seemed that the presentation of evidence was not necessarily "neutral", suggesting it favoured one course of action. Hence, conflicting views were less likely to be expressed.

> "I can't remember a case where there were very strong feelings between members of the committee. Perhaps it's the way it is written up. It's usually very clear what one should be thinking and deciding." (Lay member of an area prescribing committee, interview 18, line 373-376)

The extent to which the evidence was appraised within the committee varied between committees and depended on the expertise of the committee members, as one lay member of a regional prescribing committee described:

> "The real trawling through the evidence goes on at the Drug and Therapeutic Committee level for its approval on hospital formularies, so they are reviewing in detail the evidence. At our committee level, the joint prescribing committee level, I suppose as a subcommittee level, we don't really look at the evidence." (Lay member of a joint prescribing committee, interview 17, line 262-265)

The process of gathering the information used as a basis for the decision was in the hands of professionals who commonly had expert knowledge about the design of clinical trials.

A pharmaceutical advisor explained the difficulty of discontinuing funding for a treatment which had been traditionally used. The discontinuation of funding for an established treatment would potentially result in public resistance and the explicit statement of limited resources. One pharmacist summarised this in asking:

> "And something that we've been doing for years and years and years so how do you stop doing it? How do you pull the plug?" (Pharmaceutical advisor, interview 2, line 1580-1583)

Due to the difficulty of discontinuing the funding, any new treatment was carefully considered before a decision about reimbursement was made.

4.3.3.2 Outcome measures

Clinical efficacy and impact on existing cost expenditure were the two most important aspects about which evidence was gathered, illustrating the values placed on these measures. Clinical efficacy, in the sense of the type of benefit, the effect size, and the probability of its effect (expressed as NNT) which can be expected from using a specific dose of a certain drug under trial conditions, was the starting point for the discussion about reimbursement. Although ideally effectiveness, which is the type and quantity of benefit in clinical practice (rather than clinical trials), was the preferred

outcome measure, there was commonly an absence of effectiveness data. If a treatment was regarded as ineffective or that there was insufficient evidence presented regarding efficacy, it was not further considered for reimbursement.

Appraisal of the economic data was the second step after the appraisal of the evidence about clinical efficacy. Usually, a treatment was only funded if it was regarded as 'worth' the costs in financial terms in order to produce a certain benefit. The policy-makers acknowledged that this was the decisive factor in the decisions regarding sildenafil, statins and rivastigmine. One interviewee speculated about the reason why the cost implications of a drug treatment were so important in decision-making.

> "Sometimes we do get all focused in on cost because that's the one thing that everybody can... it's very visible. Very hard to get away from. And drug costs are always sort of there. You know, it's the one thing that is constantly pressurised". (Clinical governance pharmacist, interview 5, line 421-424).

Evidence about costs was considered in various ways, for example total cost expenditure, cost-effectiveness or cost-utility, depending on the available data and the expertise within the committees. There was no universal approach in using health economic data and what was defined as costs. Some interviewees thought that total cost expenditure in monetary terms was most commonly considered, as two of them explained:

> "It's an issue when it's double the price in actual hard cash terms. That's when it makes them sit up and take notice." (Lay member of a prescribing committee, interview 16, line 327-329)

> "Or the incidence of the disease is low, so the total cost of treatment is low, then there's less incentive I suppose to block it." (Representative of the pharmaceutical industry, interview 13, line 411-412)

Not only the direct costs of the drug were important in deciding about funding. Apart from cost savings in the future the interviewees explained that the costs of the service associated with the use of the drug had also to be taken into account. This was especially important in deciding about the funding of rivastigmine.

> "And the issue again is that it's not just the funding for the drug, because it actually meant a new service. Because these patients weren't being referred to the hospital before, because there was nothing the hospital could do for them." (Pharmaceutical advisor, interview 2, line 430-436)

Some interviewees thought that these indirect costs had often been neglected, for example in the case of statins the costs of monitoring liver function:

> "Nobody ever talks about it – when they talk about it being two pounds a month or whatever – never talk about how often the patient has to come up, or the monitoring, and things like that". (Clinical governance pharmacist, interview 5, line 819-822)

4.3.3.3 Safety of the drug treatment

Safety was one aspect of the assessment of the treatment benefit beside effect size and was discussed during the decision-making process about rivastigmine, sildenafil and statins. Particularly in the case of statins, safety played a role where there were no long-term data about the use of the drug in a large population. One interviewee explained that in the case of the absence of larger RCTs, safety concerns were raised by several professional opinion leaders, which had an important influence on the decision.

> "And there was all this talk about - will people commit suicide with statins and all the rest of it. And I always thought it was extremely poorly based. [...] My own feeling was it was a public relations war, but I minded that very ethical and very evidence based people like [name of a professional opinion leader] were initially very cautious. " (Health policy analyst, interview 1, line 104-107, 166-169)

This highlights that proof of safety was not possible and professional consensus played an important role in the absence of RCTs.

Another factor influencing the debate about safety was the effect size, the number of people exposed and the expected size of effect. For instance, the probability of achieving a significant improvement in erectile dysfunction (ED) with sildenafil treatment was much higher compared to a significant improvement in cognitive function in AD patients (as described in Chapter 3). That meant that regarding the safety of rivastigmine one argument against its funding was the relatively high number of patients who needed to be exposed to achieve an improvement in cognitive function in one patient compared to other treatments such as sildenafil. As the clinical significance of the improvement in cognitive function was doubted, there was debate over whether the exposure of patients to rivastigmine was worth the potential benefit.

> "If the number needed to treat is a lot, then basically you could be damaging a lot of people with this treatment before you actually get the one that's going to benefit." (Regional pharmaceutical advisor, interview 4, line 842-845)

Another reason was that rivastigmine had only been used in a smaller number of patients compared to sildenafil.

In considering the safety of a product the experience of the physicians prescribing and monitoring the drug was also taken into account. Less experience of the physicians was seen as a potential risk and a reason to aim for the use of alternative products, with which the physicians were more familiar. Alternatively, access was only provided through specialists who were experienced in the use of the new drug.

4.3.3.4 Quantitative approach of decision-making

There was a tendency within the committees to prefer measures to quantify various aspects of the evaluation of evidence. For instance, according to the level of certainty of the evidence, it was allocated points. Meta-analysis scored highest. An interviewee mentioned that there were several attempts to extend the purely quantitative approach towards to the whole decision process, which included giving different points to various characteristics of the treatment under considerations (e.g. life-saving treatment scored higher than preventative treatment). Some attempts were made to use a quantitative approach to assess ethical issues involved in the decision about funding of a drug treatment. The ethical framework used by one PCT included "some rough attempt to quantify" the ethical values involved using a point system allocated to certain characteristics of the treatment. It was noticed that this purely quantitative method could not capture all the necessary aspects in order to decide for or against funding.

> " You give different scores to different things. You add up with... you add other things as well, or if it scores eight out of ten we'll give it this, and we'll have it. It just doesn't work." (Regional pharmaceutical advisor, interview 4, lines 1241-1245).

A similar limitation was found by using quantitative measures such as QALYs where length of life and quality was combined in one quantitative measure. The debate about

the QALY measure is exceptional in that decision-makers were aware of the values built into it.

> "And a lot of the quality of life scales are disgraceful, and if you are in a wheelchair by definition your quality of life is already half of what it is if you can walk. That's rubbish!" (Chair of a patient organisation, interview 16, line 540-544)

4.3.3.5 Interpretation of evidence

When asking the policy-makers how the outcome measures were used to make the decisions they explained that the data about efficacy and costs required interpretation in order to be useful for decision-making. The variation in the interpretation was one of the main controversies in making decisions about access to treatment. On one side, these variations in the interpretation were related to the knowledge about the outcome measures used in the clinical and health economical studies. On the other side, it was due to the differences in the values given to these outcomes and their political impact as well as the conversion from trial data to real life outcomes. The difference in the interpretation of the data illustrated that the technical measures were closely related to political and ethical aspects, although the debate was not phrased in that way.

For example, although the effect size of statins (NNT 25/14 to prevent one death/ non-fatal myocardial infarction for secondary prevention, as mentioned in Table 3.3.1) was smaller compared to the effect size of rivastigmine (NNT 13 improved 4 or more on the ADAS Cog scale, see also Table 3.3.1), it was not debated as much. This was due to several reasons: first, mortality was a more familiar and easy to understand outcome compared to the assessment of cognitive function as an intermediate outcome. It was felt that clinical outcomes such as cognitive function needed to be translated into effects visible in the day-to-day life of the patient in order to support decision-making. The more visible or detectable the clinical effect, the more powerful as an argument for funding, as one pharmacist summarised:

> "Which to me is what we are looking at, you know, does this drug make a difference to patients in real life? So, it was until we got more of that data that it actually did change real people in real life." (Pharmaceutical advisor, interview 2, line 672-679).

Furthermore, a higher value was given to the prolongation of life rather than cognitive function, which is equivalent to the 'rule of rescue', which will be discussed in section 4.3.5. (The 'rule of rescue' means that a higher priority is given to the treatment of a patient in an acute life-threatening situation than to preventative treatment). The example of statins compared to rivastigmine illustrates that the types of treatment for which effect size or outcome was difficult to measure were questioned more than those assessed using purely quantitative methods.

Similar to the data about efficacy, health economic data needed to be translated into the particular health care setting. One pharmaceutical advisor and one lay member commented:

> "So there's a lot of stuff, quite detailed health economic modelling, and things went on in some health authorities to try and work out what it would mean." (Medicine information advisor, interview 3, line 522-525)

> "What portion of the budget it would take up rather than the cost/benefits arguments which are quite complex. Economic arguments are quite complex and *distrusted* by a lot of people because once you start taking time off work and a loss of revenue. You can make things look really good." (Lay member on a joint prescribing committee, interview 17, line 320-324)

The interviewee in the latter quote used the expression of "distrust" which was an important theme in the account of the interviews. The "trust" or "distrust" of the evidence and not only the evidence itself affected the size of influence on the decision. Familiarity with the concepts of the outcome measures was one factor influencing trust. Most decision-makers had a medical background and were more familiar with data about clinical effectiveness than about health economic considerations. On a regional level this seemed to be more an issue than on national level where institutes such as NICE had a body of health economics experts involved. One pharmaceutical advisor explained:

> "To be honest there aren't enough people around with that level of health economic expertise. We leave that sort of thing to NICE usually." (Pharmaceutical advisor, interview 6, line 513-515)

One variation in the interpretation of economic data was related to the different comparatives chosen, and depended on the health care setting in which treatment was provided. Whereas in primary care there was a fear of cost escalation in providing

treatment with sildenafil, the use in secondary care was thought to reduce the costs since it was cheaper than many other treatment alternatives for ED.

In the interpretation of cost-effective data, ethical considerations of fairness of resource allocation and need for treatment were taken into account as one lay member described:

> "If you have something like multiple sclerosis or something like that although there is a very convincing case for prescribing a certain drug if the cost of that drug is so much that there would be not much money left for something else it does make life difficult." (Lay member of a PCT prescribing committee, interview 18, line 143-147)

Some interviewees explained that the cost expenditure had to be reduced in other areas in order to fund the new treatment. This meant that another treatment which was previously funded would "lose out". However, the comparison of benefits between different drug treatments across different medical specialities or patient groups was perceived as difficult, time consuming and often neglected. It was more common that each drug was considered on its own and only compared with one other treatment option for the same condition.

> "So in each meeting we are looking at drugs individually and assigning them a priority. But we've never ranked them against each other, if you see what I mean". (Pharmaceutical advisor, interview 6, line 142-145)

This had significant implications on the priority setting and was seen as a disadvantage.

> "I think when you're making decisions on something you don't make decisions about what you are disinvest in. It doesn't seem to come very much into the equation. It is sort of saying yes or no. But not if we say, yes, what does that mean." (Lay member of a joint prescribing committee, interview 17, line 673-677)

The differences in the data interpretation reflected the controversy regarding the question of how generalisable the results of the clinical trials were. A pharmacist described the decisions made about drugs which were available in the hospital:

> "You don't want to be having six or eight ACE-inhibitors because each have got a little tiny license of variation. But at the same time if it's a class effect or whatever... [...] And I know that on the whole the formularies just make up a general decision, they either decide it's a class effect and only have a few [drugs], and everybody has to use that, or they don't and they have a lot." (Clinical governance pharmacist, interview 5, line 254-256, 260-263)

Many interviewees mentioned the difficulties of using the data from clinical trials to make decisions about therapy of patients in non-trial conditions, which limited the certainty with which a specific amount of benefit from therapy could be expected. For example, one of these uncertainties was due to the unknown extent of non-adherence among patients. As some of the interviewees pointed out, the impact of non-adherence was often neglected in making decisions resulting in the waste of resources and a decrease of the effectiveness of the intervention.

> "But the real issue is after about six months my rule of thumb is that about fifty percent ain't using them [statins] even if they are cashing the script." (Health policy analyst, interview 1, line 449-452)

One important finding from the study was that despite the emphasis of the need for an "objective" appraisal of the evidence the interviewees acknowledged that subjective factors influenced the interpretation of evidence. The "feeling from the evidence" came into play.

> "But at the end of the day you've got that bit of subjective sort of bit at the end that weighs – oh well, let's have it anyway." (Pharmaceutical advisor, interview 4, line 287-1290).

> " Some questions in ordinary people's minds about the evidence base – whoever do we believe?" (Health policy analyst, interview 1, line 1425-1427).

One pharmaceutical advisor mentioned the emotions and the personal involvement in decisions about life-saving treatment, which influencing the appraisal of evidence, which will be further discussed in Section 4.3.5.

The term "exiting" was frequently used by various decision-makers to describe some novel therapies such as sildenafil or statins. The interpretation of the evidence became more subjective if the evaluation was driven by the excitement of a new treatment.

> "And the other thing about statins is that they are really quite exciting drugs and there's accumulating evidence that they probably work and have their benefits in ways which were at first unexpected." (Consultant for care of the elderly, interview 10, line 671- 674)

Another example was a novel cancer therapy as one clinician told:

> "The data is not very good and the enthusiasm for giving them, by oncologists is enormous. [...] And the oncologist is going and telling you about a wonderful new drug and a big argument and discussion and you say- yes, yes,

> yes, right, put it on the formulary". (Chair of the new drug panel, interview 7, line 152-153, 156-158)

The complaint of a pharmaceutical company representative about an absence of a certain extent of excitement, which he regarded as necessary to make decisions about access to treatment, shows that the industry recognise its influence on the decision:

> "I see very little excitement within the review that says – this offers a great deal of potential. And I don't think we are given the benefit of the doubt very much. Which I think you need an element of when you are introducing new products." (Representative of a pharmaceutical company, interview 13, line 345-349)

The representatives of the industry wished that the policy-makers would give the medicines the "benefit of the doubt". Stakeholders such as the pharmaceutical industry used the knowledge about the influence of excitement on the decision and its outcome to create or reinforce the excitement by decision-makers.

4.3.3.6 Definition of access criteria

The general approach to decision-making in many cases was to use evidence available to limit or rule out the use of the drug. That means, if there were any doubts about the clinical effectiveness of the drug, policy-makers decide to go for the minimum proven by evidence. One of the lay members used a case example to illustrate that approach:

> "One of the respiratory physicians said when we were discussing these drugs, he said, well, I haven't use them much, but I have tried them in a couple of patients, he said, unfortunately they were working really well. He said, unfortunately, they were working really well, as if to say, 'Oh God, these are going to be really good, they are going to cost us a fortune'. And it was an extraordinary thing to say." (Lay member on a joint prescribing committee, interview 17, line 225-231)

The lay member continued:

> "Their view is, this is some expensive drug, how do we limit it... And that takes a huge... and then they look for reasons. They do it the wrong way around. [...] They think we don't want this, now let's look at the evidence and let's see if we can justify not making it widely available or limiting it." (Lay member of one of the prescribing committees, interview 17, line 189-192)

In the cases of sildenafil and statins the government set up guidelines to regulate prescribing in the same manner. Overall, most of the interviewees agreed with the choice of the criteria, which were defined to control costs rather than according to their

clinical efficacy. Asked for their opinion about the criteria defining access to sildenafil two interviewees commented:

> "Researcher: What do you think now about the criteria?
> Interviewee: The criteria? I think they are broadly sensible. [...] I think they [the government] originally said they didn't want to spend more than.... I think it was .... you can check this, but I think they originally said it was something like nineteen million, they didn't want to spend more than nineteen million pounds a year on this drug." (Medical information provider, interview 3, line 301-304, 323-330)

> "I wouldn't say that the categories are too wide because they are fairly narrow, so I'm sure that's right. It's also useful that they put a restriction on it as to four tablets a month." (Pharmaceutical advisor, interview 2, line 912-917)

The criteria of the use of statins were defined in the way that the budget was not overspent. Achieving effective cost control was seen as one part of a good decision.

> "Statin prescribing in primary prevention, there's no doubt about it that the limits that have been set were set bearing in mind cost considerations." (Consultant in medicine for the elderly, interview 10, line 677-679)

Clinical criteria were used to define the groups of patients which were eligible for funding, arguing that this ensured that the patients "most in need" would have access to a treatment, which was reimbursed by the NHS. The argument that the "worst off" were advantaged against other patient groups seemed to legitimise that some patients were excluded who could benefit. One interviewee explained the way in which the criteria for treatment of rheumatoid arthritis were defined:

> "What would be, if you wanted to treat it, what would be the strictest criteria? [...] Well, it was really, a cost perspective, at the end of the day, because, you know if you are told that the pot of money that you've got is so minuscule, you've got to find some criteria that will.... They agreed some criteria that would mean that those people that were the worst would get it". (Clinical governance pharmacist, interview 5, line 787-793, 797-801)

In this case the committee decided that at least three other treatment options needed to be tried without success in order to be eligible for the drug treatment under consideration, although patients would benefit from the treatment at an earlier stage in their disease. The case illustrates how much the technical measures used to define access to treatment were linked with ethical considerations. The underlying issue of which group was regarded as "most in need" was defined by using clinical measures

such as diagnostic markers in the case of statins or in the case of rheumatoid arthritis the treatment outcomes of previous therapy received.

One of the strategies used to achieve cost-control was the introduction of increasingly rigid definitions. This resulted in some cases into the definition of criteria which were not useful in practise. One interviewee used the access criteria to treatment with sildenafil as an example:

> "There's a few of them, which if you actually look at them very closely, you can't define. Like single gene hereditary disorders, there's a full database of those. There are thousands of disorders, and you know, nobody, you speak to even genetic experts, they can't define what that is." (Medicine information provider, interview 3, line 261-263)

Other strategies apart from the definition of clinical access criteria were used to delay access to the drug in the cases of sildenafil and rivastigmine. Patients who suffered from severe psychological distress caused by ED or from AD needed to be referred to a specialist where waiting times of months to obtain an appointment were common. One interviewee referred to the case of sildenafil:

> "Then the only real stumbling block is this sort of low-defined psychological aspect which requires a hospital referral." (Medicine information provider, interview 3, line 261-263)

Avoiding uncontrolled cost expenditure as well as preventing bypassing of rationing was an important goal for policy-making. Uncontrolled expenditure was described as "disaster", "terrible" or "going mad". Hence, the decision made and the process in place needed to guarantee cost control. Since cost control had such importance, the threat of budget overspending was used as an instrument to influence decisions. It was found that the costs estimated for the funding of the drug therapy were often unrealistically high and purely based on epidemiological data without reference to the likely uptake of the innovation. In addition, some responders pointed out that British physicians were found to be more conservative than their colleagues in other countries, which would slow the uptake of new therapy. A clinician called for caution when using epidemiological data to estimate the cost expenditure by using statins as an example:

> "Some of the great anxiety, of course, was what it would cost the area health authority if everybody who needed secondary prevention, or everybody who had a cholesterol over seven or eight or some magical figure... it would cost millions, you see. So there was all this anxiety. But we used, in the areas prescribing committee, to say - oh calm down. GPs are just not that efficient at

finding these people, they are all out there, I agree, but the GPs won't find them so why bother?" (Clinician in medicine for the elderly, interview 7, line 346-354)

4.3.3.7 Decisions in the 'absence' of evidence

The interviewees admitted that the absence of good quality studies made it difficult to decide about the introduction of a novel treatment. An absence of evidence – especially of long-term studies on efficacy and cost-effectiveness - was common in cases where a drug had been licensed recently. For instance, one advisor expressed his concern:

> "It just...decisions are made, largely within a very worrying degree of absence of evidence." (Medicine information provider, interview 3, line 1258-1260)

The interviewees seemed to be worried that the absence of evidence would result in the introduction of less or ineffective treatment and money would be wasted. In contrast, the presence of evidence meant that the decisions made would result in a higher degree of certainty about the delivery of effective health care and controlled expenditure.

In a situation where there were no controlled trials or where the effect size of the drug was doubted to be meaningful, anecdotal evidence from health care professionals (HCPs) in direct patient contact or patients themselves became increasingly important. One clinician used a case example to emphasise the importance of anecdotal evidence, which resulted in bringing an effect 'to life' in showing the human implication of an effect.

> "And he [the patient] had a number of slightly difficult social consequences of his illness. And after he'd taken donepezil some of these abated. And he went to this graduation ceremony, and it meant a lot to him. It meant a lot to the family and to his grandson. And it's that sort of anecdotal experience, which illustrates the sort of benefits. [...] And on human level people like stories, you know. Don't they? Do you present all the figures, but you tell them a few stories, you know. And people are interested in that." (Clinician in the care of the elderly, interview 10, line 165-170)

The decision about funding of a drug treatment was especially difficult if they were no other alternative treatment options. A smaller amount of evidence was required in order to fund the drug, although it was not clear what the minimum required level was. One pharmacist explained:

> "So sometimes, for some of the drugs, if there is absolutely nothing else to treat it, or it's a major new advance that will treat a new group of patients that previously weren't covered by treatment, you probably would accept lesser volume of published evidence, but you would still need something". (Clinical governance pharmacist, interview 5, line 534-539)

A greater risk of harm was accepted in cases where the harm without therapies is also substantial. This might explain why especially the decisions about donepezil for the treatment of AD or some types of cancer treatment were regarded as difficult. There was an absence of evidence from long-term studies with a large number of participants at the time of their market approval, and at the same time no other treatment alternative was available.

However, some committee members emphasised that their committees would not accept anecdotal evidence and were strict in their criteria regarding what type of evidence was accepted:

> "We won't accept anything that's not published in a peer review journal. Fully published. And we won't accept anything that's not had a randomised trial really". (Chair of a new drug panel, interview 7, line 176-178)

In a situation of an absence of RCTs or meta-analyses, the committees tended to agree the use of the particular drug under an agreed protocol which defined clinical criteria under which the clinicians were able to use the drug in a certain group of patients. At the same time the clinicians had the obligation to monitor the effects and adverse events of the therapy very closely. The continuation of treatment funding was only provided if the programme showed benefit. This meant that the use of the drug under protocol would create a form of evidence from which continuation of funding in the future would be judged. The use of a preliminary stage of treatment funding was a convenient way for the committees to avoid conflict. The committees did not need to deny access to the treatment and could argue that a certain number of patients were treated. Additionally, the obligation of the clinicians to evaluate benefit of the programme gave the committees tighter control over costs instead of "opening the floodgates" for universal funding of the drug and stop funding if the treatment was not cost-efficient.

> "But I think there is more and more the view that where there is limited evidence, but some evidence, then it's about controlling the introduction, so that it's not just – yes, OK, we'll fund it for everybody. But allowing a further

> limited trial to take place, so that we learn from that before it's kind of funded all round". (Chair of the specialist commissioning group, interview 8, line 320-324)

4.3.3.8 Timing and dynamics of the process

The technical aspects of a drug and their evaluation needed continuous review since new research findings were quickly generated. This dynamic process consumed a considerable amount of resources, which was difficult to provide. It was pointed out that, once a decision regarding funding of a treatment had been made, it was necessary to revise that decision as soon as new data arrived.

> "So it's an ongoing process, it's not that we'll say no to something and that's it." (Pharmaceutical advisor, interview 2, line 412-413)

Some interviewees criticised the absence of a regular review process. Additionally, since the use of resources following the introduction of the drug was not evaluated or the evaluation was of low quality, improving the use of resources was seen as difficult. The disinvestment in many areas was a frequently raised concern.

There was a discrepancy between decision-makers in what they regarded as the appropriate point in time to decide about the access to treatment. Some argued that the decision should be made at the same time the drug was licensed, others such as one representative from the industry thought that the product needed to be evaluated in practice to decide about access to treatment.

> "Well, maybe after five years. I think that would be reasonable. We are constantly learning and re-evaluating with our own medicines and monitoring." (Representative of a pharmaceutical company, interview 12, line 286-289)

Decisions generally had to be made within a time limit. This meant that the organisation of the process had to be compromised in order to deliver decisions on time, potentially influencing the quality of the decisions. It was necessary to give the members of the committee sufficient information on which they were able to base their decision. At the same time the amount of information needed balancing against the time required to read it. A pharmaceutical advisor explained:

> "But not too much information so that people won't read it. Because if people won't read it they are not thinking about it". (Pharmaceutical advisor, interview 6, line 680-683)

4.3.3.9 Summary of the technical aspects

In summary, the technical aspects were very much in the foreground of the discourse of the interviewees. Clinical efficacy and costs of the treatment, which were measured and expressed in a variety of ways, were regarded as the most important criteria to decide about allocation of funding. Evidence about efficacy and cost-effectiveness were used to define the patient group eligible to receive the drug. Definition of these criteria depended on the funding available rather than on the size of the clinical benefit. Cost considerations were very often reduced to concern over the total expenditure, possibly due to lack of knowledge about health economics, the large mistrust in the quality of economic studies and the absence of data about the empirical impact of the therapy. Comparative studies with existing therapy to establish effect size were often absent and made prioritisation between treatment options for the same conditions difficult. On one hand the emphasis on technical aspects seemed to be used as a protection against the accusation of randomness and arbitrariness in the decision-making process. On the other hand the account of the interviewees made clear how little is often known at the time of a funding decision and that the uncertainty of effect size and total expenditure worried the policy-makers. The technical aspects of decision-making were likely to be seen as "neutral" and mostly value-free whereas in fact they were closely linked to the political and ethical aspects of decision-making. For instance, the choice of outcome measures were closely linked to what was valued most as a health benefit and what was seen as the target of treatment. Despite the emphasis on a "rational" and scientific evaluation of evidence, the interpretation of it was influenced by the excitement about the novelty and potential benefit of the treatment as well as the personal experience in prescribing or using the therapy.

4.3.4 Political aspects

Apart from the technical aspects, decision-making about access was characterised by the interaction of various interest groups and their agendas in gaining influence and power in the decision-making process. Throughout the interviews it became clear that each of the interviewees felt part of a group with a common agenda and a specific accountability rather than acting as an individual. Some of the interviewees belonged to interest groups who were active decision-makers and committee members such as medical professionals, pharmaceutical advisors and lay members. The other groups were stakeholders, who were not decision-makers themselves, for instance the pharmaceutical industry, the media, patient organisations and the courts. The government was both policy-maker and also stakeholder. The term 'political' was chosen to describe the interplay of the decision-makers and stakeholders. The account of each of the groups, policy-makers as well as stakeholders, can be divided into three main themes: the policy-makers' and stakeholders' views about their role, their key arguments in the debate and the views by others. Although the results will not be grouped under these subheadings, the themes can be found in the account of nearly all policy-makers and stakeholders.

4.3.4.1 Policy-makers

Physicians in secondary care

All participants mentioned physicians as either the most powerful or one of the most powerful interest groups, which influenced the decisions about funding of drug treatments in various ways. The physicians – mainly consultants in a specialist area – were acknowledged as experts, who had the clinical knowledge about the drugs and their use. Other decision-makers felt dependent on them in providing their expert opinion about the drugs under consideration. The fact that expert knowledge was most important confirms that clinical effectiveness and safety of the drug were key arguments in the debate.

However, the interests of the physicians in secondary care were often seen as being in conflict with the views of the commissioners. The clinicians regarded financial implications as less important compared to the potential benefit of the drugs. Some decision-makers accused them of totally ignoring the cost-implications. The conflict

between the clinicians' responsibility for treating the individual patient in his or her best interest and the need of the commissioners to balance a range of priorities created a continuous tension between these parties. One pharmaceutical advisor explained:

> "I think one of the problems, particularly with consultants, less so with GPs because GPs are quite good at taking a broad population view, because they have to look after a population of patients. Consultants have vested interests, which is their speciality." (Pharmaceutical advisor, interview 6, line 631-635)

The clinicians saw their role in acting in the interest of the patients. One clinician explained:

> "I see my role as using the drug when it is available and actually rooting for an adequate supply for my patients. I don't see my role as having to arbitrate between the different groups of patients. I'm not in that place. I mean, I realise that some people have to do that. But I think it's my job to, as a geriatrician, to actually act as an advocate for my patients." (Clinician in the care of the elderly, interview 10, line 487-492)

Acting in the interests of the patient was one of the strongest arguments of the clinicians but could also be misused to mask self-interest. Some respondents believed that there was a tendency among some physicians to mask their personal benefit from research and financial gains by pretending to act in the interest of the patient. The physicians used other groups to act in their interests, such as patients, by increasing their expectations and writing to MPs or the media. Two pharmaceutical advisers explained:

> "Um, consultants, the lead consultant involved was very clever in terms of trying to manipulate various people here to get funding for it." (Pharmaceutical advisor, interview 6, line 815-817).

> "And I mean, obviously, some of the other higher profile consultants get onto the BBC and local radio and local press, etc. Which they do. If they are really, really angry. And the other thing is writing to MPs." (Medicine information provider, interview 3, line 591-595).

In some cases the power of the clinicians (e.g. the cancer tsar) was seen as comparable with the legislative power of the government and described as a "tsar must-do" compared to the "government must-do".

Since the clinicians as policy-makers were so powerful, keeping a positive relationship with them had occasionally a higher priority than cost containment, which was normally the most powerful constrain.

> "And trying to, you know, accept that clinicians are, you know, prescribing certain drugs, even if we are not convinced of the efficacy. Is it something that we should really go back and say - you may not prescribe that? You know, how strongly do I feel about this? And how much is that going to damage the relationship with the clinicians? You know, how big of a cost is it? And, you know you want generally to be working with clinicians. And supporting them." (Chair of a specialised commissioning group, interview 8, line 391-400)

Mutual trust was seen as another important factor to achieve collaboration between the committees and the clinicians. However, in order to achieve more transparency, establish trust within the committee and successfully negotiate between the physicians and other committee members, some of the committees asked the applicants to state any relationship between themselves and the pharmaceutical company concerned.

The clinicians' experience of the successful use of a drug in some patients was put forward as an argument for the reimbursement of the drug therapy even if the evidence of the RCTs did not reflect the consultants' experience. One clinician explained that his personal experience in using one of the acetylcholinesterase inhibitors (AChEIs) had strong impact on him to promote the wider use of them.

> "And also I did prescribe to a few patients, who were able to afford to pay for the drug, donepezil. And I was actually quite impressed by the effect that it had on those people. You know? It was individual clinical experience. They did appear to be benefited. Their families were please with what had happened and that helped, actually helped to convince me that these drugs were of value." (Consultant in medicine for the elderly, interview 10, line 543-549)

This indicates first, that through having patients' contact, the clinicians were able to understand the meaning of the improvement for the patients and their lives and second, that the evidence of RCTs was supplemented by personal experience of the physician in his daily practice as another form of evidence.

In contrast to other committee members, this direct patient contact had a large impact on the consultants' perspective as one lay member summarised:

> "Some of the consultants feel very strongly about certain treatments and they are *actually treating the patient, which is the main thing really.*" (Lay member of a prescribing committee, interview 18, line 340-342) [my emphasis]

Direct patient contact was an important difference between decision-makers and divided the group of committee members into those working with patients and those

having no patient contact. The interviewees differed in their views concerning the influence of working closely with patients. Some thought that only patient contact would enable policy-makers to have a 'realistic view', which was welcomed by patient organisations. Others, mainly commissioners, pointed out that the decision-making would be 'emotionally' influenced and lack the sense for 'reasonable' decisions.

> "No, they [the clinicians] get very emotional, they get confronted by the patient." (Consultant in the care of the elderly, interview 7, line 722-724)

> "I am accepting them because I think they're coming from somebody *who has got practical experience of their patients and I listen to that.* I would always listen to a specialist nurse, always, because they are close to their patients. The professor sitting in the University of something I pay less attention to." [my emphasis] (Chief officer of a community Health Council, interview 14, line 969-974)

Pharmaceutical advisors

Commonly, the pharmaceutical advisors were pharmacists who, as one of their duties, provided the prescribing committees with a review about clinical efficacy and cost effectiveness of the drug therapy in question. Since many pharmaceutical advisors had no direct patient contact, lay members in particular viewed the advisors as focused on cost expenditure. In addition, one lay member pointed out the different values placed on the outcomes of treatment by various decision-makers. Pharmaceutical advisors were in his opinion more interested in clinical outcome measures such as morbidity, mortality or adherence, rather than quality of life.

> "And I think the decision-makers, pharmacy, prescribing advisers aren't very interested in 'Oh, they do better on the SSRIs*, that sort of folk type, yes they're better, they're much happier on them'. They don't want that. They want studies that show that the quality of life is improved. And may be more than quality of life being improved, maybe they want better outcomes or better compliance, so you get better quickly because you take the drugs, where there isn't just quality of life good enough." (Lay member of a joint prescribing committee, interview 17, line 447-456) *SSRIs: Selective seretonin re-uptake inhibitors

Pharmacists were mainly in charge of monitoring the adherence to the guidelines and of 'policing' drug use in primary or secondary care. This influenced their view on decision- making in terms of focusing on preventing bypassing of guidelines. For instance, they saw it as part of their role to present clinical evidence for or against the

use of a certain drug to the GPs to "protect" them against what was seen as the negative influence from the industry.

> "So, again, it's our job, as we see it, to try and give the GPs the information they need in order to say to the patient – well, actually you've only got one side of the story here. And to actually give a balanced view." (Pharmaceutical advisor, interview 2, line 303-308)

However, the pharmaceutical advisors pointed out that they had very limited influence to police the guidelines in the community.

> "GPs effectively are not really that cash limited in that they can write a prescription and there's not somebody saying - no, you can't write that prescription because there's no money left." (Pharmaceutical advisor, interview 6, line 61-64)

Lay members

In this sample, lay members were defined as individuals appointed or elected to represent the lay view, which means the view of the public or the health care user. They were neither HCPs nor commissioners but some could have specialist knowledge related to health and health care. Lay members were part of the PCT prescribing committees or national committees such as the NICE appraisal committees.

Generally, most policy-makers interviewed saw it as necessary to involve lay members since it was government policy. Whereas PCT prescribing committee members found the contribution of lay members useful, the lay members themselves often felt as an "extra token" instead of being full members of the committee. One lay member said:

> "At first I was introduced as [name] from the CHC*. Yes, that's great, we have got a lay person on the committee and he will just sit and listen, but we don't want to him to say anything or yes, that's all very well but he will just not understand what's going on." (Lay member on a joint prescribing committee, interview 17, line 82-86) *CHC: Community Health Council

This was due to the fact that lay members experienced difficulties to make their voice heard in the decision-making committees in which medical professionals were in the majority and their views dominated. One lay member of a national committee explained:

> "So that the debates takes place on territory that was specified by the professionals decades ago which means then that's the lay members of such

> committees are fighting, there are working uphill." (Chief officer of a patient organisation, interview 16, line 273-276)

Another lay member judged his influence as very limited:

> "I mean as a lay person I couldn't sway that opinion in terms of making them change their minds over its availability. I think I don't have that influence in the committee." (Lay member of one of the prescribing committees, interview 17, line 249-252)

One of the main difficulties of the lay members was their limited clinical knowledge. Hence, in the beginning the lay members of the PCT prescribing committees were provided with some educational material mainly about the terminology used in order to understand the issues discussed and to take part in the decision.

All the lay members interviewed mentioned that it was important to gain credibility within the committee. Whereas the professionals had credibility through their degree and professional experience, lay members felt they needed to prove their credibility and respect among the committee members. This was achieved by showing understanding of the subject discussed, asking meaningful questions and finding a balance between their interests and the agenda of other interest groups. One interviewee reported:

> "I think a lot is about personal relationships and whether you are respected and you are seen as somebody who knows what they are talking about. [...] I think if you are respected as knowledgeable and particularly, for example, if you are from a group or organisation that is authentic to them [other members of the committee]. (Chief officer of a community health council, interview 14, line 470-472, 479-481)

This shows that the lay members had to establish themselves as a credible source of evidence. The lay members reported that their contributions and arguments were not regarded in the same way as those of the professionals and commissioners. One interviewee explained:

> "It's difficult for me to present an argument in a medical setting when I haven't got the evidence that they see as – what is the word – robust and genuine." (Chief officer of a community Health Council, interview 14, line 630-632)

One of the reasons was that the lay members were unsure what type of evidence was expected from them and how it was judged in comparison with the clinical evidence contributed by the professionals.

There were conflicting views among the lay members in the way they represented their views. Some thought they could only represent their own point of view as a potential user of the NHS.

> "I am not there to represent anybody. I am not there to represent a particular patient group. I am there because I have got experience and know broadly about some of these subjects. I don't have any worries whether I am representing anybody." (Lay member of a national prescribing committee, interview 15, line 679-682)

Others thought they should act on behalf of others and present the view of patients, as another lay member explained:

> "And often when we make decisions or you're thinking what patients think I'm thinking of the old lady who lives over the road or the next-door neighbours. People you might meet on the streets." (Lay member on a joint prescribing committee, interview 17, line 658-661)

All lay members in the sample were appointed rather than elected. As they were not democratic representatives, one of the dilemmas of the lay members mentioned was to what extent their views represented the one of the majority of the public or the patients. Therefore, the lay members thought that using survey data to underpin their view would be a powerful argument in the debate. However, usually the lay members had no means to quote evidence which weighed equally to that presented by the professionals.

The lay members felt accountable to make the voice of the "unidentified person" who is suffering form a certain disease heard and to present the individual rather than the population. One lay member explained:

> "They [the professionals] are talking about patients in general and if they have to put up with side-effects because it's saving our budget a lot of pounds a lot of money, I think the decision goes with the money rather than with Mrs Smith because it's not an identified person. But when it comes down to a decision what they take as professionals, as a choice, they would go for the safer and cleaner and better tolerated drug, that's what we would do all. I think that sort of thing doesn't come into the decision-making process enough." (Lay member on a joint prescribing committee, interview 17, line 414-421)

4.3.4.2 Stakeholders

Pharmaceutical industry

The pharmaceutical industry had no representatives in the decision-making committees. The influence was indirect, through provision of information about drugs to the committees. Many trials, especially before licensing of the product, were sponsored by the pharmaceutical industry. In the views of most interviewees the pharmaceutical industry mainly influenced decision-making through their impact on the design of drug trials, which were sometimes seen as flawed by methodological limitations.

> "Some of the trials are clearly, you know the drug companies trying to get their product on the market, without necessarily producing any useful data." (Pharmaceutical advisor, interview 4, line 1053-1057)

In addition, interviewees believed that the companies suppressed negative evidence.

> " So it's in the drug company's interests, and it's only when that data is probably less than convincing that they don't make it available" (Medicine information provider, interview 3, line 1573-1576).

Although as global operating companies the industry representatives denied that studies were specifically designed to suit the requirements of a national or regional committee deciding about resource allocation, one representative admitted:

> "I mean, they might do some small tactical studies in the local market that met the need." (Representative of a pharmaceutical company, interview 12, line 381-382)

In addition, a common complaint of policy-makers was the selection of outcome measures and the short duration of the trials, usually under six months.

As the pharmaceutical company representatives were not direct decision-makers, they used various ways to influence other interested parties, who were either policy-makers or stakeholders. Policy-makers such as physicians in secondary and primary care were important targets to lobby for specific products. The companies promised clinicians financing for conference fees or education material:

> "And they provided me with a set of lovely slides, fabulous slides, which were mostly about stroke, you know, but a few things thrown in about their product. Now, this is all dressed up as education". (Consultant in medicine for the elderly, interview 9, line 223 – 225)

Therefore, most interviewees, especially medical professionals, highly mistrusted the information presented by the drug companies and stressed that they were avoiding contact or called for caution when dealing with the representatives, since they feared the loss of objectivity in making a judgement about the product. This high level of fear shows powerful influences of companies and perhaps a lack of clear ground roles for the committee members.

> "So there were concerns on the industry side to obviously earn as much as possible out of these molecules. [...] And remember, there's a corrupting interest from the industry which will try and market things even when they don't work. So, you have to stand back, be confident of your judgement." (Health policy analyst, interview 1, line 741-45)

> "I do think there are sometimes some difficulties in terms of working with the pharmaceutical industry because whether you like it or not, bottom-line, pharmaceutical industry is to make money. Well, that's everybody's perception of it. They obviously say that's not. But this is bottom-line." (Head of public affair of a patient organisation, interview 19, line 730-734)

The industry tried to bypass the decision-making by sponsoring drug trials and delivering treatment for patients free of charge as long as the drug was not licensed. At the time the product got its market approval the health care institutions become aware of the need to continue treatment to patients already receiving the drug and to pay for the drug. One participant described bypassing of the usual decision-making process with "the drug slips through the net" and the sudden financial pressure as "a gun to the head". The interviewee reported:

> "But we'll probably end up having to pick up that bill, even though it's been introduced through drug companies giving the drug free of charge on a sort of clinical trial basis. So there are some, there are quite a lot of routes in that drug companies use to get a drug in the system, which then leaves us in a position where we really don't have much choice. It's not the way we would like to do it. [...] And suddenly something is, you know, a gun to the head, you've got these patients on this drug, you now need to continue funding." (Chair of the specialist commissioning group, interview 8, line 270-276, 282-284)

This was in contrast to the view of the representatives from the pharmaceutical industry about these trials. It is interesting to compare the differences in the terminology used.

> "It can be seen as an access route. I think the kind of terminology is a *seeding* trial where you get lots of these, but I think it depends on how you view that type of trial. I mean, most of the trials that we run, I would classify more as *compassionate* trials, particularly in areas like oncology, where we, you know,

> the period between submitting your dossier and getting your license is going to be at least a year. What do you do in the meantime?" (Representative of a pharmaceutical company, interview 13, line 1023-1029)
> [my emphasis]

This representative refers to the time between the submission of the product application for market approval and the decision made by the authorities to give or deny the approval. Especially in case of oncology therapy where there is no treatment alternative available, it is in the interest of the patients, clinicians and the industry to make the product available as soon as possible. Later on during the interview the representative from the industry admitted that a "nasty surprise" could wait for the commissioners when they realised that they needed to pay for these drugs which were provided freely in the past.

One of the key arguments by the industrial representatives for including them as decision-makers was their unique knowledge about the drugs, which the decision-makers should take advantage of. A representative talked about the disappointment of being excluded from health policy decision-making.

> "And that's frustrating for, obviously, because we develop these drugs from the start, and because we've had huge global teams working on them, there really is no-one out there who knows more about our drugs than we do. So it's extremely frustrating for people to see." (Representative of a pharmaceutical company, interview 13, line 194-198)

Hence, there should be an active collaboration between the pharmaceutical industry and the policy-makers.

It is interesting to recognise that for the reimbursement of certain drugs the representatives from the industry used similar arguments to the patient organisations. For example, in the case of sildenafil one of the representatives stressed the individual impact and experience of ED and describing the life of patients suffering from it as "ruined":

> "It's alleged to... you know, provide a quick fix for young men who want a thrill, and it's not what it's about. It's about people who are unable to achieve an erection, for whatever reason, and *whose lives are being ruined by that.*" (Representative of a pharmaceutical company, interview 12, line 520-523)

Another representative used the same terminology in describing that, in not treating behaviour disorders in children with methylphenidate (Ritalin ®), the life to the family could be "ruined".

Patient organisation representatives

Representatives of patient organisations or charities promoting health and social care for certain conditions were not permanent members of decision-making committees. However, they were asked by regional and national committees such as the PCT prescribing committees and the NICE's appraisal committee to submit documents and statements of their opinion about access to certain drugs. In addition, it was possible for patients to give evidence "in person" about a specific condition. The link between the committees and the patients was often via a patient organisation.

There was a discrepancy in the views of the interviewees on including patient organisation representatives in decision-making. Some highlighted the difficulty in implementing the views of the patients due to their subjectiveness and inter-individual variation. This was seen as a problem rather than a benefit or something good to be expressed. Additionally, the patients' view needed to be weighed against the need of the local population. Others emphasised the importance of hearing their views, which could be very different compared to the one of HCPs and other committee members. One pharmacist involved in regional decision-making described the different views:

> "Because it's quite interesting, I went to a ward round for the Infliximab ® and the person came along to talk about what the injections had done to her, for teaching. And the biggest thing for that person, they made something like a six hour round trip to get their injections and it was, what they really liked about having this was that they could get out of bed and make a cup of tea in the morning". (Clinical governance pharmacist, interview 5, line 722-727)

Other decision-makers especially the medical professionals perceived the influence of the patient interest groups as controversial. The action of some patient organisations was criticised as opportunistic and strongly influenced by the industry. The term "lobbies" was used to describe the link between patient interest groups and industry or political parties trying to promote a specific treatment. These lobbies were recognised as very influential on committees as one clinician highlighted.

> "I think in areas where they've already got quite active lobbying groups, for example with the new guidance on schizophrenia drugs. [...] And I think, because there are already quite powerful lobbies within mental health, I think they will probably effect that change quite quickly". (Clinician in care of the elderly, interview 9, line 271-272, 274-276)

Similar to the lay members mentioned earlier, the representatives of patient organisations expressed their concern that other decision-makers called for patient involvement not without self-interest, as it was politically "fashionable". One chief of a charity commented:

> "The trust could see that this was quite good for them in some positional sort of sense, they were taking notice what patient groups were saying. And they wanted to be seen as progressive. So there were some wins for them." (Chief executive of a charity, interview 15, line 153-157)

The patient representatives believed that in many cases the current clinical and economic evidence was of limited value in making decisions about access to treatment. Hence, the patient organisations saw it as their task to raise awareness of the limitations of the current clinical and economic data, and complement the data by presenting evidence about the quality of life of patients receiving treatment compared to those untreated. The representatives explained:

> "Does it mean anything at all if you're measuring with a tool that is not actually picking up the key issues for the arthritis patients? So at that point if the trials are not telling that who can tell you that? That is where patient information and the patient group evidence becomes incredibly important." (Chief executive of a charity, interview 15, line 430-435)

> "And I think patient groups are one of the main pressure agency to push that, to just keep reminding people that it is not actually about numbers it's about feelings, and quality of life as well." (Head of public affairs of a patient organisation, interview 19, line 203-205)

The impact of the disease on the patient's life or the potential impact of the drug therapy on the quality of life of the patient was used as a key argument of the patient organisations to lobby for the use of a particular drug. For example, in the case of sildenafil the argument was based on the improvement of quality of life as one interviewee explained.

> "The problem with Viagra ® you could only argue about the quality of life, isn't it? But you can make the case for quality of life quite well." (Chief officer of a community health council, interview 14, line 309-310)

The same strategy was found in the case of rivastigmine where the patient organisation provided data about the benefit of the therapy, which did not receive the same recognition as clinical evidence.

> "We identified the outcomes that patients wanted and valued and they are very different from what clinical trials have been collecting. And I think that has been one of the most critical influences on the debate." (Head of public affairs of a patient organisation, interview 20, line 112-115)

The aim of the patient organisations was "filling in the gap in the data" with the research about patient's experience living with the condition. For example, stressing the consequences of living with ED was in the centre of the argument of patient organisations wanting funding of sildenafil.

The main difficulties for the representatives of the patient organisations was first, whether the right issues were considered in evaluating the drug treatment and second, whether they were measured in a way that represented reality. For instance one of the representatives said that for treatment of diabetes, the studies lacked the outcomes which are important for patients.

> "The great vast quantity of evidence that we receive is about blood sugar and some factors around it and it is defined and it's been categorised, structured, analysed in terms that are meaningful to the practitioners rather to the patient." (Chief of a patient organisation, interview 16, line 358-362)

More and more patient groups tried to collect their own evidence and had their own scientific advisors. The more widely accepted the scientific contribution (large sample sizes, statements from professional leaders), the stronger the argument.

> "I believe the fact that we have done research in this area has added to the credibility what would otherwise be seen as just a patient organisation. But by having solid independent research that informs your work and can inform health care delivery, that has added to its credibility." (Chief executive of a charity, interview 15, line 42-47)

Organisations who did not carry out their own research seemed to "lose out". Smaller patient organisations were clearly disadvantaged, since they did not have the financial resources to carry out research, which was perceived as unfair.

Backing their arguments with research was one way for patient organisations to increase their credibility and to counteract the argument of other decision-makers that the organisations were "emotional" and "hysterical". The research conducted was a

way to compete with what was regarded as a "rational" approach to decision-making. One chief executive of a patient organisation commented:

> "We carried out research. The responses you still see in the media, the noises made by some patient associations tend to be emotionally based. [...] Arguments based on 'This is desperately needed and people with condition x should have it', they don't get you anywhere." (Chief executive of a patient organisation, interview 16, line 86-94)

One of the main difficulties of using the patient's evidence was its evaluation along with clinical and economical data. There was the question about what constitutes lay evidence and how to validate it externally. On one side there was a call not to use individual experiences of the disease, on the other side this was often one of the most powerful arguments.

> "Most meetings I go the bit that I always walk away with is the individual powerful experience that's what I remember. It's what you go back with and talk about. It's not scientific, it's not evidence based but it changes our mind." (Head of public affair of a patient organisation, interview 20, line 418-421)

NICE appraisal committees, for example, allowed patient organisations to invite patient representatives to attend the committee meetings and give "evidence" of their personal experience with the disease and the therapy. However, the patient organisations carefully selected these representatives in order to guarantee that the "evidence" was presented in the most acceptable way for the committee, as one of the representatives of a patient organisation explained:

> "We had to select a person if you like, that we thought would be appropriate, and almost make the balanced case rather than making an over excited case, which is what I just said, which gets dismissed as an overexcited person who isn't sent representing of anyone else." (Head of public affairs of a patient organisation, interview 19, line 251-255)

In this case the acceptable way of giving evidence by individuals was their rational speech, which was not driven by emotions. A lay member of one of the NICE appraisal committee commented:

> "Probably in many cases the most effective evidence to be given at those appraisals committees to date has been the individuals with conditions who have experienced the technology in question. And they have come and in a kind of dispassionate way they have explained to the committee how it's like for them. And they have been honest about the pros and cons of the particular drug. [...] It wasn't an emotional 'Give me this drug!' it was presented in a dispassionate sort of way." (Chief executive of a lay member organisation, interview 15, line 464-471)

Some interviewees admitted that a good network of professional leaders making the case in favour of funding the treatment was a successful strategy to achieve a positive assessment. One interviewee believed:

> "If you know who is going to be sitting on the right committees... a lot of it is still 'old boys' networking' and I know that we have NICE now to get away from that whole issue. But if you know somebody on NICE or if you have got one of the members..." (Chief officer of the Community Health Council, interview 14, line 432-436)

The interviewee explained that it was important to have links to HCPs who "have the patient's heart at their heart". However, this was often only possible for large patient organisation with good links to key professionals.

The media

The media was used as a tool by different interest groups to influence decision-makers in various ways. In some cases it was a highly effective way to create public attention and achieve access to treatment, as one chief executive of a patient organisation illustrated:

> "I think a man in Plymouth, I think, it was, who couldn't get it, went to the Daily Mail and he got the drug. Another woman in Sheffield was on Channel 4 television and she got the drug before the programme was broadcast because of the screaming headlines and no one likes that." (Chief executive of a patient organisation, interview 16, line 216-221)

Although the representatives of the patient organisations were aware of the media's influence on decision-making, some of them warned that using the media could damage relationships. As mentioned before, maintaining relationships was one important aspect of decision-making.

Hence, many interviewees confirmed that a decision was made to avoid media coverage. One pharmaceutical advisor commented:

> "There's not that many Chief Executives who will find themselves happily on the front page of The Times or The Sunday Mirror, Sunday Mail, claiming that they are denying life-saving treatments. They will do anything to avoid saying that" (Pharmaceutical advisor, interview 4, line 832 – 837)".

This illustrates that there was a high fear of bad publicity stating that rationing was a reality in the NHS. The media was creating public expectations and public opposition against rationing decisions.

The media had indirect influence on regional committees in alerting some politicians about the funding decisions of regional bodies. The politicians then addressed the committees and demanded revision of the decisions made. The function of the media in addressing injustice in allocation of funding was seen as positive in some cases, although their methods of highlighting them were criticised.

> "But sometimes those are absolutely the right things to do. And it's frustrating though that it has taken the media and ministers to make it happen locally. But sometimes that is, in a sense, for an individual who is trying to campaign, that can be a more effective route, sometimes, than going through the right channels, which can take some time, I have to say. So I do understand that". (Chair of the regional specialist commissioning group, interview 8, line 356-361)

The law

Beside the goal of achieving cost containment, the account of the decision-makers indicate that generating legally defensible decisions was an important factor determining access to medicines.

Interviewees explained that the whole process of decision-making was designed to generate decisions that could not be challenged in court by governmental agencies, stakeholders such as pharmaceutical companies or individual patients. It was found that most of the interviewees used common terms such as "robust", "rigorous" or "due" to characterise a process generating legally defensible decisions.

> "So I am more about getting the process right and... the decision flow from that. And if you don't have the process right and you make decisions you can't carry then forward because you'll be challenged and it will all fall down. So if you don't get it right that doesn't hold up." (Chair of a regional specialist commissioning group, interview 8, line 698-701)

Some interviewees focused on that aspect of decision-making. The outcome of the decision was not important in safeguarding certain values; the process had to be designed in the way to enable defensible decisions.

> "If you can't demonstrate you've got a robust process then it's useless. So that is very important. And it's almost... it doesn't matter what your decision is when you come to the end. It's the process that's got to have been absolutely rigorous." (Pharmaceutical advisor, interview 4, line 1003-1008)

One member of a PCT prescribing committee expressed uncertainty about how a court would judge if a patient challenged the decision made about rivastigmine:

> "If a health authority was taken to court because they'd only given money for sixty patients and number sixty one took them to court, I don't know how that would stand up. [...] I assume that's the same. I assume the court would say - well, the due process has taken place and the funding decisions are down to the individual health authority to prioritise. So..... I assume, but I don't know. There's not been a test case, so we don't know. And I'd rather [prefer] that we weren't the health authority in the test case in the court really. But, at some point, maybe someone will do it. I don't know. It hasn't happened yet. (Pharmaceutical advisor, interview 2, line 1452-1455)

"Due process" was considered as a protection against the successful challenge by a patient. The quote above also shows that the potential liability of the PCT was perceived as a threat: "I'd rather [prefer] that we weren't the health authority in the test case....". Later in the same interview, the pharmaceutical advisor mentioned that the policy-makers in her PCT undertook a course on legal issues making her more confident that, as a result, the committee was able to achieve legally defensible decisions:

> "I think we are much more clear now that if we have gone through a due process that is seen as robust, then that decision will not be challenged in court." (Pharmaceutical advisor, interview 2, line 592-594)

Rivastigmine was also used by another pharmaceutical advisor as an example for a decision that could be challenged in court if the PCT guidelines on its use were not in line with the NICE guidance. The consultation of all relevant stakeholders was regarded as one important feature of a decision which was made according to "due" process.

> "You've got, you've taken on board everyone's comments, and you've come up with something that everyone's signed up to, that's a good process. You come up with a decision at the end. Which would be very difficult to challenge." (Pharmaceutical advisor, interview 4, line 1017-1021)

4.3.4.3 Views of the policy-makers and the stakeholders on the government and NICE

Many views of the interviewees concerning the role of the government were in agreement with the findings of the document analysis, such as acting in the interests of the pharmaceutical industry, which was seen as a particular British problem. Furthermore, the issues raised by the policy-makers and stakeholders were in line with some of the aspects derived from the literature and discussed previously in Chapter 1. For instance, the abolishment of postcode prescribing of high profile drugs was thought to create differences in other sectors of health care provision.

A novel finding was that interviewees were aware of individual members of parliament (MPs) who tried to influence decisions made by the PCTs. One advisor admitted that in case of donepezil, the first licensed AChEI, the influence of MPs was so powerful that it resulted in a revision of the decision against reimbursement being considered.

> "I think, with donepezil, it was very hard because we got a lot of pressure from patients and from MPs to fund donepezil. And we spent so much time responding to complaints from MPs. It was becoming a bit untenable, we knew we had to probably think about changing our decision". (Pharmaceutical advisor, interview 6, line 776-781)

The avoidance of organisational hassle, for instance spending "so much time responding to complaints", was another factor influencing health policy decision-making.

Another new finding was that some interviewees reported that NICE guidelines were used by the patient organisations or HCPs to support funding of the drug locally, which would exert pressure on the PCT.

> "We certainly found them useful. And it's very useful because we can go along to the health authority and say these are the guidelines and these are the ones we are going to follow. This is what has been recommended and we are not trying to do something different. All we want to do is to do what we are recommended to do. So that's a hugely powerful weapon actually." (Clinician in the care of the elderly, interview 10, lines 652-658)

4.3.4.4 Stakeholder representation and group dynamics

Although the organisation of the process of decision-making was described as a technical issue, it was strongly influenced by the various interests of each group of policy-makers and stakeholders. An important aspect of decision-making was the stakeholder representation in the advisory or decision-making committees. In the view of a pharmaceutical advisor, a PCT prescribing committee for example should include physicians working in primary and secondary care, commissioners, an economist, a HCP delivering the service under consideration and patient group representatives. She summarised:

> "Certainly, my experience, the last few years, it's been a – who do we need to have around the table to make this decision?" (Pharmaceutical advisor, interview 4, line 379-381)

The composition of the committees determined the direct influence of the interest groups on the decisions. Some interviewees acknowledged that the negotiations were influenced by the personality of the committee members, a fact, which some of them perceived as a threat to achieve rational decisions.

> "I think if there's no majority then it has to be a sort of, more discussion, and I think if you've got very strong charismatic characters who head committees they usually can carry colleagues with them." (Clinical governance pharmacist, interview 5, secondary care, line 344-347)

4.3.4.5 Summary of the political aspects

Overall, interactions between decision-makers played a major role in decision-making. Since the decisions were made in committees, group dynamics as well as maintaining relationships had influence on decisions. Traditionally, the medical professionals, especially physicians in secondary care have been one of the most powerful voices in the decision-making committees. They were in charge of prescribing and initiating the treatment used in secondary care, and had direct patient contact. In contrast, a considerable number of decision-makers had neither direct patient contact nor were mainly users. A key argument of the physicians was their expert knowledge about the drug therapy in questions and their experience in the use of it. In addition to citing clinical evidence as an argument, they could use the emotive patient's narrative to make a strong case for funding. In contrast, lay members felt they had to gain credibility, as they had no professional expertise. Their main difficulty was to

contribute with arguments that were respected in the same way as the one of the professionals. Evidence used by lay members was often anecdotal and not accepted by other decision-makers.

The pharmaceutical industry and the patient organisations were not committee members and only influenced decisions through their submissions, including clinical trials or pressures exercised on decision-makers. Recently the patient associations have been increasingly invited to play a more active role. Patient organisations often chose patients, who were members of the organisation, to represent patients' interests in a committee meeting. The pharmaceutical industry was keen to build good relationships with the patient associations since both parties had similar interests in achieving the reimbursement of a drug by the NHS. Their arguments were often phrased in similar terminology. However, the strong links between the industry and the patient organisations resulted in the accusation of "lobbying" and were seen as threatening rational decision-making by making emotional appeals. Besides links with other stakeholders, the industry also built relations with policy-makers to enforce their message as well as to lobby decision-makers to win them over to their interests. These interactions and dynamics between the decision-makers and stakeholders revealed important aspects about how policy was made.

Despite the use of technical data about the effect size of the drug and the overall cost impact there were many uncertainties related to the decisions about allocation. Each of the interest groups tried to fill the gaps of uncertainties in different ways. For instance, in case of the uncertainties about the clinical effect of rivastigmine, the Alzheimer's Society provided qualitative data showing that patients and carers in particular experienced significant benefit from treatment with rivastigmine. It was the intention of the Society to submit evidence from the patient perspective about the benefit of the treatment. Hence, part of the decision-making was the knowledge about what counts as a powerful argument, and the most effective way to present it.

4.3.5 Ethical aspects

The findings presented in the previous sections concentrated on the scientific analysis of the evidence or the interactions between policy-makers and stakeholders concerning gain or loss of power in influencing decision-making. The following part focuses on the ethical aspects of decision-making retrieved from the accounts of the policy-makers and stakeholders interviewed. Ethical aspects concern considerations about 'right' and 'wrong' or what 'ought' to happen in contrast to what happen in reality. In terms of decision-making about access to drug treatment, it is closely related to the issue of fairness.

The findings of the interviews show that in contrast to technical and political aspects, ethical concerns were mostly implicit. In the previous sections some of these implicit ethical considerations have already been mentioned. However, in some cases ethical aspects of decision-making were openly discussed. These cases had some common characteristics. They included drugs that:

- were thought to result in considerable cost expenditure,
- were used in a life-threatening condition or conditions leading to a considerable degree of disability,
- were used in conditions for which no other treatment alternatives exist,
- had benefits not widely accepted or their use not restricted to health care.

Rivastigmine, sildenafil and statins shared some of these characteristics. In addition, differences in the professional interests or between national and local priorities as well as dealing with individual cases often resulted in a discussion about ethical aspect of policy decisions. From the data six main ethical aspects were found: rule of rescue, utilitarism, definition of health care need, individual right and responsibility, equal access for equal need and transparency. Each of these aspects will be discussed in more detail in the following section.

<u>'Rule of rescue'</u>

All participants explained that they often felt obliged to follow the 'rule of rescue', which means to allocate more funding to the treatment of a patient in an acute life-threatening situation than to preventative treatment or to cover potential future expenditure (see also Section 4.3.3.5 above).

> "We had a couple of haemophiliacs, huge money. So I am talking about over a million pounds a year to keep somebody in treatment. Something as big as that would come to the board for a decision. And I have to tell you we have always agreed to continue or indeed to find the funding and give that person a life. Because that's at all costs, to keep people alive. [...] But there was never any doubt it was the right thing to do. Otherwise we would be condemning these people to death." (Chair of a PCT, interview 11 line 136-141, 450-452)

Cases of life-saving treatment were regarded as exceptional cases, handled differently and potentially overruled other commonly used criteria. For example, even in the absence of good quality of clinical evidence, extra funding would be agreed in order to fund a potentially life-saving treatment. (The possibility of approval of extra funding in the case of life-saving treatment is one example that shows that the budget was not necessarily fixed. It partly depends on the policy-makers to use extra sources of financial resources.) The participants' emotions, the clearly visible outcome of a life saved or prolonged for a certain period of time and the strong demand by the public led to a situation where refusal of funding was difficult. One interviewee referred to a case in which the government strongly advised the regional bodies to fund a certain type of cancer treatment under the pressure of public demand:

> "There is no evidence to support its use. Very limited amount of evidence. But there's no way chaps you are going to stop people having this. Because everyone is ready there to complain the instant we take away this supposedly life-saving drug" (Pharmaceutical advisor, interview 4, line 1298-1304).

In this case the policy-makers responded to the public's rule of rescue. Since the government commonly did not become involved in funding decisions of individual drugs, this illustrates how politically important it was for the government to guarantee funding. That cancer is one of the priority areas of the NHS might have contributed to the action chosen.

In general, cancer treatment was mentioned as an area where it was particularly difficult to make decisions about reimbursement of treatment. Beside the absence of RCTs, the lack of knowledge of many policy-makers about this specialist area, the high drug costs and their life-saving potential were some of the main difficulties in making a decision. Since the argument of a potentially life-saving treatment was powerful and difficult to counteract, interest groups used it as an instrument to impose pressure on decision-makers.

> "The oncologist, you know, accuse us of murdering all their patients because we won't let them give this, that and the other." (Consultant in medicine for the elderly, interview 7, line 719-721)

Not all life-prolonging treatment was provided. Interviewees distinguished between life-saving treatment, which restores full health and others, which only prolong life without curing the disease and leaving a patient with a certain degree of disability. The prolonging of life was not necessarily seen as positive as the comments of one interviewees illustrates:

> "Is this saving lives, is it simply extending a life for a few months which is going to die..?" (Chair of the regional specialist commissioning group, interview 8, line 192-193)

Curative, life-saving treatment were valued clearly higher than non-curative. Some responders explained that it would be necessary to take the quality of the lifetime gained into account.

The participants also made a distinction between potential life-saving treatment of individuals already diagnosed and individuals at risks of developing the disease. Even if prevention was generally preferred the interviewees saw a moral obligation in funding first the treatment of a disease before prevention would be considered for reimbursement. The interviewee used statins as an example:

> "Yes, you will introduce these things more easily initially when you've got somebody pretty crocked up with heart disease, and then it's perfectly reasonable they should take them." (Health policy analyst, interview 1, line 1006-1010)

Prevention was described as the "less sexy end", where it was politically more difficult to justify funding instead of life-saving treatment with its clear visible outcome. Similar critique was raised by stakeholders that most funding would be available for acute rather than chronic conditions.

Utilitarism

In general, the majority of interviewees expressed their belief that policy decisions had to be based on the principle of the greatest good for the greatest number of people.

> "I think it goes back to some of the things we were talking about in terms of looking at the needs of the population and where we get the greatest benefit for the population and what resources we've got available in terms of taking a

> healthcare policy decision." (Pharmaceutical advisor, interview 6, line 842-845)

For instance, in the case of statins, it was generally accepted that treatment could not be made available for all people who could benefit.

> "Well, no, I suppose what we've gone on is it's secondary prevention that's the must-do, and as you say, OK, the people that are at very high risk in primary prevention." (Regional pharmaceutical advisor, interview 4, line 206-210)

In contrast to the "must-do", treatment with statins for primary prevention under a certain risk level was called the "fancy bits" or the "nice dos", which is similar to the view of prevention as the "less sexy end", as mentioned before.

The lower priority of prevention compared to treatment might be related to the view that omission of an action with potential benefit was preferred over the commission of an action, which could cause harm. One interviewee referred to the case of statins, when he said:

> "[...] The costs of letting people die through neglect are very much lower than the costs of letting people die through overtreatment" (Health policy analyst, interview 1, line 316-317).

Definition of need

The term "need" was mostly mentioned in connection with the decision regarding sildenafil. One of the core problems of the decision was related to the definition of need for health care as one pharmaceutical advisor explained:

> "There is this clinical need. And it's very difficult with something like impotence to define what that need is". (Regional pharmaceutical advisor, interview 4, line 582-584)

Definition of need changed through times and was related to social and cultural factors.

> "(...) Ten years ago people would have accepted that [erectile dysfunction] was either part of diabetes or part of getting old, or whatever. You know, it's a life style issue, I suppose, as oppose to a disease" (Medicine information provider, interview 3, line 351-355).

One difficulty in the definition of need regarding sildenafil was the decision about the frequency of treatment necessary (i.e. the frequency of intercourse) as this affects the extent of need for treatment and therefore expenditure.

There was a discrepancy between policy-makers and stakeholders how they perceived the legitimacy to determine need. The policy-makers justified their action partly with their professional accountability for preventing 'abuse' of drugs and reducing waste of resources. The representatives of the patient organisation criticised that the need for treatment was assessed by policy-makers and often did not include the view of the patients. Some interviewees such as the pharmaceutical industry representatives questioned the legitimacy of the policy-makers in defining need.

> "I mean it depends on your classification of need and it depends on, you know, who do you think should be responsible for that? Should it be a doctor and a doctor patient relationship?" (Representative of the pharmaceutical industry, interview 13, line)

In making a decision about the need for a drug treatment the interviewees mentioned the necessity of taking the wider impact of drug treatment into account. Beside the clinical effect, the social impact, such as burden of care for the next kin and/or for society, needed to be considered together with the individual benefit. The interviewees felt that the border between social and health care need was difficult to define. This was not only the case for sildenafil; rivastigmine also resulted in discussion about balancing social versus health care need. The comment of one interviewee was characteristic in describing the considerations about health care versus social care and the predominate emphasis on regulating and rationing of drugs instead of other type of care.

> "I would be concerned about the arguments about - what's health care, what's social care and what's going to cost me and bankrupt me. [...] So I think seen against that background it brings out, to an extent, what sacred cows we've made of drugs treatments, compared with the other things." (Health policy analyst, interview 1, line 1477-80, 1484 -88)

In contrast to 'need', 'abuse' was defined as the use of drugs for fun and leisure. For instance, in the case of sildenafil it depended on the definition of the role of sexuality in human life and the importance of it for the individual if it is seen as pleasure or need or both, which made the decision about reimbursement difficult. The policy-makers argued that restoring "normal" functioning was an important aim of the health care system. Hence, the main arguments used by the patient organisations were the consequences of ED for the patients' lives. One interviewee explained how the case for sildenafil was made:

> "So it was about making it known to policymakers the *real* effect of withholding the treatment could have on that person. [...] And what we had to do is talk about the *devastating consequences of impotence to an individual and to a family and family group.* And we had cases of relationships splitting up and divorce happening when men experience long-term impotence. So instead of it making this something that you take on a Saturday night to make your girlfriend happy it's about dealing with *the trauma and a terrible psychological distress* and this could actually help people to deal with it." (Chief officer of a community Health Council, interview 14, line 128-130, 345-352)
> [my emphasis]

Only in claiming that the disease was affecting the patient's life to a degree in which the individual's normal life was not possible, was it judged as a legitimate need for treatment.

Individual rights and responsibilities

Some respondents mentioned the relationship between an individual's right to receive health care and their individual responsibility. In promoting the use of statins and making them available over-the-counter (OTC), people would be able to have access to treatment, which would extend their personal rights. However, it could also mean more responsibility for individuals in the prevention of diseases. This interviewee explained that in return, the state had the obligation to promote the use of statins, which was not happening at present.

> "[...] Do we make it clear to people that it might still be well worth their while, personally, especially if you've got a higher income, buying these products over and above what the NHS rationing allocation is, and make it very easy for people to do so?" (Health policy analyst, interview 1, line 1649-1655)

One interviewee explained that the preventative nature of statin treatment might have contributed to their rationing. Prevention was more likely to be seen as an individual responsibility rather than a priority of a publicly funded health service.

On one side, the change of the legal status of the drugs from POM to OTC could give the individuals more incentive to adhere to their medication since they purchase the drug at their own expense. On the other side, many interviewees feared that the strengthening of self-treatment would disadvantage individuals with lower income and widen health inequalities. For instance, a clinician interested in offering rivastigmine for NHS patients regretted that before the national guidelines were published, the PCT

had refused to fund the drug, which meant that treatment was only accessible to people who were able to pay for a private prescription:

> " But of course there were patients who could afford it. But as you probably know, these treatments...cholinesterase inhibitors are expensive. You know, it's a minimum of one hundred pounds a month to pay for them, and that's a lot of money, for many people." (Consultant in medicine for the elderly, interview 10, line 106-110)

Most interviewees supported a system of collective responsibility where health care was provided regardless of the ability to pay. Only the representatives of the pharmaceutical industry favoured a model in which patients would have more responsibility, together with an increased access to POM and consumer direct advertisement.

Very strict regulation of access to treatment could result in minimising the options provided at an individual level. Some interviewees regarded it as important to offer the clinicians as well as the patients some choice of treatment.

> "We don't want to set down that they have to...there are all these algorithms, where the patient has to sit there and conveyor belt along and if the patient doesn't fit that it's tough. We don't want to be there. We want to be able to respond to people. And also, I think, there needs to be the option of using different things to treat the same disease that will fit in with a patient. [...] Because there has to be an amount of allowance, some freedom for choice of what fits in with the patient". (Clinical governance pharmacist, interview 5, line 210-216, 284-286)

The patient organisation representatives especially emphasised the importance of choices as an individual right.

It was regarded as an individual's right to appeal against a decision made about funding and to claim that they are eligible for funding as an exceptional case. One solution to this type of request was the referral to a committee specifically dealing with these cases in which patients felt that they had the right of treatment and policy-makers had to acknowledge their "exceptional circumstances". The establishment of these committees could be seen as a defence mechanism against patient claims, where difficult decisions were referred to these committees dealing with exceptional circumstances.

> "The closest they come to saying no is – this drug will only be provided through, you know, one approved by, on an individual basis, once approved by an exceptional circumstance committee, for example. [...]. So that's less dramatic. [...] I think the cynic in me would say – they can say yes at an individual level, no at a policy level." (Medicine information provider, interview 3, line 676-690, 735-737)

However, interviewees admitted that the way in which requests were put forward to the committee was not necessarily fair since often only the cases of the most confident and persistent patients were put forward.

> "So you've got this committee, and they probably approve most exceptional circumstances, but actually you have to be quite keen I mean the doctor and the patient has to be quite keen to get themselves through to this level of decision-making." (Medicine information provider, interview 3, line 737-743)

This again shows the discrepancy between what interviewees thought should happen and what happens in practice.

Equal access for equal need

In the opinion of many interviewees the publication of guidelines was an instrument to counteract inequity successfully. Relying purely on individual clinicians to regulate access was seen as not sufficient in order to guarantee equal access to equal need. Referring to the criteria defined in the guidelines one interviewee said:

> "[..] These are the criteria, it's quite clear, everyone is being treated equally, that's another thing, it's all equity" (Pharmaceutical advisor, interview 4, line 1619-1625).

The criterion 'age' for priority setting was commonly rejected as seen from the critique of many responders who talked about examples, where rationing had been performed on the grounds of age. For example, the referral to a specialist was made less frequently for older patients than for younger. One reason was the system in place, which might disadvantage older people.

> "I wonder how many GPs do refer people with heart failure for specialist intervention. What other areas? And again, it tends to be in older people. That if you are say, old and diabetic you are managed by the GP, whereas if you are young and diabetic you are sent to a specialist clinic". (Consultant in medicine for the elderly, interview 9, line 295-300)

Some of the participants emphasised their commitment to protect the right of older people who they characterised as 'vulnerable'. Only one lay member referred to the

"fair innings" principle where the allocation of resource should be done in favour of younger people.

In addition, most of the responders agreed that patients who had practised unhealthy behaviour should be treated in the same way as patients who did not. In their opinion "punishment" of patients would result in further inequity.

Some representatives from patient interest groups raised their concern that the influence of their organisation was partly due to the large number of members and against the principle of equal access for equal need.

> "And I think that this is unfortunate in a way in that that is pressure from size. So, those patient groups or conditions where there are not so many of them how on earth do they motivate or bring to bear that pressure because there aren't that many. It's a bit unfair really." (Public affair representative of a patient organisation, interview 19, line 881-885)

Other policy-makers confirmed that the large membership organisations potentially had more influence on policy than smaller groups, which was judged as unfair.

Transparency

Transparency was mentioned as another crucial element of a fair process of priority setting, especially by stakeholders who highly depended on it in order to influence decision-making. The interviewees expressed mixed views about the transparency of different committees in the NHS. On one hand they recognised a clear tendency towards more transparency, which they welcomed. It was less likely that the decisions could be made in an "insidious" way and "behind closed doors". In contrast to some responders who saw this as sufficient to achieve transparency, other interviewees demanded further clarity and openness about decision-making. For example, some decisions were only announced within the NHS. In addition, the reasoning for decisions was not published and some interviewees interpreted that as dishonesty or "quasi"-transparency.

> "And in theory you can go to any health authority and look up their decisions on a website. But I don't think the no-decisions are on there. [...] So I don't think there is a transparency, per se" (Medicine information provider, interview 3, line 853-856).

As Martin *et al* (2002) described transparency can be divided in internal - within a committee - and external transparency. Concerning internal transparency, in some committees it was required to give information about possible links to pharmaceutical companies as described earlier. The transparency within the group was thought to enable a critical appraisal of the evidence used by the interest group to support their request, understand their arguments used and enable negotiation between the different parties.

Regarding external transparency representatives from patient organisations mentioned that there was no feedback provided to the kind of contributions made by them. It was not clear what kind of reasoning was used to accept some comments and to reject others.

> "But there is no feedback process. No one has ever said to us this is what we thought about your evidence, this is the impact it had. The guidance that was produced by the committee referred explicitly to the evidence submitted by us. What it didn't say was whether that was the individual sitting there or our written submission." (Chief executive of a patient organisation, interview 16, line 73-80)

In the view of some participants, the strengthening of local autonomy resulted in more transparency and accountability of decision-makers since HCPs involved on local level were known by their peers and had more direct contact with them. A disadvantage was the increasing number of local decision-making bodies, which were an obstacle towards transparency.

4.3.5.1 Summary of ethical aspects

In summary, the decisions made about rivastigmine, statins and sildenafil illustrated various ethical aspects of policy-making. Instead of talking explicitly about ethical dilemmas involved in decision-making, policy-makers as well as stakeholders used a more implicit description of values. However, it became apparent that all of them had a strong sense of what they perceived as being "fair" and/or "just". The data also suggest that there were some common, underlying values, which most of the decision-makers shared and were raised in one form or another in all of the interviews. The main issues were the rule of rescue, social and procedural justice, and need as the main determinant for access to treatment.

The rule of rescue had a high priority and was a powerful argument used by various interest groups. The lives of identifiable individuals received higher priority than unidentified individuals. In an intuitive ranking of funding priorities saving of lives had the highest priority, which was followed by treatment. Prevention came last. The aspect of social justice was tangible in the concern of equal access for equal needs. Age, individual life style and socio-economic considerations were commonly rejected as criteria for priority setting, although many stakeholders were concerned that age could play a significant role of rationing on the micro-level.

Procedural justice received much attention by policy-makers as well as stakeholders. It included safeguarding both internal and external transparency, the participation of all relevant stakeholders and the opportunity to appeal. All of them are criteria of Daniels and Sabin's (1998) framework of 'accountability for reasonableness'. Compared to regional guidelines, most stakeholders favoured national guidelines as they regarded them as fairer in achieving equal share of resources and transparency. This is in agreement with the recent trend in the NHS whereby decisions about access to new therapies are increasingly made on a national level. However, some decision-makers warned that this was no guarantee for procedural justice. It potentially resulted in regional differences in other health care services and a trend towards "quasi-transparency". In addition, policy-makers mentioned that the introduction of an appeal mechanism to consider exceptional circumstances would not necessarily guarantee that all exceptional cases were heard.

The discussion about the boundaries of health care and about the definition of need for treatment was most overt in case of sildenafil and partly in the decision about rivastigmine. Considerations of restoring or preservation of "normal" functioning, as Daniels (1985) and Doyal (1995) suggested, was only partly applied. For instance, psychological causes of ED on individuals were given some consideration. However, while treatment for ED of physical origin was available in primary care, therapy for ED of psychological origin was only accessible through specialists even though both groups of sufferers experienced the same disadvantage in terms of "normal" functioning.

Ethical considerations did not take primacy over all other factors influencing decision-making, nor did policy-makers have a fixed hierarchical order in which they applied ethical principles. Instead of referring to a certain ethical framework, the policy-makers used predominantly common sense to decide about what is regarded as "right" and what as "wrong". Ethical considerations went along with other political and technical concerns. Policy-makers made several compromises, for example the principle of equal access for equal needs was overruled in order to safeguard expenditure.

4.4 Discussion of the results of the in-depth interviews

The results of the interviews confirm as well as complement the findings of the document analysis in various ways. In addition to the views of the interviewees on the decision-making about the three drug therapies, the interviews enable the exploration of a wider picture of reimbursement decisions. This included the participants' views on what 'ought' to happen and the reasons why decisions could not be made in an ideal way. Furthermore, the interviews highlight that decision-making is mainly an interactive process between policy-makers and influenced by stakeholders.

The document analysis showed that the decisions appeared to be derived directly from the clinical evidence and implemented without much adjustment. The findings of the interviews reveal a more complex picture of health policy decision-making: the clinical and economic data were used as a tool to generate legally and politically defensible decisions as well as constructing and negotiating the access criteria to treatment. Furthermore, "informal" factors such as personal and political considerations were found to have a significant role in decision-making, but have been neglected in the theoretical and empirical work about health policy decision-making. The findings of the study are in line with other research in the field arguing that the current assessment of health technologies, including drug therapies, commonly lacks consideration of social and ethical factors and an awareness that technical measures incorporate political and value judgements. The findings suggest that minimising uncertainty and legitimisation of decisions were two important driving factors in health policy

decision-making. However, before going into discussing the results in detail, the limitations of the interview findings will be highlighted.

4.4.1 Limitations of the interview findings

The professional background of the researcher was known to the interviewees, which may have influenced their answers (Silverman, 1993). Since many of the interviewees were HCPs, it is likely that they used their professional terminology. Some of them might have felt more open in talking to a professional colleague. Others might have been more hesitant to utter negative opinions about pharmacists as policy-makers. In particular, comments about pharmacists and their work were potentially more positive. The transcripts were read carefully to check for this influence. As Hammersley and Atkinson (1995) described, the analysis was conducted with awareness and self-conscious reflection for the influence of the researcher on the data.

Field notes were made to support reflection on any preconceptions of the researcher which could explain some variation in the way the questions were asked or in the reactions of the interviewees. During the analysis, the meetings with two other researchers from different research disciplines (pharmacy and philosophy) supported a critical reflection of the key themes identified and the theories and hypotheses developed. Janesick (1994) discussed the advantages of a multidisciplinary approach. In the present study it was regarded as fruitful in identifying themes related to both pharmacy and ethics.

The study was conducted in the London Region, which includes many highly specialised services. The interviewees assumed that in other areas of Britain decisions were made under different circumstances and influences. As qualitative research does not aim to generate statistical generalisation (Murphy *et al*, 1998) the described interaction between various interest groups and their influence cannot be directly transferred to other parts of the country or to other health care systems. However, the data can be indicative for significant pattern in health policy-making in the NHS and potentially in other public funded health care systems. They allow the drawing of a picture of authoritative decision-making about access to drug therapies.

In contrast to previous research which focused on one or two clinical specialities (Martin *et al*, 2001) or one type of policy-makers (McDonald *et al*, 2001), three different kinds of drug therapies were chosen and a variety of policy-makers and stakeholders interviewed. Although the data are not extensively describing all potential influencing factors, in using a selection of medicines and a variety sample of participants it was possible to describe some possible scenarios that are repetitive and recognisable in many other decisions.

The participants described how decisions about statins, sildenafil and rivastigmine have been made in the past. Their recall might have been inaccurate and influenced by the events after the decisions were made. Hence, their accounts were treated as a reflection and individual interpretation of the decisions made rather than a historical description of the events. It cannot be concluded from the study if the participants acted in the way they described. Denzin (1989) argued that there is no reason to assume that the interviewees give a true account of their experiences and views, although it is more likely that with established good rapport they will feel more able to do so. Therefore, the interviewer tried to create an atmosphere in which the interviewee felt at ease.

The following discussion of the results takes these limitations into account.

4.4.2 Construction and negotiation of access criteria

The document analysis showed that access criteria were derived from diagnostic or clinical markers. This gave the impression of accuracy, objectivity and certainty, although a closer analysis revealed that there was much uncertainty related to their definition and implication in practice, which could disadvantage certain patient groups. While the findings of the document analysis were inconclusive about the 'how' and the 'why' of defining access in this way, the interviews showed that the reason for defining access criteria in the way described was primarily to ensure cost containment. For instance, in the case of statins, where the provision of therapy to all patients who could potentially benefit was thought to far outreach the financial resources available, the diagnostic marker of the annual risk of developing CHD was used to define the group of patients with the greatest capacity to benefit. The size of the group was

determined according to the financial resources available. The group of patients requiring treatment for secondary prevention was chosen, because first, it was possible to fund treatment for this group and second, the capacity to benefit from treatment surpasses the one of the patient group requiring statins for primary prevention.

This way of defining guidelines has been described by Dewar (2000) for the case of sildenafil, where at first glance access criteria appeared to be chosen arbitrarily. In fact, the aim was maintaining expenditure at a level of previous budget allocation for this area of treatment rather than considering a new allocation on the basis of need and greater ability to respond to need. Policy-makers and stakeholders in the present study explained that a greater response to need was hampered by the difficulty of reorganisation of budget allocation during the financial year. Urgent funding for a newly marketed drug could only be provided on the basis of the budget available for these cases as an "ad hoc" solution.

Defining access criteria in the way the decision-makers described means that they are partly based on the capacity to benefit (except for sildenafil, where this is not the case). The guidelines for the treatment with statins state that the criteria were set according to "need" (NSF, 2000), which means that need is defined on the basis of the extent of clinical benefit, determined from RCTs. Hence, the need for treatment was based on a very biomedical concept of health compared to a wider view of health implementing socio-economic determinants beside the biomedical ones.

As previously mentioned the criteria give a picture of an accurate and thorough process to determine access to treatment. This was partly reflected in the account of the policy-makers who believed that the clinical markers would result in objective and consistent decision-making on an individual level nation wide. Two studies investigating micro allocation decisions and guidelines defining access to treatment indicate that - in contrast to the belief of consistency and objectivity - there is potential for significant variation in the interpretation of the access criteria, which potentially result in considerable discrimination of certain patient groups (Varekamp *et al*, 1998; Hughes *et al*, 1997). Varekamp *et al* (1998) investigated the use of "medical urgency" as a determinant of resource allocation for renal transplantation and psychogeriatric nursing home care. They found considerable variation in the decision-making and use of the

criterion, which they related to the different attitudes of the HCPs towards clinical criteria as a tool to regulate access. Hughes *et al* (1997) studied micro allocation decisions in the NHS and found that access to cardio-surgical interventions and neurological rehabilitation was significantly influenced by the doctors' individual judgements of the patients' social and demographic factors, rather than a decision purely based on diagnostic or clinical markers commonly used in guidelines.

Hence, using guidelines to define access to treatment means a shift in the use of clinical trial data from being advisory about the use of the drug in terms of safety and efficacy, to being used to support a gatekeeper function (Shulman, 2001).

4.4.3 The importance of a legally defensible decisions

The reason for defining access criteria in a certain way was mainly cost containment. The strong emphasis on the financial implications for the organisation is probably enforced by the legal framework of PCTs, which states that according to the NHS Act 1977 (Section 97D) "each PCT has a duty not to spend more then the sum of the amount allocated to them by their Health Authority (HA) (the cash limit) and any other receipts" (Department of Health, 2000c). Hence, achieving cost containment was part of generating legally and politically defensible decisions, which was the predominant goal of decision-makers.

It was not only the governmental agencies who could challenge the decision-making of the HA, patients could also appeal against the decisions made. In Britain the case of Jaymee Bowen, which gained high media interest, has certainly influenced the design of the decision-making process to be defensible (Ham, 1999b). In the case of this girl, known as 'Child B', a HA decided against the funding of a second bone marrow transplant, costing about £75,000, to treat her relapse of acute myeloid leukaemia, since the treating paediatrician advised against it and estimated a chance of 10% of achieving remission. In summarising the lessons which could be learned from the case, Ham (1999b) wrote: "Given that there will often be controversy over tragic choices in health care, it is incumbent on those responsible for decision-making to show that they have followed due processes and have been both rigorous and fair in arriving at their decisions." He draws a comparison between the legal and the health care system and

points out that the health care system might need to implement "quasi-judicial features".

The denial of funding treatment with beta interferon by an English HA, and the following High Court decision that this was unlawful, got less public attention than the Child B case. Nevertheless, it had important implications for policy-makers at local level in being prepared for a challenge by individual patients or stakeholders in court (Dyer, 1997). The case underlined the importance of having no blanket bans and considering individual cases in a formal appeal process. (The local policy-makers in the present study confirmed this and described the introduction of committees to deal with cases of appeal.) As a consequence of these historical incidents, legal defensibility ranks as the first goal of the policy decision-making for regional decision-makers (Jarivs, 2001).

The historical cases also showed the way in which decisions are more likely to be defensible in court. One of the difficulties in generating legally defensible decisions is that, although the NHS offers a 'comprehensive' service, de facto there is no list of 'core' services that receive priority. This means there is no direct right to certain health service resources (Newdick, 1998). Instead, the Secretary of State's duty is 'to meet all reasonable requirements'. However, this means that there is certain flexibility over what is defined as 'reasonable'. Previous court cases established that the court did not accept the refusal of a HA to fund a service if it was based on the argument of costs alone (Newdick, 1995). For example, in the Child B case the HA was not challenged because the HA gave clinical reasons such as the limited chance of remission as the main justification for denial of access and not financial reasons. Hence, the clinical evidence has become increasingly important as Newdick (1995) comments, "but the very fact that such clinical evidence were required would focus minds on ensuring that these unenviable decisions are reasonable and defensible and would help to satisfy patients and public that the questions have been properly addressed". The clinical evidence has started to play the role of demonstrating reasonableness and defensibility in the eye of the public.

Using a technology assessment, or clinical and economic reviews, as the basis for decision-making supported the view that the decisions were made in a "rational" way.

The systematic and rigorous assessment was regarded as protecting against arbitrariness and "unfairness". However, the degree to which the evidence was debatable and how much it relied on interpretation was often neglected or not mentioned at all. Rodwin (2001) concluded that evidence-based medicine was used as a political tool, even though decision-makers described evidence-based medicine as a way of replacing politics. As Frith (1999) pointed out, the interpretation of evidence from RCTs is often presented as "pre-existing facts waiting to be discovered by medical science". This is in line with the results of the study presented in this thesis indicating that policy-makers had the tendency to regard the evidence derived from this type of research as facts which are value-free and neutral. Other health policy analysts have also described this view of evidence as replacing values whereas in fact, it implicitly incorporates them (Nordheim, 2002).

The way in which interviewees emphasised the importance of using clinical and economical evidence as a basis for making reimbursement decisions shows its relevance. Although the document analysis and interviews revealed the tendency to describe the function of the evidence as directly determining the decisions, the results of the study suggest that it had often a symbolic and legitimising function.

4.4.4 "Informal factors"

Following on from this, the results of the interviews allowed exploration of the factors that were more hidden behind the view that policy decisions were purely based on technical data of efficacy and cost-effectiveness. In the following, it will be argued that these factors have received less attention in the theoretical and empirical studies of health policy decision-making, but nevertheless have considerable impact on the way how and why decisions are made. The present study gives a detailed account of these "informal" factors and the way in which they influenced decisions. These aspects included personal factors such as excitement about the novelty and the potential benefit of the drug therapy as well as the desirable ends of decision-making, for example the importance of the continuation of the relationships with policy-makers or stakeholders and the avoidance of organisational burden.

The excitement about the novelty and the potential benefits of the therapy might have been an important factor for a decision in favour of continuing the funding of rivastigmine. The evidence about the benefit of rivastigmine was contested and does not necessarily explain why a decision in favour of funding was made. In addition, statins were frequently discussed as "exciting". That excitement about the novelty and potentially benefit of a treatment influences decisions has also been found by Jenkings and Barber (2003) in studying decision-making in DTCs in secondary care. An alternative explanation of why novel therapies received a more positive assessment may be the desire for good news or hope for the treatment of stressing conditions (Heitman, 1998). Arguments for or against the new drug therapy can shift from symbolising 'hope' or 'threat' depending on the agenda of the policy-makers or stakeholders. The pharmaceutical company representatives in this sample tended to emphasis the 'hope' in relation to the introduction of the drug therapy. Others talked about the 'momentum of technology', where the question about 'whether' is replaced by the question 'when' (Brown *et al*, 2000).

Another factor influencing decision-making was the importance of maintaining relationships, for example between decision-makers and influential doctors. Halpern and Stern (1999) highlighted that the continuation of relationships can have considerable impact on decision-making, especially in organisations. They referred to social decision theory, which predicts that decisions are made in order to preserve social relationships. Finally, the management of the organisational burden was found to be of importance, which means the decision-makers took the 'total' costs of the decision into consideration, such as the financial costs of change as well as the potential loss in trust.

In Klein's (2002) opinion it is necessary to include more than the commonly used rationales in policy decision-making. He called this the need for 'reality judgement' where decision-making takes into account implications and acceptability for the relevant interest parties. However, these other 'pragmatic considerations' of policy-makers have received less attention. That the commonly used rationales are in the foreground might have two reasons: First, personal factors may be regarded as arbitrary and "less" valuable to make decisions. Second, their influence may undermine the trust of the public in the authoritative decision-making and result in

opposition or change of the current form of health care funding in solidarity. New (1996) describes a form of social reassurance and stability as one of the goals of a health service, which might be in danger by acknowledgement of these personal and political factors if they are generally judged as arbitrary. However, the data from the present study showed that not all of these informal factors are necessarily arbitrary or 'unreasonable'. For example sustaining good relationships between policy-makers and HCPs is reasonable as well as an important consideration. It is likely that a more careful analysis on a case-to-case basis could help to distinguish between relevant, reasonable and arbitrary factors.

However, the results suggest that these factors were a relevant part of health policy decision-making that many studies of health care priority setting have neglected. The need to legitimise the decision was one of the factors that contributed to the masking of these informal factors, which interviewees often characterised as "noise" in the system. Although being accountable for their action may improve decision-making, it may also result in political tactics to satisfy stakeholders or 'sell the message' to them or the media. The need to justify decisions potentially makes it more difficult to reveal what is hidden beside the facts that are made explicit.

4.4.5 Limitation of the evidence used as a basis to inform decision-makers

Clinical evidence about the efficacy and economic data concerning the treatment were used as the main information bases to decide about the provision of access to the medicines. Relying on a very specific set of data to assess the characteristics of a drug therapy had several consequences; there was uncertainty about external validity of the data, some patient groups were potentially disadvantaged regarding access to medicines, and the absence of clinical or economic data was used to defer decisions. Although other authors have discussed these limitations, the examples of the three drug therapies chosen illustrate them and complement the previous findings.

Uncertainty about external validity

One limitation was the uncertainty of the policy-makers about the external validity, the extent to which the results of the clinical and economic studies were applicable to

widespread clinical use. One reason was that the majority of the studies were carried out for the licensing process, with the main purpose of assessing efficacy (quality and quantity of the benefit under trial condition) and safety, which was different from the task of policy-makers (as mentioned previously in Section 3.5.2).

Dobrow *et al* (2003) describe that an understanding of so called 'external contextual' factors, which are not considered in the trial but nevertheless influence the context in which the evidence is applied, as essential for the understanding and applicability of the evidence in practice. Adherence to treatment was one of the external factors that policy-makers in the present study mentioned as related to the external validity and the uncertainty of the effect of the medicines in practice. In the case of statins, some of the interviewees voiced their concern about potential disinvestment due to non-adherence. To prevent disinvestment the access criteria of rivastigmine included the assessment of the likelihood of adherence to treatment. However, it is less clear how this criterion can be assessed in an objective and reliable way. The likely uptake of the new drug therapies by doctors was another external factor, which contributed to the uncertainty about the financial impact about the drugs. Since beliefs, attitudes and the experience of HCPs influenced their use of the new therapies, it was difficult to predict (Rosen and Gabbay, 1999). Some stakeholders used the absence of data to create potential fears about uncontrolled cost expenditure caused by a large uptake of the new therapy, for example in the case of statins or sildenafil.

The preference for certain outcome measures

The results of both the document analysis and the interviews showed that there was a preference to use outcome which were more easily measured and to neglect the social and ethical implications of the therapy in question. This had significant impact on the decisions, which will now be described in more detail. For instance, clinical outcome measures were preferred compared to other measures such as quality of life. One reason was that the measurement tools for the latter were thought to be of insufficient reliability or validity. Furthermore, there was an absence of qualitative data about the impact of the disease on the individual and the effect of the treatment. Stakeholders gathered data to complement the clinical and economic data cited in the health technology assessment. The representatives of the patient organisations for AD and

diabetes patients illustrated this for the cases of rivastigmine and sildenafil respectively. However, the decision-makers reported difficulties in using the qualitative data gathered by patient organisations alongside with quantitative data derived from clinical or economic studies. This was due to the absence of consensus as to how to assess the personal experiences in an objective and reliable way, which made it difficult to use it along evidence gained by statistical means.

There was also the tendency to prefer outcomes which had an immediate effect on the patient's life over intermediate outcomes such as cognitive function. This is surprising considering the fact that, for instance in the case of sildenafil, the outcome which had an immediate effect was assessed subjectively compared to the assessment of cognitive function which was done by HCPs. That outcomes which had an immediate effect were preferred might be another reason why decision-makers prioritised saving the life of an identifiable person over prevention. Klein (1993) explained the tendency to fund life-saving interventions even when this means other treatment can not be funded as a consequence with the NHS having a "symbolic role in society" demonstrating the "society's willingness to care". Hope (2001) discussed arguments for and against this preference. One argument is that a more visible outcome (a life prolonged which was otherwise lost) might create more agreement and popularity among the public. Interviewees in the study confirmed this with their descriptions of avoiding media coverage and being accused of denying funding especially for life threatening conditions. Hope's central critique of the discrimination against prevention is that with the saving of one visible life many others will lose their lives in the future, which cannot easily be justified.

There was a lack of evaluation of the social outcomes and ethical consequences of the introduction of new therapies even though the definition of HTA includes these aspects. "A HTA studies the medical, social, ethical and economic implications of development, diffusion, and the use of health technology and informs policy decisions. Its aim is to improve the quality and cost-effectiveness of healthcare" (NHS Centre for Reviews and Dissemination, 2003). However, the social and ethical implications of the drug therapies were generally not rigorously and systematically evaluated, for instance the social implications of therapy with rivastigmine on carers were not studied. Johri and Lehoux (2003) highlighted the importance of social, political and ethical

dimensions of the technology assessment; for example, the technology potentially changes social relationships. In the case of rivastigmine, the patient organisation referred to such change in the social relationship between carer and patient. However, their data were based on a survey conducted by the Alzheimer's Society and it is unlikely that smaller patient groups will be able to do similarly. Banta (2003) suggested that the paucity of ethical and social evaluation is probably because social and ethical analysis has not developed at the same pace as quantitative forms of assessment. The present study suggests that most of the decision-makers were HCPs and not familiar with research from other disciplines.

Lehoux and Blume (2000) warned that defining health technology, including drug therapies, in a very narrow way would diminish the quality of the assessment. Instead, they called for a broadening of the agenda including much wider social and ethical aspects. In doing so, they believed that it potentially improves the dialogue between stakeholders and policy-makers and the outcome of decision-making. Giacomini (1999) argued that the definition of health technologies, including drug therapies, and their boundaries in particular has important implications for the assembly of choices. She pointed out that the assessments of new therapies often conceal the assumptions that are built into them. The comparatives chosen might move certain features of the therapy to the foreground and hide others. The case of rivastigmine illustrated that. For instance, one interviewee mentioned that at one point in the discussion, social care was seen as the alternative to the funding of rivastigmine, which the patient organisation judged to be inappropriate. Social care was regarded as an inadequate replacement for drug therapy.

Limited evidence

Although there was the call for high quality of evidence on clinical efficacy and cost effectiveness, many interviewees admitted that there was often very little evidence of sufficient quality to use it as a basis to make a decision about access provision. The paucity of evidence had several consequences. First, the absence of evidence about comparative studies resulted in a potential distortion of the treatment benefit. This was mentioned in discussing the results of the document analysis regarding the absence of comparative studies for rivastigmine versus other AChEIs. Some of the interviewees

mentioned this as a difficulty with the cost-effectiveness assessment compared to other health care or social care interventions.

Second, the absence of evidence resulted in the exclusion of certain groups of the population from the benefits of the intervention since they were less likely to be included in clinical trials. For instance, from the interviews and from the document analysis, it became apparent that the limited evidence about the clinical effectiveness of statins in the elderly resulted in excluding older people from treatment recommendations in the SMAC guidance.

Third, the limited amount of evidence was sometimes used to defer decisions until certain evidence was available, a tendency which has also been described by van den Heuvel *et al* (1997) investigating utilisation of technology assessments in Dutch policy decision-making. They used the six ideal types of research findings utilisation according to Weiss to demonstrate that technology assessments have various functions in policy-making. One of them is tactical purpose where policy-makers can postpone a decision with the excuse that more research is necessary. There was no consensus about the amount of evidence necessary to provide access to treatment. In the case of rivastigmine many regional committees deferred the decision on the ground of an absence of evidence. Similarly, in the case of sildenafil the government deferred the decision until expert assessment of evidence was available.

In summary, relying on a very limited set of data as the basis to make health policy decisions had considerable consequences for the outcome of the decisions and the way in which they were made. In the context of limited evidence, Klein (1993) highlighted that there will always be the problem of inadequate information and uncertainty even though technology assessments will be carried out. However, the tendency to rely mainly on a very specific type of data, resulting in the disadvantage of certain groups, is risking the goals of a fair way to make health policy decisions. In addition, the results showed that in situations of limited data about the clinical and financial implications of providing access to treatment, other "informal" factors were more overt.

In conducting empirical research using two distinctive methods of data collection it was possible to obtain a detailed account of public decision-making about access to health care. Whereas the document analysis was focusing on the results of the policy decisions, the in-depth interviews with a range of policy-makers and stakeholders enabled important aspects of the process and the reasons for the decisions to be highlighted. Taking political and legal defensibility as well as personal and political factors into account complements the understanding of the way in which health policy decisions about access to treatment are made.

PART C: INDIVIDUAL DECISION-MAKING ABOUT CHOICES OF MEDICINES

Part C addresses the second aim of the thesis, to investigate in secondary care the health care professionals (HCPs)' and the patients' views on decision-making about choices of medicines within the medical consultation, including the use of IC for medicines.

CHAPTER 5: QUALITATIVE STUDY OF THE HEALTH CARE PROFESSIONALS' AND PATIENTS' VIEWS ON DECISION-MAKING ABOUT CHOICES OF MEDICINES

This chapter presents the qualitative study, which aims to investigate the views of HCPs and patients on decision-making about choices of medicines in secondary care, including the use of IC for medicine. The description of the study's objectives and methods is followed by the analysis of the interviews with HCPs and patients. Finally, the chapter ends with a discussion in which the accounts of the participants will be considered and placed in the context of the relevant literature.

5.1 Objectives of the study

The study had three main objectives:

- To investigate the perception of the current practice of giving information about medicines prescribed in hospital including information about costs and presenting treatment choices to patients.
- To explore what role and process doctors and hospital in- and outpatients prefer in making choices about the treatment with medicines at the time of initiation of treatment.
- To investigate the views of doctors, nurses and hospital in- and outpatients on IC for medicines, its benefits, risks and feasibility in practice.

5.2 Methods

5.2.1 Choice of methods and settings

5.2.1.1 In-depth interviews

As described in Chapter 2, little is known about the patients' and doctors' views on choices and reasoning why certain models of doctor-patient interactions are preferred. Therefore, a qualitative method instead of a quantitative method was chosen for the present study. Quantitative methods are thought to be suitable for testing existing hypotheses (Bowling, 1999a), but they are considered inappropriate for the generation of new ones. Some qualitative studies use observation and tape-recording of consultations to investigate doctor-patient encounters and communications. However, their main focus is on aspects of actual patient-doctor interaction, and therefore, they do not reveal the motivation and reasoning for action. Although focus groups are commonly used in qualitative research, they require that the participants are mobile, will invest time and have confidence and the ability to express their opinion in the presence of many other participants. For example, Kitzinger (1995) reports that she experienced difficulties when gathering the views of nursing home residents when some participants tried to discourage others from voicing their criticism about the staff. Finally, although focus groups can explore issues in breadth, they often do not allow an exploration in depth (Reed and Payton, 1997). Taking the aims of the study and the limitations of each research method into account, face-to-face interviews with the participants were considered the most suitable format to meet the study requirements.

5.2.1.2 Settings

Between November 2001 and January 2002 and between November 2002 and June 2003, study participants were recruited from hospital wards and outpatient clinics at a London Teaching hospital. Reasons for the choice of hospital ward setting was that first, hospital inpatients commonly receive their medication from the nursing staff on the wards without any written information about their medicines, whereas patients in primary care or outpatients have the opportunity to read patient information leaflets before they take their medicines (Raynor and Britten, 2001). Hence, it was assumed that it is more difficult for hospital inpatients to inform themselves about their medicines and to make an informed decision if they want to take the medicine or not.

Second, there is usually more than one doctor prescribing medicines for the patient and the patient is less familiar with the doctors, which makes it more difficult for the patient to be involved in decision-making. Third and perhaps most importantly, in secondary care there is an increased use of medicines with potentially severe adverse effects, so patient involvement in decisions associated with serious risks might be considered more desirable (Rosenberg, 2001).

Between April and May 2001 a small pilot study was conducted to identify medicines that are both commonly used on general medical wards and associated with serious risks. After discussion with clinicians and a survey of the drugs used on the wards, it was decided to include three drugs: amiodarone, digoxin and warfarin.

Patients recruited on a cardio-surgical ward commonly underwent surgery. It was assumed that this situation might influence their attitude towards decision-making about medicines. In previous studies it has been found that patients who are more severely ill had preference for the doctor making the decision for them (Blanchard *et al*, 1988; Ende *et al*, 1989). However, it should be noted that the previous studies investigated various health care decisions rather than decisions specifically regarding medicines.

For the study purpose it was desirable to include in the sample non-surgical patients and patients with different types of conditions varying in severity. Apart from inpatients it was the study aim to recruit participants who did not suffer from an acute life-threatening condition and were more independent than hospital inpatients. Hence an outpatient setting was chosen, which also offered the advantage to recruit patients who had contact with a doctor working in primary care as well as a doctor who was based at a hospital. This provided the opportunity to investigate if patients perceived a difference between the health care settings regarding decision-making and in what way they thought it would influence decision-making. It has been argued that shared decision-making is more likely to take place in a primary health care setting where patients and doctors are well known to each other (Rosenberg, 2001).

The setting of the cardiology and urology clinics offered the opportunity to sample patients with a range of acute and chronic diseases of different degrees of severity and

HCPs experienced in treating patients with very different conditions. Since there was only a limited number of doctors working in the cardiology and urology outpatient clinics, it was necessary to recruit doctors from other clinics. Rheumatology and endocrinology outpatient clinics were chosen because it was expected that the doctors would see mostly patients with a chronic condition. In addition, certain treatments in the outpatient clinics are rationed in the NHS, which limited the range of the treatment options offered by the health care provider. In rheumatology, newer drugs (for instance, certain immune modulating drugs) were only offered to patients who had not responded to conventional disease modifying drugs. Additionally, certain analgesics such as selective inhibitors of cyclo-oxygenase-2 were restricted to patients with certain risk factors. Similarly, in cardiology, lipid-regulating drugs were rationed in primary prevention for patients with a risk of less than 3% of annual CHD risk, and in urology, treatment for erectile dysfunction was only available for certain indications. This would offer the opportunity to ask patients and doctors about the impact of rationing on decision-making.

5.2.2 Ethical approval

Ethical approval for the recruitment of hospital inpatients, outpatients and the conduct of interviews with hospital staff was obtained from the local research ethics committees.

5.2.3 Sample and participant recruitment

5.2.3.1 Hospital inpatients and HCPs on the wards

Hospital inpatients and HCPs were recruited on a cardiology / cardio-surgical ward. This was the ward where the drugs included in the study were most frequently used. Since there were only a limited number of HCPs based on the ward it was necessary to recruit HCPs from other wards such as the coronary care unit or the haematology wards where the medicines concerned were also used, though less frequently. The consultants and nursing managers working on these wards agreed to the study.

Using the drug chart at the end of the patients' bedside the researcher identified patients who were newly prescribed one of the drugs studied. Adult patients were

asked if they were interested in taking part in a study concerning their medicines and the information they received about them. If they confirmed their interest they were given a leaflet, which explained the purpose of the study and what it involved (see Appendix 5.2.1). The researcher explained the aim of the study and answered questions. In the majority of cases potential participants were given a day to consider their participation. If the patients so desired, the interview was held on the same day. Before the start of the interview the patient signed a consent form stating that they had received information about the study and that they were aware that they could discontinue the interview at any time. Recruitment of patients continued until no new major themes emerged.

A purposive sample of HCPs was recruited; as age and professional experience may influence views, the researcher deliberately asked nurses of various ages and doctors in different positions (senior house officers (SHOs), registrars and consultants) to participate in the study. If the HCPs expressed interest, a convenient time and place was agreed. Sampling of the HCPs was continued until no new themes emerged.

5.2.3.2 Sampling of patients and doctors at the outpatient clinic

The receptionists' lists of the urology and the cardiology patients booked for the outpatient clinics within the study period were used to recruit participants. Since the list did not contain demographic factors such as age for all patients, a random sample was chosen instead of a purposive one. Each patient on the list was given a consecutive number and the numbers were written on separate cards. The cards were mixed with the numbers not visible at the back. It was anticipated that around one in three patients would agree to participate. Accordingly, the number of cards drawn were three times the number of patients to be recruited at one clinic appointment. Each patient corresponding to a drawn number was sent a letter. The letter explained the purpose of the study, and asked the patients if they would be interested in participating in the study (see Appendix 5.2.2). The letter included a consent form, which patients were asked to sign and to bring along with them to the clinic if they were interested. When they arrived at the clinic the researcher asked patients if they had received the letter and were interested in being interviewed. Patients who agreed to participate were asked which interview location they would prefer (e.g. pharmacy department,

outpatient clinic, or their own home) and a time and place was arranged for the interview. In allowing patients to choose the place of the interview it was more likely that they would feel comfortable and relaxed during the interview.

A purposive sample of doctors (varying according to their professional experience/grade) was asked if they were interested in talking to the researcher after the clinic or at another time convenient. It was planned to continue sampling until no new themes arose. However, since there were only a limited number of doctors, it was assumed that a maximum of 3 to 4 of them from each speciality would be recruited.

In both settings, participants who were unable to understand the researcher, to read the leaflet or in doubt of their competence were excluded. This was assessed by the patient's ability to communicate meaningfully with the researcher.

The sampling of patients and doctors in the outpatient clinics was continued until no new major themes arrived. This was expected to include in total 10 to 20 HCPs and patients.

5.2.4 Interview topic guides

The researcher designed topic guides according to the key themes identified from the literature and objectives of the study. The topic guides were sent to another researcher to ensure face validity. The final guides were used during the interviews as a reminder of the areas of interest (see Appendix 5.2.3 to Appendix 5.2.6). During the period of data collection some changes to the text were made, since it was felt that some of the original questions were unclear. In addition, matters which arose during the early interviews and which the researcher wanted to explore in more depth in the subsequent interviews were added to the topic guides.

Patients were asked about the current practices of information provision about medicines and how satisfied they were with these practices. The focus was on the general topics of information provided during the consultation and on the organisation of the process of information delivery as the basis of decision-making rather than to test patients' recall of information in detail. In addition, the views and preferences of

patients of their role in selecting a treatment and potential reasons for their views were explored. IC was used as an example of an applied model of decision-making where the patient makes a choice. Participants were asked about their opinion about the risks, benefits and feasibility of IC for medicines.

The topic guide for the interviews with HCPs recruited on the hospital wards focused on IC and its use for medicines. In contrast, IC was only one aspect of the interview with doctors working in outpatient clinics. Additionally, the topic guide for interviewing doctors working in outpatient clinics explored aspects of information provision and their perception of the doctor's and patients' role in selecting a treatment option in two settings – hospital wards and outpatient clinics. The interviews explored the participants' concept of decision-making about medicines and their understanding of it. All participants were asked some demographic details such as age for example (see Appendix 5.2.3 to 5.2.6).

To obtain some comparative quantitative data on the current health status of the outpatients, the Euro-QoL EQ-5D self-evaluation questionnaire was used (see Appendix 5.2.7). It has been shown to be reliable and valid in various patient groups and has been tested in three national surveys over the last decade (Kind *et al*, 1998). It asked participants to score their current health status on a scale from 0 to 100 where 100 is the best imaginable health status (Euro-QoL rating). In addition, it contained five questions on mobility, self-care, usual activity, pain and discomfort as well as on depression or anxiety (Euro-QoL description). One out of three levels (I have no problems = 1; I have some problem = 2; I am unable/ I am extremely...= 3) is chosen resulting in a five-digit number where 11111 is the best possible result and 33333 is the worst. The interviews were to be held at the clinic before and after the patient's appointment. Due to the limited time available to fill out an additional questionnaire only the Euro-QoL rating was to be obtained without the Euro-QoL description. However, as most patients were interviewed in their own homes or in the pharmacy department it was possible to use the Euro-QoL description in addition to the Euro-QoL rating. For inpatients, their reason for admission was used as a marker.

5.2.5 Data processing and analysis

The interviews were tape recorded if patients consented and transcribed verbatim (Appendix 4.3.1 gives a list of the transcribing conventions used). Additionally, the researcher made field notes during the interviews to document non-verbal communication of patients. After the interview the researcher added any observations, which was regarded as important for the analysis of the interviews. For instance, one patient told the researcher that she was very tired and did not know if she could answer all the questions. Therefore, the researcher focused only on some aspects of the study. This was described in the field notes, which were attached to the interview. In cases where the interview took place at the patient's home field notes were used to describe the surrounding of the patients.

The interviews were analysed by looking for the participants' view of decision-making in particular elements related to information provision, the doctor and patient preference of their role in decision-making and the reason for their preference. In addition, the interviews were searched for the participants' understanding of IC and all aspects related to the concept such as legal issues and recent changes in practice. The analysis focused on how the participants conceptualised decision-making and IC and how they attached meaning to their preferences and actions. The data analysis was influenced by the study of the theoretical background of IC and other recent models of decision-making, which have been described in Chapter 2. This means that some codes were already known to be relevant from the start of the analysis (for example the disclosure of side effects of the proposed treatment). Others were derived from the data such as the blame for refusal of a therapy.

The same strategy of data analysis as described in Section 4.2.5, which refers to the theoretical background of the method of analysis, was used. First, the transcripts were coded line-by-line. A code means a label given to a small text unit, either a word, part of a sentence or a paragraph. The labels were descriptive or interpretive. At a later stage, codes within the transcript of one interview or between interviews were compared and the codes were organised into categories and subcategories. As described in Section 4.2.5, the computer software program "Qualitative solutions and research: Non-numerical unstructured data – Indexing, Searching and Theorising"

(QSR NUD*IST, version 4.0) was used to support the organisation and retrieval of data.

As for the study of health policy decision-making, the transcripts of the interviews and subsequently the first stage of analysis was discussed with two researchers experienced in qualitative research and with a philosophical and natural science background. This allowed reflection of the codes and categories chosen and scrutinising each step of the analysis. In some places the coding frame could be refined or new aspects were discovered. Lincoln and Guba (1985) have argued that the review of the data analysis with the co-operation of other researchers with different backgrounds could support the researcher in considering aspects, which she or he had assumed, and therefore, enhance the comprehensiveness of the study. As described previously (Section 4.2.5), several methods were used to ensure the rigour of the qualitative research.

5.3 Results

In total 25 HCP interviews and 28 patient interviews were conducted in both settings (see Table 5.3.1). The samples from each setting will be described in more detail in the following parts.

Table 5.3.1: Overview of the sample

	Hospital ward setting	Outpatient clinic setting	Total
Health care professionals *	15	10	25
Patients	12	16	28

*Doctor and nurses were recruited in a hospital ward setting, only doctors were recruited in the outpatient clinic settings

5.3.1 Demographics of hospital inpatients

Of the 28 patients identified as potential participants 12 patients (aged 63 –73 years) agreed to participate (Table 5.3.2). The remaining patients did not want to take part for a variety of reasons, for example some were close to their discharge from the hospital, others were distressed, or they could not speak English (see Table 5.3.3). All participants described their ethnic background as white. All patients except three were surgical patients; most of them had a valve replacement or a bypass operation.

Table 5.3.2: Demographics of hospital inpatients

No*	Gender	Age	Type of intervention	Reason for admission +	Newly prescribed medicine ++
1	Male	64	Surgery	Valve replacement	Warfarin
2	Female	65	Medical	Investigations after myocardial infarction	Digoxin
3	Male	65	Surgery	Bypass operation	Amiodarone, Warfarin, Digoxin
4	Male	72	Surgery	Lung operation	Digoxin
5	Male	63	Surgery	Bypass operation	Amiodarone
6	Male	73	Medical	Arrhythmia	Digoxin
7	Male	72	Surgery	Bypass operation	Amiodarone
8	Female	67	Medical	Myocardial infarction	Digoxin
9	Female	66	Surgery	Bypass operation	Amiodarone
10	Male	67	Surgery	Valve replacement	Warfarin
11	Male	72	Surgery	Bypass operation	Warfarin
12	Male	67	Surgery	Valve replacement	Amiodarone

*No: Number, + information obtained from the patients, ++ Information obtained from the drug chart

Table 5.3.3: Details about non-participating hospital inpatients

Gender		New prescribed medicines			Unwell/ Distressed	Not interested	Language problems	Discharged before the interview
M*	F**	A†	D††	W†††				
10	6	5	3	2	6	1	6	3

Legend: *M: Male, ** F: Female, † A: Amiodarone, †† D: Digoxin, ††† W: Warfarin

Patients were interviewed on the ward because many of them were bed bound. The ward had six open bays with six beds each and four single bed side rooms. Although the study site was a mix-gender-ward, the bays were commonly single gender. Only one of the patients interviewed was in a side room. During two of the interviews a relative (spouse) was present.

The interviews lasted between 15 and 45 minutes with an average duration of 30 minutes. All of the participants agreed to be tape-recorded.

5.3.2 Demographics of the patients recruited in the outpatient clinics

Sixteen outpatients were interviewed. Seven were recruited from the urology clinic and nine from the cardiology clinic (Table 5.3.4). A total of thirty-two patients (66%) refused to take part; most of them were not interested in the research (Table 5.3.5). The participants were between 23 and 72 years old and the majority (75%) classified their ethnic background as white. There was a difference in the overall health status of the urology and the cardiology patients. The majority of cardiology outpatients scored their present health status as below 60 whereas most of the urology outpatients reported 60 or more (Euro-QoL rating).

Table 5.3.4: Demographics of the participating urology and cardiology outpatients

No*	Patient gender	Age	Ethnic background	Age when leaving full-time education	Euro-QoL D**	Euro-QoL R***	No* of drugs
U+1	M	58	White- British	21	-	60	>5
U2	M	70	Black – Caribbean	16	-	75	>5
U3	M	42	White- British	-	-	90	0
U4	M	23	White- British	18	-	95	0
U5	M	78	White- British	No formal education	-	60	>5
U6	F	39	White- British	21	11111	90	1
U7	M	72	White- British	14	11111	80	2
C++1	F	59	Middle-East	-	-	-	>5
C2	F	84	White-British	14	-	40	>5
C3	F	80	White-British	17	21122	40	3
C4	M	72	White-British	15	11121	80	0
C5	M	79	White-British	17	22221	45	>5
C6	M	62	Middle East	20	11222	40	1
C7	F	50	Black-mixed	14	22222	65	>5
C8	M	67	White-British	14	22222	40	>5
C9	M	63	White-British	14	11112	80	5

Legend: *Number, **Description, ***Rating, + U: urology, ++ C: cardiology

Table 5.3.5: Details about the non-participating outpatients

Clinic	Gender		Reason given for non-participation				
	M*	F**	Cancelled/ patient did not arrive	Patient did not receive letter/not interested	Language problems	Unknown	Other
Urology	9	5	4	6	1	3	-
Cardiology	10	8	5	8	-	4	1***

Legend: * male, ** female, *** one patient was mute

Eight outpatients preferred to be interviewed at home; five in a quiet room in the pharmacy department and three were interviewed at the outpatient clinic before their appointment. Only one interview was conducted in the presence of a third party (a relative of the patient). Since the outpatient interviews could be conducted in a quiet environment and the patients were commonly in a better health status compared to inpatients the interviews lasted longer than on the wards (between 45 to 90 minutes with an average of 60 minutes, except one interview which lasted around 30 minutes). Interviews within the home setting also allowed information gathering about patients in their usual surroundings, which sometimes enriched the understanding of the patients' verbal account.

5.3.3 HCPs recruited on hospital wards and in outpatient clinics

Out of 29 HCPs approached on the wards 13 (four SHOs, six registrars, one consultant and two nurses) did not take part; most of them gave lack of time as a reason. Nine doctors (four SHOs, four registrars and one consultant) and six nurses (Grade B to F) were interviewed (see Table 5.3.6). Out of 15 doctors approached in the outpatient clinics three consultants and seven registrars agreed to take part (see also Table 5.3.6). Similarly to the HCPs on the wards, all of the doctors not participating in the study gave lack of time as a reason. If possible the interviews with the HCPs were held in a quiet room on the wards or at the outpatient clinics. In four cases the interview took place at the nursing station on the ward where interruptions were more likely. All participants agreed to be tape-recorded. In one case the interview could not be tape-recorded due to technical difficulties. The interviews lasted between 30 to 60 minutes with an average of around 40 minutes.

Table 5.3.6: Demographics of HCPs

Interview No	HCP*	Gender	Grade	Current speciality
1	Nurse	Female	E	Cardiology
2	Nurse	Male	F	Cardiology
3	Nurse	Female	D	Acute admission
4	Nurse	Male	F	Haematology day unit
5	Nurse	Female	E	Cardiology
6	Nurse	Male	F	Coronary care unit
7	Doctor	Female	SHO^{+}	Cardiology
8	Doctor	Male	SHO+	Cardio-thoracic
9	Doctor	Male	SHO+	Cardiology
10	Doctor	Male	Registrar	Cardiology
11	Doctor	Male	Registrar	Cardiology ++
12	Doctor	Male	Registrar	Cardiology ++
13	Doctor	Male	Consultant	Cardiology ++
14	Doctor	Male	Registrar	Urology ++
15	Doctor	Male	Registrar	Urology ++
16	Doctor	Male	SHO+	Haematology
17	Doctor	Male	Registrar	Haematology
18	Doctor	Male	Registrar	Haematology
19	Doctor	Male	Consultant	Haematology
20	Doctor	Male	Registrar	Haematology
21	Doctor	Female	Consultant	Endocrinology ++
22	Doctor	Female	Registrar	Endocrinology ++
23	Doctor	Male	Consultant	Rheumatology ++
24	Doctor	Male	Registrar	Rheumatology ++
25	Doctor	Male	Registrar	Rheumatology ++

*HCP: health care professionals, ** No: number, $^{+}$ senior house officer, ++ HCP recruited in the outpatient clinic

5.3.4 Results of the interview analysis

The results of the interviews can be divided into three broad areas:

- the views of patients and doctors of the provision and the function of the information about the medication;
- the views of patients and doctors of their role in decision-making about medicines;
- the doctors', nurses' and patients' views of IC for medicines including its risks, benefits and feasibility.

Each of these areas will be discussed in turn. In relation to the first two areas, the term 'doctor' will be used instead of 'HCP' because these will focus on prescribing decisions in in- and outpatient hospital settings where, at the time of the study, only doctors prescribed medicines. The term 'doctors' includes surgeons as well as clinicians; the term 'patients' will include in- and outpatients. If only one of the patients' groups is meant this will be specified.

5.3.4.1 Information provision

Starting with the first aspect of information provision, the patients' views of information provision about their newly prescribed medicines will be analysed, especially information about side effects and treatment alternatives as well as the patient's ideal level of information provision. This will be contrasted with the doctors' perception of informing patients about their medicines. Beside the contents and the depth of the information provided the perceived role and function of the information and the perceived barriers are other important aspects of information provision.

5.3.4.2 Patients' perception of information provision about medicines

The results of the study showed that hospital inpatients received very little information about any aspect of the medicines including side effects. Although, compared to inpatients, outpatients commonly received more information about their medicines, both patient groups received practically no information about treatment alternatives. Whereas patients varied in their views of the need for information provision about costs, in- and outpatients raised issues which indicate that the role of information goes

beyond the provision of facts and serves the purpose of reassurance and trust in the medicine.

Information given to hospital inpatients

Most inpatients became aware of newly prescribed medicines due to the number and appearance of the tablets they received from the nursing staff after each drug round. Three patients reported that the doctor or the nurse told them that they were prescribed new medicines but without giving further information. Two other patients became aware of plans to prescribe them medicines by listening to the conversation between doctors on the ward rounds. The patients' report illustrates that although they both wanted to receive information about their treatment, especially their treatment plan, the doctors talked about the patients without directly addressing the patient. One inpatient explained:

> "They had a little conference around my bed this morning. They said... you know someone said 'Oh, he has been taking atenolol' you know. And the consultant said 'Oh, well, you know, we are already on two [medicines], we go on and have a third'." (Inpatient 3 after bypass operation, line 126-129) (Appendix 4.3.1 gives a list of the transcribing conventions used)

The hospital patients interviewed related the lack of information partially to the manner in which the medicines were given to the patient. The medicines were usually received in a little plastic cup and it was not possible for the inpatients to see the box with the label and the leaflet to get information about the medicines. The following explanation was typical:

> "Whatever they give me goes in there [the locker]. So they don't show me 'This is the new medicines' [...] 'Here is the leaflet'. I don't get this." (Inpatient 12 admitted with atrial fibrillation, line 262-264)

This may indicate the importance of visual information of the print on the box and the importance of handling the box along other medicine information. Another inpatient explained that he had difficulties remembering details about the medicines since there were two names for them, the brand name and drug name.

Although all except one inpatient were not aware of side effects, they emphasised that they wanted to know about their new medicines and the risks involved in the treatment.

> "But I like to have a good idea of what they are doing and what to expect from them" (Inpatient 2 admitted for investigations after myocardial infarction, line 104-105).

It was regarded as important that this was done in a language the patient could understand.

> "Well, if they took the bother explaining it and what needs to be done, maybe you'll be able to discuss with them, you know. If you said 'Take aspirin that will thinning your blood'. You know, simple talk and we sort out the route. But unless you are in the profession, you don't know what they are talking to you." (Inpatient 5 after bypass operation, line 164-169)

Not all hospital inpatients wanted to be informed about their medicines. Two patients saw no benefit in receiving information. As one explained:

> "Well, I wouldn't mind knowing about it. But it doesn't make me lose any sleep not knowing. And my main concern is getting out and being up again." (Inpatient 11 after bypass operation, line 108-110)

As the patient's quote shows, it is likely that the patients were mainly concerned about their recovery and their discharge.

Information given to outpatients

In comparison to inpatients outpatients were commonly better informed about the medicines they were prescribed. The majority of outpatients emphasised that the information about side effects was important for them, especially their severity, probability and impact on their lifestyle. One outpatient mentioned that, although he had information about the side effects, it was difficult for him to judge their severity.

> "They told me all the side effects to expect. They mention it and it all happened. But the extent to which it happened is a question of degree and that is very difficult to quantify. What constitutes a bad mouth ulcer from a normal mouth ulcer?" (Cardiology outpatient 6, line 241-245)

Some outpatients wanted to know about the probability of side effects and asked for information, which was relevant to their particular circumstances.

> "I would probably have said, And blurred vision, is that definite? *Or how likely is that?* Somebody I could just go to and say, what does this drug do. I am lucky because my friend is a nurse anyway so she usually checks things for me. But I found this a bit disconcerting and people say, well, a bit more detail about it." (Urology outpatient 6, line 111-115) [my emphasis]

As for inpatients one outpatient also did not want to know about side effects as she thought they could possibly frighten her.

> "I don't want to know really because if I do question it and I get to know what they're doing maybe I wouldn't want to take them." (Cardiology outpatient 7, line 373-375)

Information about treatment alternatives

Both inpatients and outpatients were not aware that there might be a treatment alternative to the medicine they were receiving. Characteristic was the answer of one inpatient, who the researcher asked if he knew about a treatment alternative:

> "I've never given it a thought. I never thought about that one." (Inpatient 10, valve replacement, line 184)

An exception was one inpatient, who believed that treatment alternatives would be available since she had experienced one of her medicines being substituted. However, she did not remember that it had been discussed with her in relation to her new medicines.

Some outpatients explained that the way in which the proposed treatment was presented to the patient did not include options.

> "I don't remember that my doctor ever said I would recommend that but there are these alternatives. He never put it in that light to me." (Cardiology outpatient 5, line 241-243)

> "Obviously, if she [doctor] says, these one could have terrible side-effects but this one wouldn't have such bad side effects but the terrible one could be better for your heart and this one might not be quite so good for your heart. They don't usually go into details like that, do they?" (Cardiology outpatient 3, line 289-293)

Many patients thought that treatment alternatives only included the choice between the generic and the branded product.

The majority of patients expected that the doctor would tell them if there were treatment alternatives available.

> "I would expect - if there were options - the doctor to say, well, there are three drugs I can prescribe you: A, B, and C. [...] Now what do you think?" (Cardiology outpatient 4, line 125-128)

If they did not receive the information about therapy options they assumed that there were none. Some patients took the initiative to ask the doctor but the majority of patients were not actively requesting information about treatment alternatives (including no treatment). Requesting information about treatment alternatives was regarded as especially important in cases where the patient did not tolerate the medicine prescribed in the first place. However, even in those cases many outpatients did not receive information about other choices.

> "The tablet and it might not agree with me because that happens as well so then I have to go back to her or him [the doctor] and say, well, this don't agree with me. What else is on the market? And explain to me what is the difference between the one you gave me and other tablets on the market that might be there. You see, but they don't do this. They don't do this. [...] If you go to a shop you have a choice of three different shaving creams. It's your choice, isn't it? But with the doctor it is not your choice, is it?" (Cardiology outpatient 9, line 236-244)

Information about costs of medicines

Outpatients were asked if the clinician in the clinic or the GP should mention the price of medicines suitable for treatment. Some outpatients were strongly against it, since they believed some patients would refuse expensive treatment even if it was paid by the NHS. Other outpatients were in favour of mentioning the cost of the therapy, which was thought to result in the patients' valuing the therapy and increase the adherence to the regime prescribed. Two patients believed that, since cost of the drugs played a role for the doctor in selecting a medicine, the knowledge of the cost of the medicine would improve the doctor-patient relationship in creating more honesty between the doctor and the patient. One of the outpatients thought:

> "I think it would be interesting to know if they are prescribing you a drug which would cost the health service a lot of money and that might be the reason why they are not prescribing it. It also might make you understand a bit more because sometimes I think, I am sure doctors have some sorts of... not negotiation but they must have dealings with some drug companies. And I think you shouldn't be treated like guinea pigs if you like." (Urology outpatient 6, line 207-215)

Most patients said that they hoped costs would not play a role in the choice of medicine although they believed that in fact, it did play a role. However, it was not clear to patients to what extent the cost of the medicines was determining the doctor's choice of the medicine. Some outpatients reported their experience where cost was

decisive for the termination of a treatment course. One patient referred to his experience with one of the doctors in the outpatient clinic when he explained:

> "Another one told me I should stop taking a medication. And I said, why? And he said because it will save your GP £30 a month." (Urology outpatient 5, line 74-75)

One patient believed that it was not necessary to make a choice according to the cost of the medicine, since savings could be made in other areas of prescribing.

Role of the information provided

Patients reported various reasons why they regarded information about their medicines as important. The most frequently cited reason was that it would allow the patient to monitor side effects and report them to the doctor. Additionally, patients thought it would decrease their anxiety.

> "They were like kind of special things you can't buy them in the shops. I must say I did want to know more about them even though I knew that there was nothing, which could go wrong. Just of the peace of mind I would have liked to have more information, you know." (Urology outpatient 4, line 101-105)

This patient used the analogy with shops and commodities, which other patients also referred to. Another function of information was reassurance for the patients in the potential benefit of the medicine and its effectiveness.

> "It will give you confidence because positive thinking I think is a lot of making the medicine work well for you." (Urology outpatient 1, line 451-453)

Most importantly, information was not used to evaluate options or do trade-offs. The information was primarily used for the daily management of medication.

All patients emphasised that they were highly dependent on the doctor to provide individualised information about the medicine and the alternatives, since they were regarded as the most important and trustworthy information source.

> "I think we rely quite a lot on the doctor to inform us." (Urology outpatient 3, line 139)

> "You sort of rely on them and look up to them and because of their superior knowledge and everything." (Urology patient 7, line 360-362)

Doctors were seen to be in control of the information. Some patients thought that they potentially retained some aspects of the medical information. However, similar to the influence of cost on decision-making, it was less clear to what extent this happened.

5.3.4.3 Doctors' perceptions of the information provision about medicines

The results from the patient interviews showed patients had only minimal information about side effects of their medicines and about treatment alternatives, especially in the case of hospital inpatients. Doctors confirmed that they often did not inform patients as much about their medicines as they would like. In this section, first the doctors' views about communication with inpatients will be described before going on to analyse the views about communication with outpatients. Time was perceived as the most important barrier to information provision.

Information provision to inpatients

Regarding communication with inpatients, doctors admitted that it was reduced to a minimum or not happening at all.

> "The day-to-day running is usually very quick: 'We are doing fine, don't worry, we sort your constipation out, ok, next one.'" (Urology registrar, interview 15, line 361-363)

> "We just say – look, we are going to start some painkillers, you'll be ok." (Rheumatology registrar, interview 25, line 600-601)

> "I must admit unless they specifically ask me I must admit that the inpatients I probably don't give them nearly as much information as I give the outpatients." (Cardiology registrar, interview 11, line 345-348)

One reason why the doctors chose this style of communication was partly due to the belief that the disease affected the inpatient's understanding.

> "Well, obviously, if they are confused and they are sick and they are ill. [...] If you've broken your leg, if you have done this and you have done that and you are sick and you've got pneumonia, you are just not necessarily particularly rational." (Endocrinology consultant, interview 21, line 396-397)

Another was the organisation of the consultation process in hospital and the belief that compared to outpatients, inpatients accepted receiving less information or did not want to receive information. For example, one registrar explained:

> "The inpatient tends to look for less information and it's only the minority of inpatients, who would look for, I would say, any information about their tablets because they don't give a Goddamn what you use as long as you treat them. Because they are in hospital for that reason, that they are sick enough to need aggressive therapy." (Rheumatology registrars, interview 25, line 519-525)

As seen from the results of the patient interviews, although the majority of patients did expect to receive less information most patients wanted much more information than was currently provided (see also Section 5.3.4.2).

Some doctors concluded that hospital inpatients needed to have trust in the HCPs, since they have less influence on their life compared to a patient in primary care. It was only if the patient insisted on information that the doctor did explain the medication. Compared to outpatients inpatients commonly did not receive written information about their medication during their hospital stay.

> "I can't remember when I gave an inpatient an information sheet last." (Rheumatology registrar, interview 15, line 573-574)

Information provision to outpatients

Overall, the doctors believed that the provision of information for outpatients was much better than for inpatients. This was due to the fact that more time was dedicated to the delivery of information and that more detailed information was provided to outpatients. The amount of information provided about each aspect of the medicine was decided according to the time available and what the HCPs assumed the outpatient wanted to know. One doctor saw it as his role to decide what information is necessary to disclose.

> "It is one's skills to enjoying that balance and about one's skills in determining what the patients need to know. [...] They [the doctors] should be able to assess the patient and *get some feel for* to what extent the patient wishes to have those discussion lengthened or shortened." (Cardiology consultant, interview 13, line 60-62, 114-116) [my emphasis]

The type of information provided can be divided into six main areas: the name of the medicine; the dose of the medicine; instruction how to take the medicine; the

indication of the medicine; the most common side effects; and the treatment alternatives. In the following section, the focus will be on the last three as most doctors talked about them in more depth during the interview.

Information provision to outpatients about treatment indication and side effects

In addition to technical information about the drug name, the dose and the frequency of administration, all doctors emphasised that they wanted to explain to outpatients the indication of the medicine and the need for it.

> "I do try to get an idea whether they understand why they are on each of them. And then once I've made the decision *why I want* to change a dose, change one drug to another drug, the only important information I think is *why I am doing* it and always try to make it quite clear." (Cardiology registrar, interview 11, line 13-17) [my emphasis]

The doctors emphasised the importance of explaining their reasoning for the choice of a medicine or a dose change. The knowledge of the outpatient about the treatment rationale was in the foreground of the doctors' concerns rather than explaining how the medicine actually works.

The information about side effects and what was judged as important information varied between each doctor.

> "Some [doctors] actually who know the numbers – I don't very often – would mention the percentage of risks." (Urology registrar, interview 15, line 406-407)

In addition, the amount of detail disclosed about side effects depended on their severity, their probability and the duration of treatment. Compared to short-term treatment, long-term treatment was seen as more significant and explained in more depth.

> "And then actually you would sit down. If it's a long-term decision and not just an antibiotic for five days." (Urology registrar, interview 14, line 487-488)

> "But I would imagine that we are reasonably good at discussing some of the more straightforward, or some of the more common, is the word, side effects, or potential side effects associated with the medications. Because we are dealing with people with chronic diseases, rheumatoid and lupus, who are going to be on the medication for twenty years, you know..." (Rheumatology registrar, interview 25, line 24-31)

In addition, the time available and the setting were influencing factors on the amount of detail about side effects provided. The expression "you'll want to be practical" was characteristic for many doctors indicating that certain compromises needed to be made between the amount of the information provided and other constraints such as time. It also depended on what was regarded as common practice:

> "But I don't think anybody would tell the patient that there might be some oto- and nephrotoxicity to this drug [gentamicin] here when we give it to you. I don't think anybody does it. I don't know, you need to draw a line at some point because you'll want to be practical as well otherwise you never give the treatment." (Urology registrar, interview 14, line 329-334)

The comment above also illustrates that it is the doctor's belief that it is their duty to treat rather to help the patient to their goal. Potentially serious side effects were not necessarily discussed with hospital inpatients as it was argued that the patient was monitored during the administration of the treatment.

Overall, many doctors mentioned that it is important to be positive about the treatment in order to give reassurance to the patient compared to showing no emotions or being neutral. This was regarded as part of the duty of a HCP.

> "So I'm usually enthusiastic about it, you know, positive about it. Which again, I think, is important. For instance, we found that if you just say - this is the drug blah blah blah - and give them the information leaflet, they come back and they say - well look, on your information leaflet there's half a page of bad things. No way am I considering this. Which is a real shame, because there's at least half a page with almost every drug, isn't there?" (Rheumatology registrar, interview 24, line 81-88)

<u>Information provision to outpatients about treatment alternatives</u>

Regarding treatment alternatives one clinician explained that it is important to inform the outpatient about different kinds of interventions such as surgery versus treatment with medicines or chemotherapy versus radiotherapy. However, many doctors did not discuss options between various medicines, for examples drugs of the same chemical class or used to treat the same clinical condition. Some doctors explained that they usually mentioned that there are other options within one treatment management but without further explanation about advantages and disadvantages.

> "And I do say, if you get problems with this drug, don't worry, we've got alternatives." (Rheumatology registrar, interview 24, line 253-254)

The doctors believed that they should only mention treatment alternatives which are within the professionally agreed range of therapy for the condition diagnosed. This will be discussed in more detail in section 5.3.4.5.

One view was that it was unnecessary to explain different options of medicines within one type of treatment management, for instance different types of antihypertensive drugs since the outpatient would not "benefit" from it. One doctor talked about the information given to inpatients.

> "The patient wouldn't really benefit from knowing that there are others. [...] Then you talk with the patient about the treatment or what he or she is coming in for. And this drug will be part of the whole treatment management basically. I wouldn't specifically discuss the drug." (Urology registrar, interview 14, line 267-270)

Similarly another doctor thought:

> "*But when it's just drugs*... For instance there are lots of different antibiotics you can give to patients when they get an infection. We don't ask their opinion about which one they think would be best for them. We decide that." (Urology registrar, interview 15, line 223-226) [my emphasis]

None of the doctors discussed decision aids as a possible way to support the patient in weighing benefits and risks of the treatments proposed. In some specialities patient information leaflets were in use, which commonly explained one medicine and its side effects instead of comparing the advantages and disadvantages of various medicines with each other.

> "I think the vast majority I usually say - I think you should go on drug X like methotrexate. I then give them a leaflet on that drug. I don't give them leaflets on six disease modifying agents." (Rheumatology registrar, interview 24, line 416-420)

<u>Information provision to outpatient about costs of medicines</u>

Doctors admitted that cost considerations clearly determined the selection of the medicine.

> "I have this for prostatism where we are dishing out large volumes of drugs on a regular basis. I think we probably give the patients less choice because we are.... We have to be ruled by the cost factor and making sure that we have got a departmental policy that is working for everyone and that's not going to break the budget." (Urology registrar, interview 15, line 93-98)

Many doctors did not know the exact cost of the medicine. However, there were departmental agreements which determined the medicines which should be prescribed first line based on cost considerations.

Although cost clearly influenced the decision, the provision of information about costs was an exception to the type of information doctors provided patients. Some even regarded telling the outpatient that the decision was made on the basis of costs as destroying the patient-doctor relationship.

> "If you turn them into a cheque book medical problem they'll think you are crazy. They loose complete faith in you. And that's the end of it. You've lost, you've destroyed your relationship with that patient and you can't treat them. They may as well go to somebody else because, whatever you say in future they are going to think - you know, this guy is treating his managers, not me." (Rheumatology, interview 25, line 230-237)

This is in contrast to the belief of some patients who said that they would like to receive information about costs as mentioned earlier (see Section 5.3.4.2).

Some doctors claimed that the withholding of information about costs could be justified on the grounds that the doctor would choose the most effective medicine.

> "The things I don't talk to them are costs. I don't think there is any point letting patients know whether the drug is expensive or cheap or whether we are trying to save money. I am not sure that's necessary as long as we have chosen the best one that works clinically." (Urology registrar, interview 15, line 44-47)

That the withholding of information and lack of communication could be justified on the basis that the most effective medicine was chosen by the HCPs was a repeating theme in the interviews. On the one hand, doctors saw their role in tailoring the information to the individual patient to support their understanding of it. On the other hand, with tailoring of information the doctors already made a selection about the preferred treatment option or narrowed the potential options available.

Information contributed by the outpatients

The doctors reported situations where outpatients brought information, which they had gathered, into the consultation. All the doctors interviewed reported that this information did not result in a change of the prescribing decision. Most of the

information introduced by the patients was either judged as scientifically wrong or of limited scientific rigor and relevance. Characteristic was the comment of one registrar:

> "I can think of a few instances where we had to discuss in more details why I am prescribing another tablet because of information they have read. But I can't think of a particular time where I actually either changed or added in a further tablet because of something someone has shown me." (Cardiology registrar, interview 11, line 135-139)

Barriers to information provision

All doctors devolved part of the responsibility to inform the patient to other HCPs. They felt that, whereas nurses and pharmacists should inform the patients about the medication in hospital, GPs should advise the patients when they are discharged. However, doctors based in secondary care realised that these other professional groups had difficulties in fulfilling this role. The statement of a registrar indicates that beside the time pressure the lack of knowledge was a possible reason for the absence of information provision:

> "When I'm in clinic as well, I'm looking up the side effects in the BNF myself, you see. So I don't know them very well myself. Especially for rare medications". (Haematology registrar, interview17, line 433-346)

In both settings, hospital wards and outpatient clinics, time was most commonly given as the main reason for the lack of information provision. The time needed to inform the patient was described as "luxury". Characteristic was the comment of one clinician:

> "But I think in terms of patients telling what's going on I think sometimes with inpatients you don't always have the *luxury*." (Cardiology registrar, interview 12, line 249-251) [my emphasis]

Another physician explained his situation:

> "In terms of drugs prescribing it's harder for the clinicians to do it [informing] because it seems difficult to have the time to go through it. Although in theory I think it is important, I think in practice doctors rarely do it unless they have the time and it is important or they have another issue". (Cardiology SHO, interview 9, line 58-62)

All doctors reported making trade-offs between the time available for the consultation and the information they regarded as necessary. In this hierarchy of importance the explanation of the indication of the medicine together with potential dose or dose changes and information about the common side effects ranked highest.

One doctor explained that the time available to explain the prescription to the patient sometimes even resulted in a compromise between the most effective treatment choice and an option that is easy to explain.

> "And sometimes we even compromise on the best tablet for that because we have very little time here to explain a tablet that needs repeat visits, dose escalation, that they need to take five times a day, that, you know, is complicated. So we tend to stick to simple things because we know that one, *we have only one minute to explain the drug to them* and two, we know anything which is going to affect drug compliance, we try and restrict to a minimum." (Urology registrar, interview 15, line 57-65) [my emphasis]

Regarding information provision about invasive procedure there was the overall tendency to spend more time on information provision about it compared to treatment with medicines, despite the fact that some doctors acknowledged that medicines could have side effects of similar severity to invasive procedures.

5.3.4.4 The patients' view on their role in the decision-making process

In the following two sections the patients' and the doctors' perception of their role in the decision-making process and the reasons for their beliefs will be analysed. The results of the patient interviews will be presented first.

The patients' definition of involvement

Patients' understanding of the meaning of "involvement" varied. Whereas some outpatients thought involvement means being informed about the treatment alternatives and the doctor's rationale for the choice, most in- and outpatients defined "involvement" as actively expressing a preference for one of the options presented. One outpatient explained:

> "Well, to have the options of different medicines which have the same effect but they were different as opposed of being offered just one and having the chance to take it." (Urology outpatient 4, line 282-284)

Asked about their views of patient's involvement in decision-making about choices of medicines, all hospital inpatients and the large majority of outpatients wanted the doctor to select their medicines. Exceptions were some outpatients who preferred to be offered a choice between treatment options. However, the doctor should guide the patient.

> "The doctor should be able to guide you towards what he thinks that is the right choice which given, if he has got enough experience, probably is the right choice." (Urology outpatient 5, line 260-263)

The selection should be in accordance with the doctors' choice. Characteristic was the comment of one outpatient:

> "If it would be too extreme I would feel uncomfortable about it if you like overruling what the doctor's advice would be." (Cardiology outpatient 4, line 228-230)

The patient continued explaining that he believes that patients have a meaningful role in the selection of a medicine by expressing their preferences. However, he was the only patient who talked about the useful contribution patients can make.

> "And especially when there are genuine options that the patient has a function in choosing, being able to say, well, the way I live or the way I do this or what ever else, I mean if I did get such and such side effect that would really be

harmful. This sounds the best one to me because I can cope with this." (Cardiology outpatient 4, line 296-300)

Permission versus involvement and the importance of decisions

Patients made a distinction between making a selection between various treatment options (apart from the option of no intervention) and giving permission to a treatment proposed by the doctor. Giving permission to treatment was not defined as "involvement" in the decision. The more important the decision the more patients preferred to be consulted about the selection made. Exploring which decisions patients regarded as important, surgery was most frequently cited by hospital inpatients. This was in contrast to outpatients who mentioned decisions about long-term medication as well as medicines which would influence their life-style. Outpatients explained that they wanted to know for instance more details about medicines which would make it difficult to drive, because of the side-effects profile.

"I think I would like to know the choice, you know. I wouldn't want to get dizzy attacks when I'm driving." (Cardiology outpatient 5, line 276-277)

It was important for them to explore treatment alternatives, to enable the medicines to tie in with their working life. In these cases the priorities of the patients e.g. maintaining the driving license, were in conflict with other priorities such as treatment with the most effective medicine offered.

Reasons for non-participation

Patients mentioned several reasons why they thought that the doctor should make the selection. These were closely related to the patient's beliefs as to how medical decisions were made or should be made. The most frequently used argument was that patients felt they had no expert knowledge. Another was their wish to defer the decision and with it the responsibility to the doctor. Important barriers described by patients were their feeling of dependence and vulnerability due to their illness, the desire to trust the doctor and the hierarchy in the relationship as well as the circumstances of the patient-doctor encounter such as the lack of time. Many arguments against involvement were related to the belief of the patients, that there is

only one "right" choice. Each of these viewpoints will now be described in more detail.

Lack of knowledge and the ability to make a decision

The phrase "The doctor knows best" was frequently used. The belief that leaving the selection in the hands of an "expert" or being guided was a way to minimise the risk of side effects.

> "I think I would prefer to rely on the doctor to choose for me really than me risking things." (Urology outpatient 4, line 27-29)

> "But if it's a serious condition and you are being treated I rather go to somebody who is dealing with this condition all the day and knows exactly and is seeing 200 patients with the same problem as me. Safety in numbers." (Cardiology outpatient 6, line 474-477)

Some patients used the expression "a little knowledge is a dangerous thing" which illustrates that patients felt they would be exposed to risks of adverse outcomes if they were involved in selecting a medicine. Most patients thought that increased information provision and education would not necessarily enable them to make a selection. One group of patients believed that they are not able to understand the technical language used by the HCPs. Another group of patients thought that although they were able to understand the information provided they felt unable to make the "right" choice. Therefore, they should not make a choice at all or be involved in making a selection.

> "And me, I feel that a lot of doctors consult too much because then the patient makes the *wrong* decision even though he is an informed patient he does make the *wrong* decision because a little knowledge is a dangerous thing."(Cardiology outpatient 6, line 398-401) [my emphasis]

It is interesting to note that patients used the terminology of making the "right" or the "wrong" choice. In asking patients to explain what they mean by "right" choice they stated that making the "right" choice requires more than the information commonly provided by the doctor. They thought that the "right" choice could only be made by a person who received formal training and has experience.

> "Really I feel as far as medicines concerned that patient would have to be a trained pharmacist to make the right decisions, wouldn't they?" (Cardiology outpatient 5, line 460-463)

For many patients it was difficult to imagine that there might be different treatment options with similar effectiveness or differing medical opinion about the appropriate treatment. Different medical opinions confused many patients.

> "And I found that I have seen two doctors now, two different doctors, GPs and you find that one is doing the opposite thing. One downgraded my tablet and one upgraded my tablets. You see they don't know nothing about you personally." (Cardiology outpatient 9, line 55-58)

This indicates the patients' belief that beside the knowledge about the properties of the medicines the physicians should know the patient individually in order to treat the patient successfully. Some patients believed that the fear and the emotional involvement in undergoing specific treatment would hinder them from making the "right" choice and only the doctor would have the necessary rationale to make a choice. This would mean that certain paternalism would be required (or allowed). This was illustrated by a patient explaining that he regretted a choice he made in the past about colostomy where in retrospect his priorities changed. He wished the surgeon with "an overview" (i.e. with medical training and years of experience) had made the decision for him.

The feeling of dependence

Some mentioned the special situation of being ill and not being able to discuss options because of their condition as another important reason why they felt not able to become involved in the decision-making. The degree of feeling of depending varied from "you don't feel like arguing", through "being in the hands of the doctors" to being a "guinea pig". All of these expressions were used by in- and outpatients. For instance, one inpatient said:

> "And of course, being in a teaching hospital, you know... I was sort of a guinea pig" (Inpatient 6 admitted for treatment of arrhythmia, line 25-27).

Also most inpatients expected that in a situation of illness they had no responsibility to make decisions. They expected that the decisions to be made for them as long as they were in hospital and occasionally if they were outpatients.

> "I come in here I am sort of... I am in hospital. I think 'Well, they know what they are doing. Hopefully, they know what they are doing, you know. [...] They should know what they are doing." (Inpatient 6 admitted for investigation of arrhythmia, line 209-211)

The worries of the patients about their health or their illness made them feel "vulnerable" and seeing doctors "in a quite powerful position", which made the patient more likely to agree to the doctor's recommendation in the hope of improvement in their condition.

Trust and the hierarchical structure of the patient-doctor relationship

The trust in the doctor and the hierarchy were two other important reasons why patients felt less inclined to become involved in the selection of medicines.
One outpatient said that his past experience of effective treatment of his prostate condition had resulted in absolute trust in the skills of the clinicians. He saw no need to or benefit in becoming involved.

> "I take what he says. If it is a bit better for me to take I just digests whatever he says and go along with him because I am so satisfied with the person out there at the hospital that whatever he says I would go along with it." (Urology outpatient 7, line 75-78)

Another inpatient characterised the relationship with the doctors on the ward as strongly hierarchical:

> "They were not talking at your level. They were talking down to you." (Inpatient 5 after bypass operation, line 112-113)

Lack of time as a barrier

The lack of time available in the consultation was a frequently cited theme in all interviews. In- and outpatients talked about the time available for the consultation. Many patients perceived the lack of time as a barrier to raising questions.

> "You are always very aware of the time pressure and the other people who are waiting." (Urology outpatient 5, line 85-86)

Some outpatients said that in order to contribute to the discussion they would often need more time to consider the information given by the doctor. Only in the follow-up appointment would the patient able to respond to the information. That there was limited time to consider a choice was seen as a means of allowing doctors to force the patient to choose a specific option. For instance, one patient talked about his experience of being asked to agree to surgery when the doctor had just finished an

invasive examination. As a result, he felt stressed and pressured to agree to the proposed surgery.

Beliefs about medical decision-making

As highlighted before, most patients felt that the doctor should make the selection about the treatment for various reasons. In order to explore the reasons in more detail patients were asked how they believed doctors select the medicine for patients. In general there was a lack of transparency as to how doctors exactly made their decision:

> "If the doctor makes the right choices and whatever processes that they use which is not for us to know, they don't want to tell us because then they can't take the money. They kind of have to justify themselves." (Urology outpatient 1, line 596-602)

Regarding the ideal of decision-making some outpatients thought that the doctor's decision should be made on a very individual basis and should take personal aspects into account.

> "That is where the GP has the time and not too many patients, so that you can become accustomed to the person and they see them socially as well and form opinions on what kind of treatment would be beneficial for them. [...] But I imagine the doctor if he is an observing person can take in things about people and if he doesn't view medicine purely in a mechanistic way but in something that more approaches the whole body and mind." (Urology outpatient 5, line, 10-13, 406-409)

Outpatients who believed that the doctor made the "wrong" decision frequently added that it was the first time that they saw this particular doctor. It seemed that some patients had less trust in the skills of the doctor to make the right decisions for them if the doctor had not seen the patient before. This indicates that they expected the doctor to obtain some information from the patient. The optimum was a clinician with experience and personal knowledge of the patient.

> "And this is why I was very strict to go and see Professor [name]. [...] So Professor [name] took over and I feel very confident with him and I insisted I would see him. And I feel more confident that he is getting to know me a bit more personally which my new GPs don't seem to have that." (Cardiology outpatient 9, line 60-65)

This leads to the next theme of the patients' desire to appoint a decision-maker who makes choices for the patient.

Choice of the decision-maker

As a result of believing that an expert needs to make the decision, outpatients regarded the choice of doctor as very important. With this choice the patients appointed a decision-maker, who would choose the appropriate treatment for them.

> "It is very important to get the right GP. [...] You have got to be very lucky on the day you go to the hospital to get the right guy." (Urology outpatient 5, line 61-62, 102-103)

> "So if you don't get the doctor who makes the right decision it could be fatal for you at a later stage." (Cardiology outpatient 9, line 41-43)

However, patients were not always free to choose their doctor. Inpatients especially had very little influence on the choice of the doctor except where they were private patients. Although outpatients had less influence on the doctor, who would see them in the outpatient clinic, most patients were able to make a choice about their GP. Patients who were not satisfied with their GP told the researcher that they had tried to change the surgery. In addition to skills and professional experience, gender and the communication style of the doctor were also important for the patient when choosing a doctor. Two male patients preferred to have a male GP; two female outpatients preferred to be seen by a female doctor. One of them explained:

> "My GP, it is an all-female practice. So you did find them a little bit more sympathetic to the women." (Cardiology outpatient 3, line 125-127)

Patient's role - Feedback about side effects

If patients perceived that they had no role in the selection of the treatment itself, it was important to investigate what role they would like in the decision-making process. Instead of selecting a treatment, many patients believed that their role was to provide feedback to the doctor about potential side effects of the treatment. Having influence on the selection of medicines was only possible or justified if the patient had previously experienced the effect of the medicines prescribed. Hence, personal and practical knowledge was given considerable weight.

> "The only thing, I can do is as I said to take the medication and hope for the best. And if there are any side effects, well then of course I would go back and they would have to try to change the medicine." (Cardiology outpatient 3, line 269-272)

> "But I do question if after having taken it. Either if I have side effects or if I think it is doing me no good or if it's doing me any good I would definitely... But I will not query it when he first prescribes it." (Cardiology outpatient 6, line 185-188)

In reporting side effects and effectiveness of the medicine the patient becomes a partner of the doctor. Interestingly, since in the hospital context the task of monitoring side effects and effectiveness is done by HCPs, the patient often does not have the opportunity to be regarded as a partner of the doctor. This also highlights the fact that prescribing is a process rather than a one-off event, where the treatment is changed according to its effects. Patients feared that the prescribing decision was viewed as a one-off decision and not reviewed. As one outpatient explained:

> "I do feel quite angry that I was on antidepressants for about four or five years. And no one ever really questioned that. I would still be on them and they would still prescribe them. No one ever really said, let's just check that it is right. [...] They just assume that they got it right. They have analysed it, they have sorted it. So it is up to you really to take the tablets." (Urology outpatient 6, line 194-200, 173- 175)

In particular, outpatients explained that they became worried if they thought there was no chance to give feedback to the doctor initiating the treatment due to the problems of accessing the outpatient clinic or the GP. In addition, outpatients reported the insufficient knowledge of the GP about the decisions made by doctors in the outpatient clinic.

Non-conformity with the patient's role

The majority of outpatients told the researcher about situations in which they acted differently from that which they believed was expected from them, namely agreeing to the doctor's preferred treatment choice. These narratives of patients revealed interesting aspects about the circumstances in which patients felt they had to take a different course of action, and the factors which contributed to that decision. Interestingly, although they decided against the doctor's proposed treatment they did not use the word "refusal" to describe their action.

For instance, an urology outpatient who had broken his cheekbone signed a consent form for a proposed surgery despite his dislike of undergoing an invasive procedure.

However, after a period of time of consideration of the decision at home, he wanted to withdraw from the operation and talked to the surgeon. He was most surprised to find out that the surgeon told him that there was no problem if he decided against surgery though he might have a dimple in his face at the place where the bones would grow together. The patient was of the view that it was not worthwhile undergoing surgery in order to avoid the dimple. He felt he had not been appropriately informed about what would happen if he refused surgery.

> "If I had only been told that there is no need to have the operation it will knit on its own. That was all what I wanted to know." (Urology patient 7, line 567-569)

> "It wasn't even discussed or anything. It was just taken for granted. The only thing I was asked was would next Thursday be all right to go in at three o'clock." (Urology outpatient 7, line 376-379)

In another case an outpatient decided against taking antidepressant therapy since she regarded the risks of the drug higher than the benefit. In this case the patient reported she did not know about treatment alternatives other than watchful waiting. In both cases, there was a discrepancy between the judgement of what is regarded as necessary care between the doctor and the patient.

In summary, patients believed that the doctor as the "expert" should make the selection of the medicine due to his experience and knowledge including personal knowledge about the patient. Patients saw their role in giving feedback about the effect of the medicine, although they feared that they did not always have the opportunity to do so. The lack of knowledge, the feeling of dependence in a situation of illness, not wanting to carry responsibility for a decision and the consequences such as the feeling of regret were some of the arguments put forward to justify the delegation of the decision to the doctor, who would make the "right" choice. The impact of the subsequent feeling of the patient when looking back at the decision was most overt in the account of one patient who regretted having made the wrong decision regarding colostomy where he wished an experienced surgeon had decided for him. However, most patients expressed their desire to be asked to give their permission to treatment, which was differentiated from 'involvement' in the actual selection of treatment. In the cases where patients decided against the doctor's proposed treatment they felt ill informed. Patients were

not aware of the different underlying values placed on the process or outcome. The next section will describe the views of the doctors on decision-making.

5.3.4.5 The decision-making process from the doctors' point of view

The doctors' views of their role in decision-making

For most prescribing decisions the doctors saw themselves in the role of making the selection of medicines.

> "So I would choose the drug that I believe is most evidence-based." (Cardiology registrar, interview 12, line 31-32)

> "I must admit in my own practice I don't do discuss this [the decision] with them and I tend to make the decision myself." (Cardiology registrar, interview 11, line 25-26)

That the doctor had the control over the selection of medicines is also evident from the use of personal pronouns such as "I", "me" and "my" instead of "they" for patients or "we" or "us".

Doctors reported that the decisions about selecting medicines were based on evidence which was a combination of their own experience and their own interpretation of the result of published trials and the departmental arrangements, guidelines and policies. Since there was a clear preference in the doctor's opinion, they said that "true choices" in the sense of a variety of treatment options rarely existed.

> "Well, usually if they are several options of drug treatment there would be in the department usually a priority of those options. And this is usually based on the quality of experience or obviously evidence from the literature with that drug. Very often....let's say an anticholinergic you would start with the most favourable one and if that doesn't work for example you'll just go to the next one down and so on." (Urology registrar, interview 14, line 34-40)

The doctors regarded clinical effectiveness as the most important criterion to select a specific medicine. Commonly the most effective rather than the safest option was preferred. One rheumatology registrar explained:

> "You know, *we are not going completely soft, we still push what we want to use*. Because if the patients had their way, I suppose in an uninformed way they'd go for the safest option. Where we know the safest option isn't going to be as effective as the strongest option. *Strong - potential side effects and safe -*

> *no side effects if you like.* But we've seen enough chronically damaged joints, etc, etc to know that there is a difference. Again, you have to teach them that it's not like a payback in six months' time. It's a pay-back over five or ten years." (Rheumatology registrar, interview 25, line 278-289)

This is in contrast to the perception of many patients who preferred "not to risk anything" or the "softest" option with the least side effects (see Section 5.3.4.4). As the account of the doctors shows, their experience of using medicines may result in choosing a riskier, not necessarily a safer option. For the large majority of prescribing decisions the patient's preference was not used to make a selection of the medicine in the first place.

Exceptional circumstances in which the patients were asked to make a selection between different options were cases in which there were two or more equally effective treatment options or "equally acceptable" options from the point of the view of the physician. A consultant in rheumatology explained in what kind of situations he would give the choice about different treatment regimes to the patient:

> "But at the end of the day it may be that there is a choice of two or three choices, which would be equally acceptable in terms of the physician's opinion. And if those issues can be explained with clarity to the patient then I think the patient is at liberty to make the decision. I think that's good medicine. *But it's not very often these circumstances arise.*" (Rheumatology consultant, interview 21, line 222-228) [my emphasis]

A haematology consultant referred to some chemotherapy regimes where the "choice is not as clear-cut and you have to sort of let them know which drugs are available out there". Another doctor referred to the choice between short-acting and long-acting insulin where the patient would be offered a choice between different medicines of the same chemical class. Hormonal replacement therapy (HRT) was used as another example where the patient was given choices:

> "And it's only *when we are uncertain ourselves* that we really give them much more of a choice. We often ask them to consider whether they want it or not themselves. Things like HRT we would discuss it with them and say let us know next time, if you want it." (Endocrinology consultant, interview 21, line 39-42) [my emphasis]

Antibiotics, analgesics and antihypertensives including diuretics were mentioned as groups of drugs where choices were less commonly offered. The results suggested that in some specialities such as cardiology there were only a few situations where the

doctors regarded two or more medicines as equally effective compared to other medical specialities such as endocrinology or rheumatology. This may also reflect the certainty of an effect of the medicines commonly used in cardiology compared to medicines prescribed in endocrinology and rheumatology.

The circumstances in which the patients were informed about the treatment options and asked about their preference was due to the doctors' judgement about the potential implications of the decision. One doctor explained that for him life-threatening diseases and treatment options associated with potentially irreversible and life-threatening complications were decisions for which he took time to disclose treatment alternatives to patients:

> "It's about operations or is about complications, explaining them to patients, it's about, you know, how long they are going to live, how long they are going to be in hospital. Those things we make time during the day to go and do as an extra session." (Urology registrar, interview 15, line 373-377)

As the quote illustrates these were the type of circumstances where the treatment options were associated with complications of higher probability compared to issues arising from many other commonly used medicines.

The doctors' views of the patients' role in the decision-making

Instead of actively selecting a treatment option, most doctors defined "patient involvement" in the majority of prescribing decisions as the provision of information about the indication of the treatment and dose changes made by the doctor.

> "Researcher: Interestingly, you said, 'I want to get them involved as early as possible'. How can you achieve that?
> Clinician: I think when you start the drug you tell them. When you prescribe a new medication you'll let them know why they are going on the drug and why the drug is being changed, why the drug has been increased or decreased, so that they know enough right from the beginning why they are on it."
> (Cardiology registrar, interview 12, line 208-212)

The provision of information was expected to facilitate patient involvement and collaboration in two ways. One was providing feedback about the effects of the treatment later during the course of treatment. Patients who know about the potential adverse drug events report faster and more accurately about the effects of the treatment. As a result clinicians were able to individually tailor treatment more rapidly

until the desired effect was achieved. The other aspect of patient collaboration was compliance. Almost all doctors believed that the presupposition for compliance by the patient was information about the treatment indication.

> "When you are giving a lot of tablets for preventative medicine, just for the compliance they need to be sort of involved in the sort of decision-making if they are going to take it." (Endocrinology consultant, interview 21, line 124-127)

> "I think communication is vital, isn't it? We know that without effective communication compliance is poor. And it's not rocket science; it's understandable that if someone doesn't understand what condition they've got they can't understand why they need treatment. So it starts with a clear explanation of what I think is wrong with them." (Rheumatology registar, interview 24, line 30-35)

Good communication amounted to telling the patient the rationale and indication of their treatment rather then an exchange of beliefs or negotiation as suggested by various decision-making models. Compliance to treatment was so important that a certain degree of compromise in the process of decision-making and selection was accepted by physicians where they believed that it would result in a higher rate of compliance and therefore the desired outcome.

> "That is something I have no strong preference over and I am quite happy to sort of... you know if that improves compliance certainly." (Cardiology registrar, interview 12, line 38-40)

> "If there is something I could do that would increase their compliance than I think I would support this entirely." (Cardiology registrar, interview 11, line 393-395)

The importance of achieving compliance is evident by the comment of one doctor that he would even "bend the truth" to ensure that the patient will receive the drug prescribed.

> "And occasionally, rarely, you'll bend the truth a bit to get a patient to come up for injections rather than taking tablets. You can't really say to them - look, we don't trust you. You know? You'll say something bland like - it's our best way of knowing that you are getting the full dose. Or something like that. And that is paternalistic and that is uninformed, but I suppose we make the decisions and it's all the one to us, how they get the methotrexate, we don't care really." (Rheumatology registrar, interview 25, line 650 659)

The degree of compromise depended on the doctor's opinion about which of the treatment options available would provide what they regarded as the desired clinical outcome.

Trust in the skills of the doctor was thought to increase patient adherence to their medicines.

> "They will be quite happy to take your advice if they trust you. And all you have to say is – I believe this is in your best interest." (Endocrinology consultant, interview 21, line 164-166)

The doctors' perception of the relationship with the patient

Some doctors talked about the patient-doctor relationship as a "partnership" or the patient as the "partner" of the doctor or HCP. However, the language of the doctors indicates that the patient was very dependent on the doctor or at least not equal: "that you do something appropriate to them" instead of "for them". Most doctors used expressions such as "convince the patient", "persuade" as well as "win them over" instead of a negotiation between equal partners.

> "And I think in a lot of patients that you need to spend time to explain clearly what you are doing because in those patients if you don't, you'll loose that chance *to convince them* that you are doing something appropriate to them. You loose that rapport at that stage. You never get them back. And from then on they will query every single prescription or every single change you make." (Cardiology registrar, interview 12, line 235-239) [my emphasis]

There seemed to be a fine balance between persuasion and coercion. Later the doctor said: "Then I would insist on them having it." Only one doctor used the expression of "selling" a treatment option to the patients indicating that patients were to be regarded as consumers:

> "So yeah, you try and sell the one that you want to use." (Rheumatology registrar, interview 25, line 277-278)

Overall the communication between the clinician and the patient about many prescribing decisions was described as "one way" traffic, which may indicate that the patient was not necessarily an equal partner in the interaction with the doctor.

> "They don't query what you do. You tell them and they usually say, yes, whether they understand it or not." (Cardiology registrar, interview 12, line 230-231)

Barriers to patients' involvement in decision-making

The analysis of the interviews shows that from the doctors' viewpoint there were several barriers to patient participation. As described previously, lack of time was the most frequently mentioned reason. In some specialities such as rheumatology or endocrinology it was possible to arrange a second consultation and review the decision or initiate the treatment after the patient had time to reflect on the treatment plan proposed. However, this was only possible if the initiation of the treatment was not urgent.

Beside the lack of time to explore patient preferences there were also other barriers, for instance the individual differences between patients in their desire to obtain advice and guidance. One urology registrar explained:

> "They [the patients] are very fed up that they weren't given any choices. They find out about another drug that they could have had. And they are very angry. Other times you scare someone into a panic and they don't know what to do. *So you never get it right.*" (Urology registrar, interview 15, line 558-561) [my emphasis]

The feeling of never being able to get it right was characteristic for many doctors and they expressed their frustration about this. However, they thought that it is their task "to have a feel for to what extent the patient wishes to have those discussions lengthened or shortened". Doctors commonly did not explore patients' beliefs about medicines or their preferences for the decision-making process. If patients did not come back with complaints about their medicines or told the doctor that they had stopped their medicines it was assumed that most of them were happy to take them.

> "And I think I accept that I am going to get a certain number of patients who are going to come back to me, having stopped their medication, thinking there is an interaction, in fact there isn't. And I can inform them when they come back. It's not too many, fortunately in my experience. If I have more then maybe I would change my practice but actually, as things are, I don't have too many that come back. And I can deal with those one, two when they do." (Cardiology registrar, interview 11, line 432-440)

This indicates that the judgement of the doctor was largely built on assumptions.

Another barrier to involving patients in decisions was the difference between the priorities of HCPs' and those of the patients. The following comment illustrates how difficult a doctor thought it would be to find an agreement between differing priorities.

> "I think, you know, we look at it from a different angle. One of the common examples I always use is that patients complain about hair loss on statins. I would say, well, with that degree of heart disease my priority in this clinic obviously is to make sure that your heart stays healthy and statins have proven beyond doubt to be cardioprotective in that sense. Then I would insist on them having it. But I must admit I am not always successful." (Cardiology registrar, interview 12, line 85-93)

Only in case of equal clinical effectiveness within the professional range of authorised choices was the patient's priority given preference. Otherwise doctors thought they could not find an agreement between the patient preference and the physicians' priorities. Overall, overtly exploring the patient preferences was regarded as "luxury" and unnecessary in the many prescribing decisions. Only for major interventions such as surgery or chemotherapy and in some cases where options of similar effectiveness but different mechanism of action existed would the patient be asked about their preferences.

5.3.4.6 Comparison between the views of hospital in- and outpatients views as well as between the views of doctors and patients

The result of the interviews allowed comparison between the views of hospital in- and outpatients as well as the physicians' view with that of the patients.

The difference between the views of in- and outpatients shows the context related influence on decision-making (see Table 5.3.7). Hospital inpatients especially lacked information about their medicines, which was related to the way in which patients were consulted and medicines were administered. Inpatients were most concerned about the surgery they had undergone and put less attention to their medication. The fact that inpatients in particular were unable to choose a physician of their choice, as outpatients were able to do in some instances, and their reluctance in refusing the medicines, indicates their dependence. Outpatients highlighted the need to be informed about the effects of the medicines on their life-style, which was not something mentioned by inpatients.

Table 5.3.7: Comparison between the views of hospital in- and outpatients

Issue	Hospital Inpatients' view	Hospital Outpatients' view
Information provision about medicines to patients	Absence of information about side effects and treatment alternatives	Lack of information about side effects for example probability; absence of information about treatment alternatives
Method of information provision	Number and appearance of tablets was an indicator that the inpatient is receiving new medicines. Two inpatients became aware of new medicines through listening to conversation between physicians.	Method of information provision varies between individual doctors. Some patients chose their doctor according to his or her communication style including information provision.
Patients' desire for information	Many inpatients wanted to know about their medicines, except two patients, who did not see a benefit in being informed about the medicines.	Information about side effects was seen as important. One outpatient did not want to know about side effects because they could frighten her.
Information brought in by the patient	Inpatients did not report about information brought with them.	Outpatients reported bringing of information to the consultation.
Patients who wanted to be involved in decision-making	3 out of 12 inpatients wanted to be involved in decision-making.	6 out of 15 outpatients wanted to be involved in decision-making.
Decision-making authority about treatment alternatives	Patients made a distinction between making a selection and giving permission for treatment. The more important the decision, the more patients preferred to be asked for their permission. Surgery was regarded as the most important treatment.	Decisions about medicines influencing life-style (e.g. driving a car, working) and long-term medication were regarded as important where more detailed information should be provided and patient's view should be discussed.
Choice of decision-maker and interaction between patients and doctors	Inpatients did not talk about choosing a doctor.	Choice of a doctor was very important. However, patients experienced several barriers to exercising their choice of a doctor.
Patient's role – Feedback about side effects	Feedback about the effect of the medicines regarded as very important.	As for inpatients. Additionally, patients experienced difficulties in giving feedback.
Non-conforming with the patient's role	Inpatients felt gratitude towards their HCPs, being in hospital made them feel pressured to comply with treatment proposed. Both contributed the fact that inpatients did not necessarily feel free to refuse treatment.	Some patients did refuse the treatment proposed or prescribed, but they did not use the expression "refusal". They felt ill informed about the treatment alternatives and differed in their assessment of the need for treatment from that of the physician.

Doctors and patients had common views about several aspects regarding decision-making:

- Lack of time was regarded as the main obstacle for communication.
- According to the belief of the majority of patients and doctors, the doctor was seen as the main decision-maker regarding the selection of treatment out of a range of options.
- Reassurance of the patient was seen as significant in the treatment process.

- Patient's feedback about the effect of the medicines was thought to be of importance.

Despite their agreement regarding these aspects of decision-making there were some significant differences in their views and assumptions regarding decision-making (see Table 5.3.8).

Table 5.3.8: Patients' and doctors' assumptions and contrasting views regarding decision-making

Issue	Common assumptions or views held by patients	Common assumptions or views held by doctors
Risk	Decision made to minimise risk.	Decision made to maximise efficacy.
Responsibility	The doctor had the main responsibility for the treatment.	The main responsibility was with the doctor in choosing the medicines, but once the decision was made the doctor handed over the responsibility to the patient
Doctor-patient relationship	Hierarchy and paternalism were predominant in the relationship. Certain degree of paternalism might be necessary/ beneficial.	'Partnership' with the patient.
Treatment alternatives	Treatment alternatives would be offered if they exist.	Treatment alternatives were only discussed if they were equally effective or "acceptable".
Information exchange	Doctor would disclose or ask for information if necessary to make the decision in the best interests of the patient.	The patient would request information if they wanted to obtain information. If they did not ask they would not expect or want much information.
Effect of the information about side effects on the patient	The majority of patients thought that the information about side effects would prepare them to manage their medication.	Detailed information about side effects was thought to increase patients' anxiety.
Definition of 'involvement'	Expressing a preference for a treatment.	Information provision about the indication of the medicine.
Refusal of therapy	Patients were very reluctant to refuse therapy especially hospital inpatients.	If patients do not want to take the treatment he/she would freely refuse it.
Basis for the decision	Individuality of the patient was regarded as important to be taken into account.	Individuality in terms of preferences was usually not elicited in common prescribing decisions.
Costs	Uncertainty about the extent to which costs played a role in decision-making.	Costs clearly played a role in the choice of the treatment, either direct or indirect.

5.3.5 The patients' and the HCPs' views on informed consent (IC) for medicines

The next part describes the findings relating to the patients' and HCPs' views on IC. First, the patients' perception and their understanding of the concept is analysed. In particular two elements of IC will be addressed: the information disclosure and the permission to provide treatment. In the second part the HCPs' account of IC including their view of the feasibility and desirability of IC for medicines will be analysed. Comparing the patients' viewpoints about IC with that of the professionals shows that the latter were more comprehensive. This was expected as the HCPs were more familiar with the concept and many of them obtained consent in their daily practice.

5.3.5.1 Patients' views on IC for medicines

Most patients associated IC with obtaining it in a written form compared to verbal or implied consent. For some IC only meant the signature, the formal procedure of giving their permission with no other function.

> "I suppose it proves that a certain procedure has been followed, which to some extent is a good idea but for what sake. If it's an end in itself there is no point." (Urology patient 3, line 247-250)

The signature was viewed purely as a protection for the HCPs and organisations and meaningless to the patient. One patient thought that IC might also protect the HCPs from legal claims even if the harm was caused by the professionals' misconduct. Therefore, signing a consent form would only be appropriate in the case of life-saving surgery when this was the only way to receive treatment from the doctor.

Asked about their views on IC for medicine in more detail in- and outpatients made a distinction between the disclosure of information and the act of giving permission. Many patients regarded disclosure of information as the most relevant aspect of IC; less importance was given to the element of giving permission to provide treatment, which was rarely mentioned. Both the patients' view on information disclosure and their view on giving permission are discussed below.

Information disclosure

The results showed that many of the functions of information disclosure during the consent process overlap with the general role of information provision in the consultation, which have been described earlier such as enabling the patient to manage their medication and increasing the patient's confidence in the medicine. Although none of the patients used the term "right to be informed", they said that it is their wish to be informed which some attributed to human nature and a rational approach to medical care.

> "You are a human being, you want to be consulted about it. It is after all about you. I mean you are the one who has to take the medicines, go through that. [...] Yes, you don't want to just sort of saying you have such and such problem, take this and that's it." (Urology outpatient 3, line 75-77)

The expressions "to be put in the picture" or "to be consulted" were used to describe the act of obtaining IC. Importantly, these expressions indicate a variation in the degree of patient input. Whereas "to be put in the picture" can mean to be told that a certain intervention will be performed, "to be consulted" includes also a need for opinion or advice.

However, the information provided during the disclosure was usually not used to influence the decision of the doctor. The physician was still in the role of the decision-maker despite the information provision.

> "They have got the medical knowledge, you haven't. You have to go by what they tell you." (Inpatient 7 after bypass surgery, line 220-221)

Three patients were an exception. They mentioned that the information provided would enable them to decide about the health care received. One of them said:

> "Well, you know what's going on and you are taking control or partially about your own body, your own life. [...] Then I can have more control, more say within the workings, the operation that will be done." (Cardiology inpatient 14, line 352-354, 390-395)

Many patients mentioned difficulties in obtaining information, which were similar to the barriers faced in being involved in decision-making, which have been described before (see Section 5.3.4.4). This included that the patient did not know what information was absent or that they felt that the information disclosed would not be meaningful to them. Others had the feeling questions were not appreciated due to the

time pressures faced by the HCPs and doubted that IC would offer the opportunity for discussion. An inpatient avoided asking the doctors on the wards.

> "But do they have the time? The thing is that it [informed consent] is so time consuming and they haven't got it. I know I mean for myself at the hospital that they are so pushed. And you don't try to sort of keep them because you know that there are a lot of patients waiting. I mean it would be nice if someone could sit down with you and explaining it all to you, you know." (Cardiology inpatient 11 after bypass operation, line 158-163)

Again, the lack of time for the consultation resulted in the hesitation of some patients in raising questions and so increasing the burden on the HCPs. Hence, many patients – especially outpatients – believed that IC could not guarantee that the patients will receive the information required. They thought that many would sign the consent form without seeking the necessary information and exercising the right to information from the HCPs.

> "You would sign anyway. You don't usually say, well, go away read and come back in an hour's time after I have read the small print and I consulted my solicitor. They don't do that do they?" (Cardiology outpatient 3, line 309-311)

The feeling of exposure and being powerless is contrary to the purpose of IC. Instead of reporting being in control of the decision, patients stated that the medical and legal terminology would hinder patients understanding the implications of signing the consent, which was viewed as a contract with legal implications.

Permission or refusal of therapy

Compared to information disclosure 'giving permission' or the 'refusal' of therapy as another component of IC was rarely mentioned. One reason might be that most patients assumed that they would always give their permission. Some could not imagine a case in which they would refuse a treatment, first, because of the patient's gratitude for being treated and second, because patients assumed that the doctor's selection of treatment would be almost always right due to their superior knowledge or position.

> "Who am I to sort of say, well, no, I'm not very happy about that unless it is something really terrible. Generally, for a member of the public it would be a bit silly to go against the doctor in a way." (Urology outpatient 7, line 363-365)

> "So really I am just grateful for the advice and whatever the doctor prescribes I have to accept of course." (Cardiology outpatient 5, line 208-209)

Another reason for not considering refusal was the general attitude of patients in accepting the proposed treatment.

> "I'm one of those people – as I explained to you – if the doctor prescribed something I believe that it is necessary and you should take it. Period. There's no discussion." (Cardiology outpatient 6, line 320-322)

In addition, the patients' belief of having no choice regarding their treatment was related to the perception that it was impossible to refuse treatment in a situation where the patients' health was under acute threat. Subsequently, most inpatients undergoing either a valve replacement or a bypass operation did not regard the operation as their decision made about treatment. As mentioned before, they felt no ownership for the decision.

> Researcher: "Did you feel you had a choice with the operation?"
> Patient: "No, no choice. 'It is so bad' they said. 'No choice'." (Interview 7 after bypass operation, line 226-227)

Refusal of therapy was also seen as associated with several risks.

> "Yes, I am afraid. But it maybe if I am not taking it I expose myself for something dangerous. I don't know what will happen. But it may be that there are side effects but it may save me." (Cardiology outpatient 1, line 206-209)

An exception was two patients who thought that giving explicit permission would create some kind of ownership of the decision, which was regarded as beneficial for the patient.

> "I think it would be very good, very good indeed because then I couldn't blame the doctor and say, you told me. It's my job and I have been chosen to take it." (Urology patient 7, line 307-310)

Explicitly asking the patient to give permission was regarded as a sign of respect for the individual and as an act of politeness. However, it was seen as something unusual and it was doubted that it could work in practice.

> "Obviously you would feel involved in your own illness and it's flattering too to be asked, isn't it? So instead of just been treated like a number." (Cardiology outpatient 3, line 353-355)

The act of asking permission was seen as a "psychological" benefit.

In general, most inpatients and outpatients regarded the provision of information during the consent process as the main advantage of IC, even though it was not a guarantee of its provision. The use of IC was only regarded as necessary in cases where patients could consider that it was likely that they or others would want to refuse therapy. Most commonly, chemotherapy was mentioned, as a group of drugs where they believed written IC would be beneficial from the patient's perspective. In addition, medicines associated with appearance changes, causing addiction or medicines used in pregnancy were also mentioned.

5.3.5.2 The HCPs' views on IC for medicines

The result of the interviews with the HCPs can be divided into six main aspects:

- Meaning of IC
- Current practice of the use of IC for medicines
- Benefits and risks of IC
- Barriers to IC
- Feasibility of IC in practice

Each of the aspects are described in more detail below:

Meaning of IC

As in the case of patients, HCPs made an implicit distinction between the disclosure of information and the obtaining of the patient's permission to the treatment proposed, with their focus being on the former. For them IC meant that the patient "knows" or will "understand" the benefits and the risks disclosed. A nurse defined IC as:

> "The patient fully understands what they are about going into and that they have their eyes open. They know the benefits and they know the risks." (Nurse grade F, interview 2, line22-24)

Another physician said:

> "As long as the patient understands why they are taking it - as much as they can -, I think that is the important thing. I just want them to understand why they are taking it. And if they don't, then to ask me" (Cardiology SHO, interview 9, line 277-281)

Despite the fact that HCPs emphasised the understanding as a core issue of consent, in general HCPs were more concerned about the details of the information disclosed rather than assessing the patient's understanding of that information. Some saw IC as a way to ensure accuracy and completeness of information. For example one nurse said:

> "I think quite often bits can be sort of left out and just kind of wash over a little bit. I don't think it is true IC." (Nurse grade F, interview 6 line 7-9).

Regarding the completeness of information one doctor believed that IC allowed him to talk about long-term side effects, which would otherwise not be mentioned:

> "But in the era of written IC it makes it easier to say - and because we are getting consent I do need to tell you some of the long-term effects of these medicines. People seem to accept that." (Haematology special registrar, interview 20, line 351-360)

Some of the HCPs did not agree with the view that IC would enhance the completeness of the information. Rather, they thought certain information would frighten the patient and risk their "unreasonable" refusal. This is discussed in more detail below.

The view of some HCPs who believed that good communication including patient information leaflets and clinical letters to GPs could replace IC confirms the fact that information disclosure was seen as the core issue of IC.

> "So good communication is the key to a good outcome, I feel. And that is a form of IC." (Rheumatology consultant, interview 23, line 328-332)

> "And at the moment our IC is that we write in our letters that we have explained certain side effects that can be expected or can occur." (Urology registrar, interview 20, line 481-484)

Three doctors talked about IC as a way of enabling the patient to make an informed refusal of treatment. One of them said:

> "IC is if the patient having all the information required before they can actually agree to a procedure or to any sort of decision-making. That is having the power to say 'no' in a situation of knowledge. Being in a position of information and knowledge. That's what's IC is." (Cardiology SHO, interview 7, line 6-12)

Some clinicians viewed IC as purely a legal requirement, "a signature on a piece of paper" or a "formality". Many HCPs warned that depending on the clinical situation

and the attitude of the HCPs the meaning of consent was reduced to the act of signing the consent form.

Interestingly, another doctor commented that IC would not only legally protect the doctor but would also free him or her from feeling guilty if the patient suffers from an adverse outcome.

> "I think that it is the right of the doctor as well to be protected from the burden of having to feel the guilt on his conscience if something goes wrong because that some sort of eliminated. If the patient takes the decision to go ahead with the procedure in the light of all factors considered that it means that the doctor is then sort of some absolved in a way, you know." (Cardiology SHO, interview 7, line 44-51)

The large majority of HCPs felt that by obtaining IC the patient had the final responsibility for the decision. Hence, IC was not seen as risk sharing. The patient needed to accept the consequences of the decision. It is interesting to note that most of the doctors stressed that although it is the doctors' responsibility to explain the risk, it is the patient who accepts the risk of an adverse outcome.

> "The complication rate is quoted to the patient and he then agrees knowing that to have a procedure and he then suffers that complication ...the doctor is out of fault there. He has explained the risks and the benefits. The patient understood and then he has been unlucky. So there isn't... there is no one at fault or blame." (Cardiology SHO, interview 8, line 42-48)

The only risk the doctor would take is the risk of a legal claim by the patient in the future.

> "And if you don't inform them, the patient could sue you for not informing them. So it is a kind of risk you take as well." (Haematology consultant, interview 19, line 76-78)

One nurse and two doctors referred to the double meaning of IC. One meaning was the right of the patient to know about the treatment, the other would be to fulfil the legal requirement and protect the doctor and the health care organisation. One of them pointed out the discrepancy between the ideal and clinical reality.

> "Well, I suppose, truly IC doesn't really exist. You have approximations towards it." (Haematology SHO, interview 16, line 77-78)

The discrepancy between the ideal and the reality was a recurrent theme in the HCP interviews.

Current practice and use of IC

In current practice the permission of patients to therapy was often assumed rather than specifically obtained. A doctor talked about prescribing a medicine for an inpatient on the ward:

> "We tell them we are going to start antibiotics but we don't really do any more in terms of explaining...there's a chance you could get a rash or whatever. [...] We don't usually say – is it all right?" (Haematology registrar, interview 20, line 24-30, 48-49)

Regarding administration of medicines one nurse explained the usual procedure before administration, for example, of an injection.

> "Well, I probably do say 'Is that right putting a needle in? Is that alright?' But it doesn't seem very official and very.... Often not recorded. It's always accepted, taken for granted". (Nurse grade F, interview 4, line 116-119)

Another nurse described the practice of giving information about oral medication to inpatients. He questioned the need of IC for medicines.

> "We don't necessarily go into all of the side effects possible. If they ask, then we tell them. So they are usually given information. Whether it is always IC ...It is usually 'This is what it does. Here, take it!' So it is informed... They are informed. But their consent is not necessarily given. But then patients do refuse anyway. They have the right to refuse." (Nurse grade F, interview 2, line 190-196)

This nurse was satisfied with the current practice which is in contrast to some doctors who thought that the present practice of informing hospital inpatients was clearly unsatisfactory. One of them explained:

> "Patients get given a little pot with their tablets in. They have no idea what's in there. They get very confused about what medication they are on. They are pumping drugs into their body. They feel very much like that they have being treated almost against their wishes without any consent." (Urology registrar, interview 15, line 277-281)

The presence of the patient on the ward or in the outpatient clinic was in itself a sign of their permission to be treated. Hence, the patient was not explicitly asked for their permission.

> "I never really came across anybody refusing a treatment when they come in. I mean they come in because they want to be made better." (Urology registrar, interview 14, line 277-279)

The assumption of the patient's permission to the proposed treatment was enforced by the fact that only very rarely did patients refuse the proposed therapy.

> "To be honest I can't imagine that anyone would say no." (Cardiology registrar, interview 10)

Two clinicians were more cautious in making the assumption that the presence of the patient on the ward or in the outpatient clinic was a sign of their IC. They highlighted the fact that the fear of being disadvantaged might be a reason for the low number of patients refusing treatment.

> "But I think in those sort of situations it may well be useful, if only because it gives the patient the chance to say no, they don't want to take it. Whereas they might feel pressurised into taking it otherwise." (Endocrinology registrar, interview 22, line 370-374)

Some doctors felt that patients usually were not given much choice regarding treatment alternatives. Many hospital inpatients had an acute life-threatening condition and having no treatment was not perceived as an option. They wondered how meaningful consent would be in this situation. Especially in a situation where the doctor felt that there is only the choice between having a certain treatment and having no treatment, they thought that only little information needed to be given to the patient.

> "Do you need to give a patient more information if it's [the drug] more necessary? You probably have to give the patient less information the more necessary, essential the drug is." (Haematology SHO, interview 16, line 158-165)

The doctor continued using warfarin as an example:

> "I think it's bad practice to tell people - oh just take these pills, they are warfarin. Because they won't take the pills then. You have to tell them what it's for. But I don't necessarily think you should tell people with relatively low risk of cerebral haemorrhage that it's an adverse effect. But you might do in certain circumstances. If someone had a stroke before, many years ago, then you might say there's a risk of it." (Haematology SHO, interview 16, line 425-432)

Again, this clearly illustrates that it was regarded as important to inform the patient about the indication of the medicine in order to achieve adherence rather than to disclose side effects to enable the patient to make an informed decision.

In addition, many HCPs did not regard IC as a way of supporting the patient's decision-making between two or more treatment options. Treatment alternatives were only briefly mentioned or not at all.

> "It's usually explaining what the consequences of the choice are, rather than giving the choice." (Haematology registrar, interview 20, line 525-526)

> "But from my experience I have not really been asked that much about alternatives. You know, while taking the consent. I mean, normally the patient's either willing to go ahead with the procedure or not." (Haematology registrar, interview 17, line 301-305)

As mentioned previously (see Section 5.3.4.3), the absence of information about other treatment options was justified on the ground that the doctor would suggest the "best option", the most effective or the "gold" standard. A nurse pointed out that formally asking the patient for permission to treat did not mean that patients are taking part in the selection of treatment options:

> "I don't think he necessarily is involved in the decision-making process because usually the decisions are made beforehand. The decision is made and then they go to the patient with the decision and say 'Right, this are the tablets we want you to take. This is what they do'." (Nurse grade F, interview 2, line 279-283)

The HCPs explained that this was partly related to the process of obtaining IC. It was common to obtain IC shortly before the intervention when the decision about choice of treatment had already been made.

Most HCPs saw IC in the context of invasive procedures and associated consent with signing a form rather than giving verbal or implied consent. An exception was one doctor who saw the use of IC in all aspects of health care.

> "I mean I think IC is not only for the big procedures. It's for everything. In the moment you say 'Hello' to the patient you can seeking consent. [...] Every single interaction with a human being should be based on consent." (Cardiology SHO, interview 7, line 97-100, 102)

Whether to obtain written IC or not was related to the relative significance of the side effects and the experience and knowledge of the clinician in using the treatment in clinical practice.

> "But I have to say in my own mind prescribing of the day-to-day medication that I prescribe I don't see it as being at a level of risk such that it is actually an

> ethical dilemma in my own mind. I don't see that I'm doing actually something unethical by not inform... obtaining IC." (Cardiology registrar, interview 11, line 480-482)

As seen so far, IC was mainly seen as a tool to provide information about risk and benefit about the proposed treatment. In common prescribing situations or administration of medicines permission was assumed rather than explicitly obtained. The ethical aspect of IC in supporting informed decision-making and self-determination was an ideal not implemented in practice. As the next section highlights, legal aspects of IC are dominating clinical practice.

Benefits and risks of IC

Most HCPs saw the legal aspect of IC as the driving force for its introduction and as the main benefit for HCPs.

> "Whether that's done verbally or in a written way, it's not benefiting the patient at the end of the day, it's just covering ourselves medically, legally." (Haematology registrar, interview 20, line 230-233)

Although the fulfilment of the legal requirement to obtain IC was a protection of the HCPs against legal claims, it was not seen as a meaningful tool to protect the patients' right to give an informed permission. Patients would sign the form even they had not been adequately advised about making a choice about their care or treatment. One doctor warned that patients would be the "victims".

By obtaining written IC doctors felt that the relationship with their patients would change towards a more formalised and contractual relationship, which the majority saw as a disadvantage.

> "I think it puts something between you and the patient. It becomes less of an understood relationship, if you like. It becomes more formalised. A less relaxed relationship." (Rheumatology registrar, interview 25, line 407-410)

Overall, there was doubt whether obtaining IC in the legal sense would benefit patients, though it was an essential part of HCPs protection against legal claims.

Most of the benefits of IC mentioned by HCPs resulted from information disclosure. Written IC, in particular, would force the doctor to explain more thoroughly the benefits, risks and alternatives.

> "The patient actually gets to discuss very clearly about the drugs. In order to get IC you will have to spend time and going through the whole issue". (Cardiology registrar, interview 12, line 142-144)

Interestingly, some doctors changed their view on benefits and risks of IC during the interview. Whereas at first most of them explained that there is no benefit for patients in using IC, after some reflection they highlighted some positive issues (e.g. detailed explanation of the treatment proposed), which were worthwhile implementing in practice.

The perceived benefit of IC overlapped with the general functions of the provision of information about medicines discussed earlier. For instance, more detailed information would enable the patient to better manage their medicines as well as identify side effects to report back to the HCPs and potentially avoid hospital admission. Many HCPs believed that patients would be better informed about what they could expect from treatment as a result of IC. More realistic information would also increase the patients' willingness to give permission to the treatment.

> "But when you actually sit down and say face to face 'I want you to understand, this is what it's for, this is how it is going to effect you, this is why you have to take it, this is how you have to take it' I think they will be a lot more understanding, willing to except, that they make up the terms with things a bit quicker." (Nurse grade E, interview 1, line303-308)

Others believed that patients would be more satisfied with the care they received if they were better informed about their medicines. Both increased satisfaction and being more realistic were thought to prevent future legal claims.

> "It makes people feel the better, and you don't have people complain that – I didn't know that was going to happen." (Haematology SHO, interview 16, line 30-32)

Many HCPs believed that more information as well as more responsibility would result in improved adherence.

> "I think patients who understand what they are taking and why are more likely to stick to it." (Cardiology SHO, interview 8, line 412-413)

> "You know, we give the patient very detailed information and the main reason is compliance. " (Haematology registrar, interview 17, line 533-535)

Interestingly, the benefit of IC related to detailed information provision shows that IC was seen in a very functionalistic way. It was thought to improve patient's outcome or reduce legal claims.

Some HCPs regarded the increased knowledge of patients about their medicines as an advantage for themselves as well since patients would be able to give a more accurate medical history. Only some HCPs talked about the ethical aspect of the patient's right to know about side effects.

> "They have a right to know, I think, if they are taking medication. They have a right to know if there are any side effects." (Haematology registrar, interview 17, line 266-268)

There were, less frequently mentioned, benefits of IC which arose out of the act of giving permission to treat or making a decision about the treatment received. For example one of these advantages was that the patient became more responsible for the treatment decision made. As two doctors explained:

> "I think patients need to take responsibility for themselves and if people, patients expect autonomy in their care and pressure in the working staff are greater then I think the patients have to take more responsibility on their shoulders." (Cardiology senior house officer, interview 9, line 188-191)

> "And I think it would make patients think about it more as well, rather than – whatever you say, doctor. I think there would be people who would ask more questions." (Endocrinology registrar, interview 21, line 433-437)

Nurses differed from doctors in their opinion about the implications of the information provided to the patient. The majority of nurses stressed the negative effects of the information such as distress and an increase of the patient's anxiety. Hence, more patients would refuse medication, which was regarded as essential to treat the condition. Two nurses concluded that IC would be more harmful than beneficial. One of them explained:

> "One sort of patient will not want information and if they are giving that information the more information they get perhaps the more anxious and concerned they will be about it. And the more they are giving the more they are unable to make a decision." (Nurse grade F, interview 4, line 200-203)

Doctors mentioned this as one of the reasons for framing the information given to patients in such as way as to favour the option preferred.

> "Well yes, because sometimes what happens is we tend to tell them things which apparently help a particular thing that we want to do and we project the other alternatives in a bad light. So thereby making the patients being biased in favour of the decisions that we are going to make. That's what happens." (Haematology registrar, interview 20, line 193-198)

Whereas some doctors argued that in case where the information about potential side effects was thought to cause harm to patients, it was legitimate to disclose only certain information, other doctors argued that it is their duty to inform the patient fully to enable them to manage their medication.

> "I think we should let them know that things can go wrong, because otherwise, both for our safety and their knowledge we should let them know that these things can go wrong." (Haematology registrar, interview 20, line 58-61)

Many thought that IC would have positive or negative effects depending on the patient's character. Some patients would be more anxious knowing all the side effects about the treatment proposed and potentially refuse treatment.

Overall, the benefit of IC resulted from the provision of information and less from the act of autonomous and informed choice. Whereas nurses highlighted the negative effect of the information in fearing that it would cause patient's increase anxiety and lead to refusal of therapy, doctors focused more on the benefits in increased patients' satisfaction and reduction of legal claims.

Barriers to IC

Presentation of the information in a language which the patient could not easily understand was frequently mentioned as a barrier. The medical terminology needed to be translated into language the patient was familiar with and translators were also needed for foreign patients.

> "I worked in a 13-bed coronary care unit and it wasn't unusual to have no English speaking patients what so ever." (Nurse grade F, interview 6, line 78-81)

In addition, there were foreign doctors who were not easily understood by patients.

Knowledge of the HCPs who were in charge of obtaining IC was another barrier. One doctor explained that it was often the case that the most junior member of the team needed to ask the patient to give consent whereas the most senior member would have the best knowledge to explain the treatment to the patient.

> "So that's the irony that once while you need the people who understand the procedure to explain it, they are the ones who don't have the time to do it." (Cardiology SHO, interview 9, line 105-107)

This also shows how obtaining IC is not viewed as important. This SHO talked about the conflict between the duty to explain the procedure and the feeling that his knowledge was not sufficient to inform the patient. Other more experienced HCPs feared that the information disclosed was not accurate. In contrast, another SHO believed that most patients would feel more comfortable talking to a SHO compared to a registrar or consultant where they might hesitate to ask questions.

Apart from the time requirement to obtain IC some HCPs mentioned emergency situations, where the patient was unable to give IC, as a barrier. Doctors mentioned that the information given to the patient had to be reduced to a minimum due to the urgency of the intervention. However, they felt in a conflict between the duty to inform patients and the duty to provide urgent care.

> "So that's why you know, it's not really going to be practical in every situation, to spend so long going through the whole treatment and getting so much information from the patient." (Haematology registrar, interview 17, line 444-447)

> "I don't personally like consenting patients that's where you say, you are going to have x, sign here." (Cardiology SHO, interview 8, line 511-514)

Feasibility of IC for medicines

Although HCPs acknowledged that in theory patients should be informed about each individual medicine in depth, IC for all medicines used in secondary care was seen as unrealistic, since this would be very time consuming and potentially resulting in a longer hospital stay for patients. Table 5.3.9 gives an overview of the drugs mentioned as suitable candidates for obtaining IC in a formal way, either verbally or in writing. Warfarin and amiodarone were the most frequently mentioned drugs.

Table 5.3.9: Medicines suggested as candidates to obtain IC* before initiating therapy

Therapeutic drug class	Drug class or name (number of times mentioned)
Cytotoxic drugs	Cyclophosphamide (1) Hydroxyurea (1) Methotrexate (1)
Immunosuppressant drugs	Azathioprine (1) Ciclosporin (1) Steroids (2) Anti-TNFs (2)
Antihypertensive drugs	Beta-blockers (1) Alpha-adrenoceptor blocking drugs (1) ACE-inhibitors (1)
Anti-arrhthmic drugs and positive inotropic drugs	Amiodarone (4) Digoxin (1)
Lipid-regulating drugs	Statins (1)
Anticoagulants	Warfarin (6) Streptokinase (1) Alteplase (1)
Antiplatelet drugs	Paracetamol (1) Aspirin (2)
Antihyroid drugs	Carbimazole (1) Propylthiouracil (2)
Antibiotics	Amionglyoside antibiotics (2)
Drugs used in diabetes	Insulins (1)
Sex hormones	Anti-androgens (1)

*IC: the HCPs were asked for which medicines they think IC should be obtained. Whether IC was to be verbal or written was not distinguished.

It was suggested that IC for medicines should first be tested in a small group of carefully selected medicines, which are commonly associated with serious, irreversible or appearance changing side effects such as chemotherapy or other medicines which are used on a long-term basis. Regarding the aspect of irreversibility of side effects one doctor pointed out that this was one of the main differences between surgery and medicines. In contrast to surgery, most of the side effects of medicines were reversible. In addition, the patient was the person to take the medicines whereas the surgery is performed by a medical HCP.

> "With taking tablets there is an amount of consent in that they actually take them. Whereas with operations they don't actually do the operation. So it's very easy for a patient to refuse." (Haematology SHO, interview 16, line390-393)

Two nurses mentioned intravenous antibiotics as medicines for which IC should be obtained due to the invasive nature of their administration. One clinician explained that IC should be used for drugs, which are more likely to be used inappropriately. IC would mean that the bureaucratic effort to obtain it could work as a reminder to think very carefully about the use of the medicine and the appropriateness of prescribing.

> "Certain drugs that have that potential to be abused by the medical profession probably should have IC making sure that the side effects are clearly explained. And when people prescribe the drugs actually use the brain and think about why they are using it." (Cardiology registrar, interview 12, line 305-310)

Some doctors pointed out that it was important to allow some flexibility in the amount of information disclosed.

> "If you want this in more gruesome detail tick the box. But it just overwhelms people to put everything in." (Endocrinology consultant, interview 21, line 288-290)

The interviewees differed in their views about the form in which IC needed to be obtained. Some thought verbal IC would be more appropriate than written consent. Others thought that only written consent could provide proof that is recognised in court.

> "I think verbal consent is a waste of time. It's good for the patient who will listen and take it in. Consent is not just about patient taking it in; it is also a defensive issue as well. It is proving you are a professional. I think that needs to be written. I think anything, which is important needs to be written." (Urology registrar, interview 15, line 669-673)

One nurse highlighted there was the conflict between the need to spend more time for consultations and the interests of the doctors who wanted to start the therapy quickly. Some doctors mentioned the fact that information given to patients would need to be individualised, which would require additional resources and skills.

> "It's very difficult to individualise risk in the individual patient. How old, how fit they are?" (Haematology consultant, interview 19, line 254-255)

In response to an enquiring as to which is the most appropriate group of HCPs to obtain IC it is interesting to note that all the HCPs except two thought that another group of HCPs would be more appropriate to obtain IC for medicines rather than their own group. Some HCPs said that under the current climate of increasing litigation patients might need a person familiar with the legal requirements to advise them rather than a medical professional. Other HCPs thought that it was important that the doctor was acting as the patients' agent in obtaining IC.

> "You would want someone you could... you would place a value judgement on as being worthy of and respected enough to have the information and to study the information and take it all seriously and to be...to mean well, to be beneficent to you." (Nurse grade F, interview 4, line 286-289)

Overall, many interviewees felt the time and costs required to deliver the service should be carefully assessed, as they saw this as one of the main obstacles to the introduction of IC for medicines.

5.3.6 Analysis of quantitative data

During the analysis of the data it became apparent that although there was no obvious relationship between age and the desire to have more influence on the selection of medicines there was a relationship between the health status of the patient and their desire to be able to state their preference between various treatment options. All the outpatients who preferred to be asked for their choice between different treatment options scored themselves above 80 on the Euro-QoL self-evaluation scale (100 as the best imaginable health status) (see Diagram 5.3.1 and Diagram 5.3.2). In a UK national survey Kind *et al* (1998) found that for the age group of 60-69 years old the mean state of health on the EuroQoL Rating was 80.

Diagram 5.3.1: Rating on the Euro QoL scale in relation to decision-making preference of 15 patients

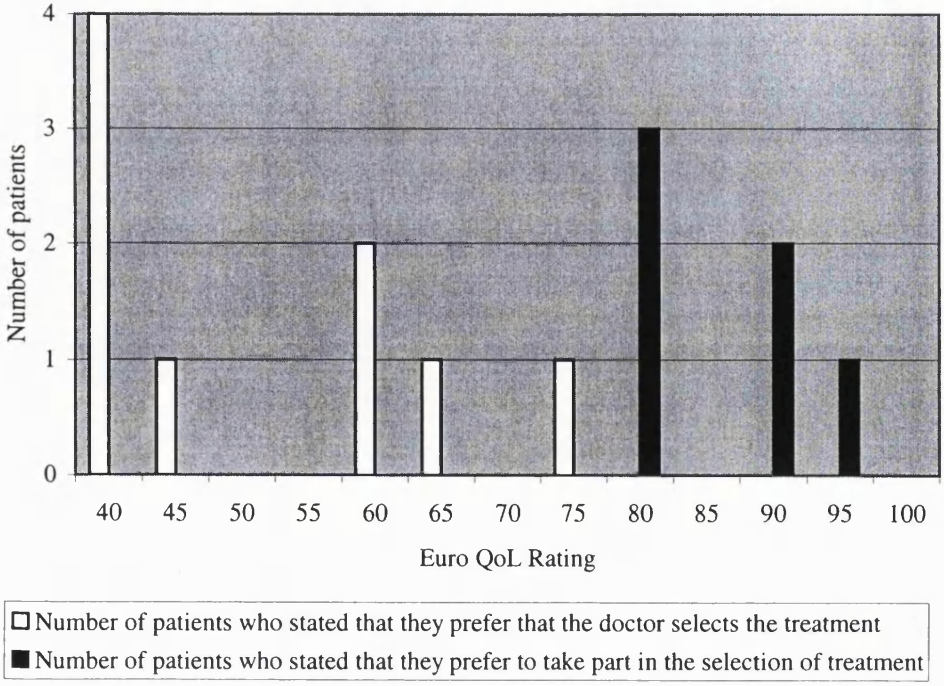
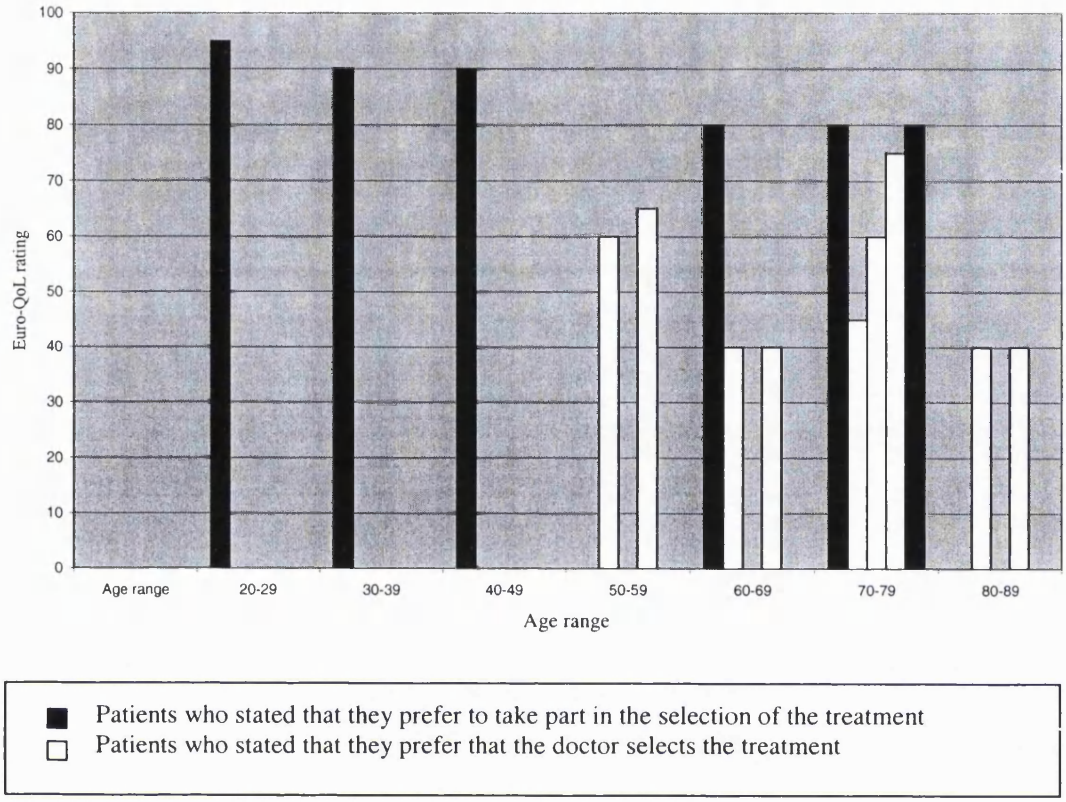


Diagram 5.3.2: Rating on the Euro QoL scale in relation to the age and decision-making preference of 15 patients



The chi-square test shows that the relationship between patient's state of health on the EuroQoL Rating and their preference for having decision-making authority is statistically significant: total chi-square value 15 which means $p > 0.0005$ for the degree of freedom (df) of one (Moore and McCabe, 2000). In contrast to many other qualitative studies, the sample of the outpatients was taken randomly as demographic data were patchy and insufficient to allow sampling purposefully. However, it needs to be stressed that the sample was very small and not powered for quantitative analysis.

5.4 Discussion

The present study investigates the views of HCPs and patients on decision-making about choices of medicines and IC for medicines in secondary care. The results reveal several important findings. First, they showed that in the majority of common prescribing decisions there was a lack of information provision, particularly about side effects and treatment alternatives. The lack of information exchange contributed to the assumptions held by HCPs and patients, which might help to explain some of the HCPs' and patients' beliefs and attitudes towards their role in decision-making. Second, most patients believed that it is the HCPs' role to select the treatment, as they have the knowledge, the understanding and experience to make a choice. This accorded with the viewpoint of the HCPs who believed that it is their role to select a treatment in order to ensure the 'best clinical outcome for the patient'. Third, although IC requires a minimum of information provision and the explicit permission of the patient to the treatment proposed, it was not seen as a tool to support informed decision-making. Finally, the study indicates that the HCPs' meaning of concordance and shared decision-making differed from the definition according to the literature. The participants' view of the concepts of concordance and shared decision-making revealed important aspects of their perception of decision-making and complemented the findings of the study. Before discussing each of these findings in detail some limitations characteristic for the setting, the sample recruited and the researcher will be highlighted.

5.4.1 Limitations of the study

One limitation is the transferability of the results to health care settings other than the one investigated. All participants were recruited at one hospital trust. However, it has been argued that a detailed study of a single site can enable theoretical explanations and a rich description of characteristic patterns (Prior, 2001). Several issues identified have been also found in other health care settings, which indicates that the findings of the study are potentially transferable to other settings. For instance, the aspect of misunderstandings resulting out of assumptions between the doctor and the patient has also been found in primary care (Britten *et al*, 2000).

Compared to general practice, hospital wards and outpatient clinics are highly specialised. Despite the fact that the study recruited participants only from five medical specialities (cardiology, haematology, urology, rheumatology and endocrinology), the medicines used were different in their side effect profiles and the participants varied in their health status. This variety allowed the identification of themes, which may well be applicable within other specialities not included in the study.

Regarding the representative nature of the sample, it was not the aim of the study to achieve statistical generalisability. Rather, the aim was to present a wide variety of views by recruiting the sample. However, it is likely that the variety was limited by the fact that the participants in the study were in general more interested in communication than other individuals who refused participation. Since patients' involvement in decisions requires the ability to communicate, the researcher expected patients' and professionals' views regarding patient involvement and IC for medicines were more positive than the majority of the patients and the professionals. In addition all patients, except four, classified their ethnic background as white. It is not known if a sample of patients with other ethnic backgrounds than the one interviewed would give different results. Some of the hospital inpatients and outpatients who initially were interested in participating in the study did have a different ethnic background. However, in many of these cases these patients were not recruited as they were unable to understand or speak English. In addition, patients who were sicker or had hearing problems (e.g. elderly patients) were less likely to agree to participate which further limits the representativeness of the patient sample.

Another limitation is the potential bias in the account of interviewees. For instance, the location of the interviews may have influenced the answers of the participants. Stevenson *et al* (2000b) pointed out that patients who are interviewed in a health care environment, as were hospital inpatients and some outpatients in the pharmacy department's offices, are more likely to give the answers they believe are expected from them. In addition, with one exception where the interview took place in a side room, it could not be avoided that hospital inpatients were interviewed on the ward, so that other patients in the same bays could hear the conversation. It is likely that this lack of privacy influenced the answers given by the patients. Similarly, this might have

been the case for the three outpatients, accompanied by their relatives, whose presence could also have influenced the interviewee's answers.

The field notes of the researcher were used to reflect on the circumstances in which the interviews were held and how situational factors influenced them. The notes were used to support the analysis of the data. For example, four of the hospital inpatients seemed to have difficulties to talk to the researcher for longer than thirty minutes since they had a hoarse voice or were extremely exhausted. Their answers were probably shorter than if they had been given under different circumstances. If future studies were to include hospital inpatients consideration should be given to split the interview into smaller parts and to visit the patient more than once on the ward.

Interviewing clinicians, especially those in higher positions, was found to be difficult. The time constraints resulted in the interview being short in duration (usually limited to thirty or forty minutes). The choice of the location also caused some difficulties. Commonly, physicians and nurses preferred to be interviewed at their working place where interruptions could not be avoided. Similar to the lack of privacy experienced by inpatients, the HCPs' comments might have been influenced by colleagues working on the ward and listening to the interview. Grbich (1999) describes the difficulties interviewing professionals in higher positions where it is difficult to obtain thick descriptive data. This is not only related to the lack of time. Professionals in higher positions may give very carefully worded answers and feel in a position where they have to represent a public rather than their personal view. With some exceptions this was felt by the researcher to be a tendency in the interviews of the consultants in the present study.

It is in the nature of qualitative research that the researcher's assumptions influence and shape the way data are gathered and analysed (Hammersley and Atkinson, 1995). The professional background of the researcher will have influenced the results of the study. All interviewees knew the researcher was a pharmacist, and this could have influenced their answers. Similar to the interviews of health policy makers and stakeholders, comments made about the pharmacy profession and pharmacists were expected to be more positive than may have been the case if the interviewees did not have knowledge of the researcher's profession. The HCPs interviewed might have felt

a power balance and able to use their professional terminology. Patients were perhaps less open because they might have viewed the researcher as a judge of their behaviour or attitude. Richards and Emslie (2000), studying the influence of the interviewer's background on patients' answers, concluded that a HCP background and a perceived power imbalance in favour of the interviewer could result in obscuring the interviewee's characteristics. This aspect was however critically considered during analysis of the study data.

5.4.2 Information provision

The study showed that in the majority of common prescribing decisions there was a lack of information provision. This was especially the case for inpatients where information about side effects and treatment alternatives was absent, which made it impossible for patients to make an informed decision about their medicines. It could be argued that the results of the present study are less reliable in that they are based on patients' and doctors' perceptions compared to non-participant observation. However, the findings of the present study are in line with previous observational studies using audio-tapes to investigate the actual information provided to in- and outpatients. For example, two US studies found a lack of information disclosure especially regarding side effects and treatment alternatives (Wu and Pearlman, 1988; Braddock, 1999). Other research investigating the patients' perception of information provision such as a recent large UK wide patient survey found that 43% of hospital inpatients felt that they had only partly been informed about their medicines (Department of Health, 2003b). In addition, qualitative research exploring the views of GPs about informing patients about medicines prescribed reported that they tended to provide the patients with a "general endorsement" rather than detailed information to enable assessment of benefits and risks (Stevenson, 2001).

Similarly, Lidz *et al* (1983) reported that lack of time, the ways in which consultations are organised and the ways in which medicines are administered on wards are important barriers to informing patients about their medicines. Similar to findings in other studies, in- and outpatients in this study expressed their desire to be informed about side effects and alternatives, although the information was not used to make a trade-off between treatment options (Beisecker and Beisecker, 1990). In addition, as

other studies pointed out, wanting to be informed did not go along with an increased desire to become involved in the decision-making (Blanchard *et al*, 1988; Ende *et al*, 1989; Degner and Sloan, 1992; Vick and Scott, 1998). Instead, the information served as reassurance and establishment of trust between the doctor and the patient as described by Tuckett *et al* (1985) for primary care.

Some doctors reported that patients brought information from the Internet into the consultation. Similar to the experience of doctors in the present study a recent work investigating the influence of the Internet health information on the doctor-patient relationship showed that most patients tended to ask the doctor about their opinion instead of requesting a specific intervention (Murray *et al*, 2003).

The present study explored the hospital physicians' and nurses' views of the current state of information provision, which they regarded as unsatisfactory. In a busy day-to-day clinical encounter on the ward or in the outpatient clinic informing the patient about more than the indication, the dose, the frequency and perhaps the most common side effects was regarded as a "luxury". In addition, the option to see the patient a second time before initiation of therapy was perceived as a privilege, only possible in some specialities. The amount of information disclosed depended on the individual clinician and the setting. The provision of information about side effects and alternatives became a legal duty only above a certain level of risks of irreversible, appearance changing side effects or where the clinician was uncertain about the benefit compared to other options. Otherwise other tasks than informing the patients had preference.

In addition, the present study enabled a detailed comparison of the views of HCPs and patients regarding decision-making. One important finding of the comparison was that there were several assumptions on both sides, which varied considerably and affected the amount and type of information disclosed and the process of decision-making. In some cases the assumptions resulted in misunderstandings, a finding which was also reported in a study conducted in primary care, which identified 14 categories of misunderstandings based on assumptions and guessing (Britten *et al*, 2000).

5.4.3 Beliefs about the role of patients and doctors in the decision-making

The belief of HCPs and patients about their role in the decision-making process determined their attitude towards it. The HCPs approached decision-making with the belief that it was their duty to select the medicine that they thought would result in the 'best clinical outcome' for the patient. Hence, in common prescribing situations the provision of information was not tailored towards enabling the patient to make a selection between various treatment options as the decision-making models suggest. The provision of information was seen as a function used to achieve "best clinical outcome" and not primarily justified on the grounds of the patient's right to information. The information provided served two purposes: first, as a motivator, in informing the patient about the indication of the treatment as it was thought that patients would agree with the doctor about the necessity for treatment and hence, they would take the medicines as directed. The function of information in persuading the patient to carry out the advice of the professional has been highlighted by Becker and Maiman (1975). However, this seems to be a simplistic view of the factors enhancing patients' compliance. Research indicates it is likely to be a combination of factors that results in increased compliance rather than a single factor such as a clear explanation about the purpose of the treatment (Haynes *et al*, 2002). In addition, Horne and Weinman (1999) suggested that an exploration of the patients' beliefs about medicines is a more successful strategy to improve compliance as it could reveal some important reasons why patients may not be compliant. However, an exploration of the patients' beliefs about the medicines proposed for treatment was not mentioned at all by the participants in the study. Instead, guessing and assumptions were common, as described before.

The second function of the information was to enable the patient to give feedback. The HCPs believed that the patient could influence the choice of the drug if they had already taken it. It is important to note that this was a belief which was also expressed by the majority of patients, who saw their role in decision-making – in the sense of influencing the choice of treatment - in giving feedback after the initiation of therapy. Only if patients had previously taken the drug was their contribution regarded as meaningful. Experience in drug use enabled the patient to take part in the selection of treatment as a 'partner'. This means that patients can only fully participate in decision-making if they have already used the medicine. The finding that information provision

about medicines was neglected as long as the patient stayed on the ward (where the side effects and the compliance were monitored) strengthens the conclusion that information provision was viewed in a very functionalist way.

The finding of the study that the majority of patients preferred the doctor to make the decisions about their medicines has been confirmed by Elwyn *et al* (2003). Most patients felt uncomfortable choosing an option without guidance of the physician, as mentioned by Silverman (1987). Similar to the findings of previous research, sicker patients in particular preferred the doctor to make the decision regarding their treatment (Blanchard *et al*, 1988; Ende *et al*, 1989; Degner and Sloan, 1992). This supports the argument that a paternalistic attitude regarding decision-making is common when patients are very sick. However, the findings of the study highlight that deferring the decision to a doctor did not mean that patients wanted to hand over full control. There was a degree to which patients preferred to have influence over the final implementation of the decision, which varied from "being consulted" to being asked for their permission. Another indication that patients wanted to have some control over the decisions made was the fact that they expressed the wish to appoint a decision-maker whom they trusted and a doctor who acknowledged their individuality. The ability to choose a HCP of their choice was perceived to be a way to solve the problem of selecting a medicine for treatment. Some patients explained that they would choose a doctor according to his or her professional experience. Others choose the doctor according to his or her communication style as previously reported by Hall and Roter (2002) or according to their own attitudes (Rogers, 1999).

In general, at the time of initiation of the treatment the majority of doctors and patients saw the selection of a certain medicine as part of the technical choice and therefore, a task of the doctor. The theme of "expert" choice is important in many ways. In the biomedical understanding the physician can establish the choice of treatment in most cases without exploring the patients' values and preferences (Sullivan, 2003). Furthermore, it is believed that HCPs have to make the choice because only they can define the most appropriate treatment option. The physicians' interviews clearly indicate that in the large majority of prescribing decisions the physicians aimed for the most effective treatment established from clinical trials and their own experience (within the range of drugs available within the hospital formulary). As a result there was commonly only one treatment option regarded as most effective. Others were

excluded since they were thought to be less effective. Only in cases where there were equally effective - or as some physicians said "equally acceptable" - treatment options were these offered as treatment choices to the patient. In contrast, in the understanding of 'patient-centred medicine' a recommendation of a treatment course would only be made after the patient preferences and values have been established (Stewart *et al*, 1995). Tuckett *et al* (1985) argued the patient is the "expert" in his own body and life. This means that the patients' contribution is seen as essential in order to define the outcome and therefore, the possible range of options and the final choice. Following on from this, the results of the study raise two fundamental questions about patients' involvement. The first is, what is the range of "equally acceptable" treatment options, which can be offered and what determines the range of choices. The second question is about the circumstances in which time, being one of the most pressured resources, should be taken by HCPs to offer treatment choices and discuss them with patients. In other words, what are the decisions in which it is "worthwhile" to practice a consultation style where the patient participates in decision-making about treatment choices.

Regarding the first question about the range of treatment options offered, Gwyn and Edwards (1999) discussed the problem of "equipoise". This means the existence of equally preferred options from the viewpoint of the physician, which they regarded as a presupposition for shared decision-making. They pointed out that limiting the range of treatment options offered might be interpreted as paternalistic whereas, in fact, it is acting responsibly. As seen from the study results the information disclosure regarding side effects of warfarin illustrates the difficult balance between acting responsibly in warning the patients about potential side effects and being paternalistic in withholding information which may influence the patients' choice.

Although physicians mentioned that they would choose the most effective treatment, the results indicate that there were many other less overtly discussed factors in play, which determined the range of choices offered. These included cost considerations; time available in the consultation to explain the treatment options; the knowledge of the physicians about each treatment option; the physicians' own experience in prescribing and monitoring the medicines as well as the ability and skill in explaining the options to the patient. These factors were largely invisible for the patients, which

raises the question whether the patient would agree to a selection of a treatment, which is determined by cost considerations rather than for instance safety. It also raises issues about the duty of the doctor to disclose, firstly, the factors influencing the selection apart from clinical effectiveness and safety and secondly, disclosing the other treatment options which are not available due to organisational constraints, as argued by Sharpe and Faden (1998). This would increase the transparency in the decision-making process, showing that the doctor felt not only a responsibility towards the patients, but also acknowledged a responsibility towards the organisation. The extent to which the physician took the role of a gatekeeper in providing access to medicines was mainly obscure for patients, but was an issue some patients were alert to.

The results showed that many patients believed that there would be only one "right" treatment option; different clinical opinions confused patients. This suggests that patients were not aware of the values implicit in clinical decision-making and the lack of knowledge or uncertainties about the clinically most effective treatment. It is likely that this is one of the reasons why patients did not see their contribution in the decision-making process as potentially useful or necessary (Robinson and Thomson, 2001).

With regard to the question of which medical decisions the patient should be involved in, it is interesting to note that studies on patient participation in medical decision-making have been carried out predominantly in oncology and surgery (Guadagnoli and Ward, 1998). The result of the present study indicates a similar trend in which patient preferences with regards to treatment were predominantly obtained at initiation of therapy which could cause serious, irreversible and appearance changing side effects. Patient participation only becomes relevant if it is legally required. However, patients and HCPs did not see patient participation in common prescribing decisions as a way to optimise the success of treatment, either due to the positive effect of involvement itself or due to the ability to tailor treatment to the individual preferences.

Charles *et al* (1997) recognised that according to setting and circumstances certain models of decision-making are more or less appropriate or feasible. It is debatable which treatment decisions are ones where involvement of patient in the selection of the treatment is regarded as significant or desirable. Kassirer (1994) argued that these are

the "utility-sensitive" decisions, where the outcomes may be viewed differently between doctors and patients. Others added that beside the outcome preference the way in which the outcome is achieved may determine the option chosen (for instance a patient decides not to undergo the intervention due to their dislike of it, even though it improves their quality of life) (Bowling and Ebrahim, 2001). Whereas many doctors in the present study used antibiotic therapy as an example where they thought selection of an agent did not require eliciting patient preferences, some studies indicate otherwise (Elwyn *et al*, 1999; Butler *et al*, 2001). On the contrary, physicians mentioned decisions which involve treatment potentially resulting in loss of fertility as important to elicit patient preferences. It is likely that between these extremes - acute treatment with antibiotics and chemotherapy with potential fertility loss - there are many others which have not yet been identified and which could be the subject of further research.

5.4.4 Patients' and HCPs' views on IC for medicines

HCPs and patients doubted the feasibility of IC in practice, though some thought that it offered an incentive to strive towards more patient involvement. The continuous tension between the ideal of IC and the feasibility to apply it in practice was characteristic for the patient's and doctor's discourse about IC. In many cases IC was either reduced to the act of information provision or the authorisation. Lidz *et al* (1988) called this the event model in contrast to the process model, where a decision is seen as evolving over time. Jonsen (1998b) warned that IC is more than either "listing the risks and benefits" or "a mere 'okay' to an obscure request". The majority of patients and HCPs interviewed focused on the information provision rather than the act of giving permission which is in line with the findings of another large survey amongst doctors in North America (Presidential Commission for the Study of the Ethical Problems in Medicine and Biomedical and Behavioral Research (PCEMR), 1982). Asked about the meaning of IC more than two thirds of the doctors in that study did not mention the act of obtaining permission. Instead, "telling" the patient about the intervention was in the foreground.

Hospital inpatients in particular saw IC as a way to guarantee a minimum level of information and the opportunity to raise questions, which indicates that apart from some patients who were satisfied, the large majority perceived there to be a lack of

information provision about their medicines. However, asking for their permission to treat was seen as an act of courtesy and respecting them as individuals, not an incentive to seriously consider treatment alternatives. Most hospital patients expressed their gratitude and trust towards the HCPs and could not imagine a situation where they would refuse treatment. This may be due to the fact that the decision to come to hospital was regarded as the only decision which they had control over, whereas all subsequent decisions, and with it responsibility were handed over to HCPs. This was in contrast to HCPs who believed that in giving consent the patient had the responsibility for the decision. Mark and Spiro (1990) in their study of IC in colonoscopy reported similarly that physicians stated that they felt the decision about the intervention was made by the patient or shared, whereas the patients thought the physician had made the decision.

In contrast, outpatients were more in control of their lives. They were more sceptical about the overall benefit of IC, which might be due to the fact that they were less dependent on the HCPs to obtain information (for example they received patient information leaflets with their medication) or that they received in general more information during the consultation than hospital inpatients. In addition, it was found that outpatients felt more able to stop or change the regime prescribed, as they were taking the medicines without supervision of the HCPs.

The theme of "not being in control" appeared several times in in- and outpatient interviews when referring to the situation of signing a consent form. This shows that one of the criteria of IC, namely to act free from the influence of another party which would determine the outcome of the choice, may be not fulfilled. Some patients felt obligated to sign the consent form; HCPs mentioned that inpatients especially might feel they were being difficult if they refused treatment. Sociological studies into the hospital organisation and the implication on patients have featured similar aspects, among them the picture of the "good patient" who is compliant (Morgan *et al*, 1993). Non-adherence would have negative implications. This indicates that the freedom of patients to refuse treatment may partially be limited.

Most HCPs admitted that the current practice of information provision to the patient was not sufficient to obtain valid IC for their medication. Despite the lack of

information about side effects and treatment alternatives the majority of HCPs interviewed thought that the current prescribing and dispensing practice on the wards did not undermine the right of the patient to refuse treatment. The HCPs argued that patients could refuse to take the medicine if they wanted. In general, their stay in hospital and the presence of the patients on the ward was seen as implied consent for treatment, which is similar to the findings of other studies (Lidz *et al* 1983; Verhaak *et al*, 2000; Aveyard, 2002).

That the permission to treat was assumed rather than elicited shows that there is still a large degree of tacit understanding (O'Neill, 2003). Although the autonomous authorisation is one of the core issues of IC, it was commonly not explicitly obtained in cases other than written IC. Instead, it was expected that the patient would give a sign of disapproval if they had an objection to the treatment given.

IC was commonly not viewed as a way to offer patients a variety of different treatment options. IC was usually obtained after a treatment decision had been made and was therefore only regarded as a formality.

Some HCPs believed that the information disclosed caused more harm than benefit, which resulted in situations where HCPs disclosed partly biased information – emphasising more the positive effects of the treatment or the intervention proposed. Nurses in particular felt that their role was to protect the patient from unnecessary fears and anxiety. This reveals the nurses' dilemma between 'causing no harm' and 'respecting autonomy'. Other studies reported a difference between doctors and nurses in their perception of ethical values according to their professional duties (Chaplin, 2002). Although nurses feared that information disclosure could result in an increased number of patients refusing therapy, earlier studies did not find an increase in anxiety among patients or an increased number of patients refusing treatment after obtaining IC (Kerrigan, 1993; Quaid *et al*, 1990). The samples in these studies included patients undergoing elective hernia operation and outpatients suffering from epilepsy. It has been pointed out elsewhere that the argument of acting beneficently in not disclosing information which might harm the patient, could disguise some paternalistic attitudes (Tingle and Cribb, 1995).

The potential harm caused by information disclosure is legally regarded as one of the exceptional circumstances in which IC does not need to be obtained (therapeutic privilege) (Faden and Beauchamp, 1986). Others are circumstances where the patient is incompetent, patient waiver, and public and medical emergencies. It is interesting that HCPs cited emergencies and that patients preferred to leave the decision with the doctor (e.g. patient waiver) as an argument against IC. This either indicates that there might be a lack of knowledge among HCPs about legally justified exceptions or that it is less clear which situation the court would construe as being a medical emergency.

In terms of benefits of IC nurses focused on the practical implication of the information for patients such as improved management of their medication and more realistic expectations of the treatment outcome on the part of the patient. This was also mentioned in the literature as one important benefit of IC namely preventing patients from feeling deceived (Wear, 1995). In contrast, doctors saw their legal protection as one of the most important benefits of IC, which was partly illustrated by the fact that the severity of the side effects determined the use of IC. It was regarded as necessary in the current climate of an increasing amount of litigation (National Audit Office, 2001). However, the fulfilment of the legal requirement was not regarded as a benefit for the patient and was open to misuse. Another study investigating the patients' perception of IC to clinical trials in oncology by using in-depth interviews also found that patients signed the consent form even if they did not understand the disclosed information (Cox, 2002). In addition, patients were very susceptible to the way in which the information was presented to them.

Some of the doctors in the present study addressed the ambivalence between the legal duty to obtain consent and the ethical meaning, the "ethos" of IC. Faden and Beauchamp (1986) described the fulfilment of the legal duty as the policy-oriented sense of IC and called it "sense 2". They differentiated that from "sense 1" of IC, which is primarily an autonomous authorisation enabling the patient self-determination. According to the HCPs' account IC appears to be a double-edged sword. Whereas on one side it seems that the threat of potential legal claims is a reminder to spend time and thought to acknowledge the "ethos" and facilitate patients' decision-making, on the other side various pressures resulted in only fulfilling the legal requirements and pure formalism without fulfilling the patients' rights.

Frequently mentioned pressures were the lack of resources such as the number of medical staff and the time needed to obtain IC for medicines even if IC is obtained only for some medicines. That the lack of time and resources results in a situation where the task of obtaining IC is delegated to the most junior member of the team, who were often not adequately trained, has been confirmed by several other studies (Richardson, 1996; Ferguson, 2001; Paice *et al*, 2001). Adequate training of HCPs needs to be taken into consideration if a formal process of obtaining IC for medicine is introduced into medical practice.

5.4.5 Comparison between the HCPs' views and the decision-making models in the literature

The findings of the study gave insight into the HCPs' views on decision-making about medicines. Although many HCPs stated that they practice shared decision-making or concordance, their accounts suggest that their understanding of the models differs significantly from the definition in the literature. A comparison of the models of concordance and shared decision-making with the views of the physicians in the present study can reveal the barriers in implementing the models in practice. It also illustrates the different opinions about what is seen as the aim of medical decision-making and the ways in which to achieve them.

The concept of concordance for example is defined as "the creation of an agreement that respects the beliefs and wishes of the patient" (Royal Pharmaceutical Society and Merck Sharp & Dohme, 1997). There were two main differences between the doctors' understanding of decision-making and the definition of concordance in practice. First, agreement was often assumed and not explicitly discussed. Second, and perhaps most importantly, the aim of the consultation was not the agreement between doctor and patient itself. The aim was to achieve a certain clinical outcome defined by the doctor. An agreement itself with the patient to improve the patient-doctor relationship was not the intention of the doctors. Instead, the patients' agreement was a means to enhance compliance. It is hard to imagine that one of the doctors interviewed would have agreed to a 'drug holiday' as suggested by Dowell *et al* (2002) to support agreement and improve the relationship with the patient.

The majority of doctors ideally favoured shared decision-making, though they acknowledged that the model was only applicable to a minority of prescribing decisions due to 'practical' reasons. First, it was regarded as very time consuming and second, and perhaps more importantly, it was often unclear how individual preferences or values can play a role in the selection of medicines if the clinically most effective treatment is given preference. In addition, the meaning of shared decision-making given by doctors differed from the concept suggested by Charles *et al* (1997). The responsibility for the consequences of the choice of the treatment was regarded as that of the patient once they had given permission for the treatment, instead of shared between doctor and patient according to the model by Charles *et al* (1997). Furthermore, none of the doctors talked about "negotiation" of treatment goals. Instead, they used phrases such as "convince", "persuade" and "win them [the patients] over" indicating that the patient was not seen as an equal partner who could give any reason that would convince the doctor that the doctor's choice was wrong. The HCPs account showed that the information was not necessarily presented in a neutral way and that doctors' preferred options were described in a more positive light, a tendency which has also been reported by Silverman (1987) investigating paediatric consultations. Many doctors believed that in the majority of prescribing situations it was their professional role to prescribe what they regarded as the clinically most effective treatment and it was up to the patient to accept that offer. Similarly, results of analysing audio-tapes of primary care consultations found little evidence of shared decision-making in primary care although GPs believed that they practised shared decision-making (Stevenson *et al*, 2000a).

Considering the different kinds of choices potentially available – the choice between different medicines, between conventional and alternative medicines, the amount dispensed, whether to take the medicine or not, the dose, how long to take the medicines – the patient has the opportunity to exercise choice by influencing the dose and whether to take the medicine or not. However, what type of medicine and the amount dispensed is defined and authorised by the doctor. It is left to the doctor to determine the degree of patient's freedom in contributing to this choice. The study indicates that among all possible degrees of patient involvement even the legally required minimum of patient's engagement, namely IC, is often not implemented.

As seen from the results there is a big gap between the models and how patients and doctors viewed their role in decision-making. One key factor was that doctors understood 'patient participation' as sharing the information about the outcome of the decision including the rationale for it. This was in discrepancy to the models of concordance and shared decision-making, which are based on the concept of sharing the decision-making authority, and deviant from informed consent, where patient self-determination is the key principle. However, the findings suggest that in many prescribing decisions neither doctors nor patients gave the highest priority to the value of shared decision-making authority or self-determination of the patient. Whereas for doctors choosing the most effective medicine had priority, the patient interviews highlight that in a situation of illness other values, for instance reassurance, trust and minimising side effects of the medicines, could be given higher priority than self-determination in the sense of selecting a medicine. This suggests that for a successful decision-making about the choices of medicines an agreement between doctors and patients about the priorities concerning the aim of the decision-making and the process is desirable. In the following chapter the implications of the findings will be discussed along with the implication of the findings from the studies of health policy decision-making.

PART D: COMPARISON OF HEALTH POLICY AND INDIVIDUAL DECISION-MAKING

Having covered the first two key aims of the thesis of investigating health policy and individual decision-making about choices of medicines, Part D will address the final aim of the thesis: identifying the similarities and differences between the factors found to contribute to each type of decision-making about choice.

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CHAPTER 6: DISCUSSION AND IMPLICATIONS FOR PRACTICE

The thesis investigated decision-making about access to and choice of medicines from two perspectives: first, authoritative decision-making about access to three drug therapies in the NHS and second, individual decision-making between HCPs and hospital in- and outpatients. The final chapter summarises their similarities and differences, and attempts to join the two perspectives into one picture of decision-making about medicines in health care. This will be followed by highlighting the main limitations of the research methods used, that have impacted on the study findings and their interpretation. Finally, implications for practice will be described, including areas for further research.

6.1 Relationships between macro and micro level decision-making

Although in the past health policy decision-making and clinical decisions concerning the care of one individual have been investigated and discussed separately, both types of decision-making are interrelated. The most overt relationship is probably the fact that the macro (policy) level directly determines some conditions of the micro (prescribing) level; if policy forbids a medicine it is unlikely to be prescribed. Regarding choices about medicines it means that the macro level influences the range of therapeutic options and the amount of care available on the micro level. Conversely, the micro level use of resources can affect the amount of resources available to decision-makers, although this influence is indirect and depends commonly on the decisions made by many decision-makers on the micro level in order to have impact on the macro level.

The other relationship between health policy and individual decision-making is that they are both nested within the wider framework of the health care organisation, such as the NHS as in the case of this research. For instance, the values promoted by the NHS influence both the macro and micro decision level (Neuberger, 2002). Being situated in the same organisation means that, apart from the common task of making decisions about access to medicines (as mentioned before, prescribing decisions can be understood as providing access to medicines), a common terminology is used. However, due to the different focus of health policy (focused on population benefit) compared to doctor-patient decision-making (focused on the benefit of the individual), the importance and the ways in which they are debated may differ. Table 6.1.1 summaries some of the common aspects of health policy and individual decision-making and how they are discussed and presented in both settings.

Table 6.1.1: Common issues of health policy and individual decision-making

Issue	Health policy decision-making	Individual clinical decision-making
Clinical evidence of the efficacy of the intervention	Discussed in detail and presented as the basis for the decision made.	Usually only considered by the clinician.
Financial costs	Commonly determines the decisions.	Clearly influences the decision, however, usually only considered by the clinician.
Most pressured resource(s)	Costs in financial terms. Costs in terms of organisational burden.	Time.
Presentation of treatment alternatives	Treatment alternatives are drawn from a narrow scope (e.g. usually only medical treatment alternatives considered instead of social care interventions).	Lack of communication about treatment alternatives.

For instance, the evidence on which the decision is based is relevant on the individual level as well as on the health policy level. However, its influence on the decision varies as well as the way in which it is discussed; whilst the clinical evidence about the efficacy of a drug is made available to health policy-makers, in an individual clinical setting this evidence is usually only considered by the HCP and perhaps partly disclosed to the patient.

One of the most striking tendencies on both levels was the finding that the technical aspects were very much in the foreground of the debate of the decision-makers (not the patients as they were commonly not the actual decision-makers). The technical aspects

included the clinical properties of the drug therapy in question and the scientific analysis of the information available about the therapy, including economic data. Whereas technical aspects were explicitly discussed, values were mostly implicit. This is likely to be related to the medical background of the majority of decision-makers who were familiar with the scientific analysis of data and regarded as the ones with technical expertise. However, most of them were unfamiliar in debating values or not aware of the values implemented in the technical approach they took. Legitimising the decision was found to be another reason why health policy-makers in particular tended to emphasise the technical aspects of decision-making. In terms of individual decision-making, the belief that decision-making about choices of medicines is mainly a 'technical' task resulting in the fact that many patients had a preference for the HCPs whom they regarded as the experts, to make the decision.

Apart from the analysis of clinical and economic data, the process by which the decisions were made was often regarded as one of the technical aspects of decision-making. The results showed that there is more agreement about the decision-making process on both levels – in both cases, there was an emphasis on procedural safeguards compared to a consensus about values. On the health policy level in particular, much importance was given to the procedural side of decision-making. On the individual level, informed consent (IC) fulfilled the function of a process, which was legally defensible. The process was used to legitimise the decision made, whereas the outcome was less important. There is the danger that in the future health policy decisions will be divided in 'sense 1' and 'sense 2', as described by Faden and Beauchamp (1986) in the context of IC. The fulfilment of the legal and political requirement as the process-oriented sense of health policy decision ('sense 2') would replace the 'sense 1', which is focused on the outcome of ensuring fair and just provision of access to medicines.

Another important finding is that the full range of treatment choices available was obscure on the micro as well on the macro level. It is surprising that despite the promotion of choice as an important value, there is little attention given to identifying the potential range of options. On the health policy level, for example, there was an absence of data comparing various treatment alternatives with each other, in particular medical with social care interventions. On the patient-doctor level the absence of the presentation of treatment alternatives was partly related to the lack of knowledge of the

decision-makers which would reduce the options of the patient's choice to either accepting or rejecting a proposed treatment. (Apart from accepting or rejecting access to treatment, health policy-makers preferred a third option, which was the acceptance of access, but limited to a certain group of patients.)

In comparing the views of the decision-makers in health policy and individual clinical decision-making with the models developed in the literature it becomes apparent that the models do not address some of the key factors of health policy and individual decision-making. For instance, on a health policy level Daniels and Sabins' framework of 'accountability for reasonableness' (1998) does not highlight the problem of a very limited set of rationales commonly used to define and justify health policy decisions. That other factors such as maintaining relationships with HCPs or avoidance of organisational burden were hidden by rationales, such as efficacy of the therapy or safety considerations, indicates that these were not regarded as an acceptable justification for a decision. However, they were important for the understanding of the entire process and affected the outcome of the decision.

On an individual level the phase of deliberation – that means exchanging values and preferences and the way they can be implemented into the decision in a meaningful way - has not been described in detail (Elwyn *et al*, 2001b; Charavel *et al*, 2001). A literature review on instruments measuring patient involvement in shared decision-making found no single instrument which was developed to measure specifically the 'involvement' of patients (Elwyn *et al*, 2001b). Instead, the stage of deliberation was described in broader terms such as "mutual discussion", "negotiation" etc. That there is no fixed catalogue of criteria which need to be fulfilled in order to determine if patients and physicians engage in the deliberative phase of a decision could be one reason why the implementation of decision-making models such as shared decision-making and concordance is difficult in practice.

The analysis showed that there was often a lack of feedback on the macro and the micro level. Despite the fact that decision-makers were aware that the decisions needed to be reviewed, the way in which health policy and individual decision-making were organised did not necessarily foster review and thorough feedback. Although national organisations and initiatives, such as NICE and NSFs, have implemented regular

review of their guidelines, on the regional and local level several policy-makers highlighted the absence of regular review due to the limitation of resources. Similarly, on the micro level patients reported that the prescribing decisions were often not reviewed due to the difficulty of getting an appointment or seeing a different HCP who did not know about their case.

Although, as mentioned before, the aims of the policy decision in serving the population and of the micro level decision in benefiting the individual are very distinct, the results illustrate that both decision levels contain elements of each other. That means that health policy decisions partly include considerations of the individual benefit and conversely, individual decisions are affected by population concerns. For instance, the Alzheimer's Society emphasised the perspective of the individual in the debate by using quotes from patients, and clinicians cited anecdotal evidence of individual cases where they saw great individual benefit of the therapy. On the individual level the doctors highlighted that health policy decisions influenced their decision-making such as guidelines and policies restricting prescribing choices. Other authors described this as the role of being "double agents" with a responsibility towards a health organisation and the individual seeking care (Vick and Scott, 1998; Sculpher *et al*, 2002). As seen from the results the balance between population or individual focus depended on the situational circumstances of the health policy and on the prescribing decisions. At the same time it shows that there may be conflicts between health policy makers and individual decision-makers. For instance, the goal of cost containment which was often the most important consideration in health policy decision-making had not necessarily the same priority when prescribing a medicine. Another potential conflict arises from the contradiction between health policy rhetoric and clinical practice. Although health policy promised increased patient choice, doctors were often unable offer treatment alternatives partly due to cost consideration, their limited knowledge or experience in using them.

6.2 Limitations of the research methods

In discussing the results of the thesis it is important to highlight some of the limitations of the research methods and the settings chosen for the work carried out. Two different

research methods were used: document analysis and in-depth interviews. The documents analysed were intended for public circulation, which meant that they were usually easily accessible. However, a limitation was that they only provided data, which were intended for publication (Silverman, 1993), which meant that authors and publishers had their own agenda influencing the contents and presentation of the document. The aspects of policy decisions, which were either regarded as less important or undesirable for publication, were omitted from the publications.
The main research method used in this thesis was the conduction of in-depth interviews with the aim of gathering information on the interviewees' account of decision-making. This permitted a limited picture of decision-making in only viewing the process through the eyes' of the participants or stakeholders. Additionally, the interviewees may act very differently in the actual situation. This has been found, for instance, in studies using both in-depth interviews as well as observation technique to study doctor-patient interaction (Barry *et al*, 2000). This does not mean that the interviewees deliberately withhold the truth. Instead, their reflection of the decision-making gives account of how they made sense out of it. Similar to the documents analysed, the interviewees were aware that the results of the research were intended for publication which may place a constraint on discussion of confidential or controversial information as well as factors which could cause embarrassment (e.g. admitting not to taking any tablets).

Health policy-making and individual decision-making was studied in the British NHS, a publicly funded health care system. Results are limited to a specific structure of health care delivery in a certain cultural setting. In addition, the research was carried out at a time of major changes in the structure of the regional authoritative bodies such as the establishment of PCTs and strategic health authorities. Many interviewees mentioned that it was uncertain how decision-making in the future would be affected by the restructuring of the regional bodies. Individual decision-making was studied in secondary care, where a large teaching hospital was chosen. Application of the findings to other secondary care settings such as general hospitals or other primary care settings is therefore limited. However, as mentioned earlier, similar results have been found in primary care.

6.3 Implications of the results

Policy analysis serves various goals; one of them is to suggest ways in which policy-making can be improved (Dunn, 1983). Similarly, the investigation of clinical decisions concerning the care of one individual can suggest ways in which the day-to-day clinical interaction between patients and HCPs could be improved. The findings presented also indicate areas where further research is warranted. The implications for the health policy level will be addressed first, followed by the implications for individual prescribing decisions.

6.3.1 Implication for health policy decision-making

The research findings in the area of health policy decision-making can heighten awareness of specific decision-making patterns and can also help to identify therapies for which it is particularly difficult to make decisions. Furthermore, clarification of the concepts of need, health technologies and "rational" decision-making can broaden the agenda and result in consideration of additional treatment options. A change in the political rhetoric of policy-makers and stakeholders, which influences public expectations, can result in more transparency and open debate.

Increasing awareness of specific patterns: The research findings can increase awareness of specific patterns in policy decision-making and lead to a conscious consideration of the current process and organisation of decision-making. For instance, one of the patterns found in the decisions made about the three drug therapies investigated was that the decision was thought to be based mainly on technical measures without much consideration of values. An increased awareness by policy-makers that values are built into technical measures can result in a more explicit debate about the desirable ends of decision-making. This could be done in explicitly addressing the values at stake when making a health policy decision and making the discussion of it a compulsory part of policy documents.

Therapies for which it is particularly difficult to make decisions: The results of the study suggest that there are therapies where it is particularly difficult to make decisions about resource allocation. As mentioned earlier these are therapies which were thought

to result in considerable cost expenditure, which were used in a life-threatening condition, in conditions leading to a considerable degree of disability and conditions for which no other treatment alternative is available, where the benefits were not widely accepted or where its provision was strongly lobbied for by an interest group. These situations reveal some of the limitations of the methods and decision-making processes. For example, the case of rivastigmine illustrates how difficult it is to make decisions about the clinical effectiveness of a therapy if there are only intermediate outcomes. Training for health policy makers could increase their awareness of potential dilemmas and support them in making decisions.

Definition of need and its consequences for decision-making: The result of the biomedical model of health is that many needs outside the model are not addressed within the public health care sector. The socioeconomic determinants of health are potentially neglected. However, if rationing is justified on the ground of need it is necessary to be very specific in the way need is defined and the limitations of the definition used. For instance, the definition of need according to expert opinion means that the perspective of the individual receiving the care may be neglected.

Concept of health technologies: It has been argued that uncertainties make decision-making difficult. From the results it can be concluded that the policy-makers try to predict uncertainties in terms of clinical benefit, including safety and cost impact. This indicates that on the introduction of new therapies the main focus is placed on these two variables of uncertainties. Inclusion of sociologists and ethicists as policy-makers can enhance the awareness of social and ethical factors pertinent to the introduction of new health technologies including drug therapies. Currently, only certain expertise is used, mainly that of professionals from clinical, epidemiological or economic sciences. This may also influence the definition of what is defined as "rational" and "reasonable". The results suggest that the concept of health policy decision-making needs to be complemented by factors which are commonly not discussed in the theoretical models of decision-making.

Exploration of ways to implement patients' perspectives in a meaningful way: To deal with the absence of consideration of social and ethical issues in health technology assessment some have suggested the inclusion of a patient impact assessment which in

particular would address the patients' experience with the drug therapy (Barnett, 2002). Although this would certainly complement the information about the therapy in question it is less clear how the patient's account should be evaluated. For instance, the results showed that the interviewees gave the patient's account of their experience more credibility if it was presented in an unemotional way. However, that does not necessarily provide a more 'truthful' account. Alternative ways need to be explored to evaluate how the patient perspective can be used as a base to assess a novel therapy.

Political rhetoric and public expectations: Defining the NHS service as 'comprehensive' may create unrealistic expectations about available services. The results indicate that absolute 'no'decisions are an exception. As seen in the case of the three drug therapies, access in general is provided but limited to a certain group of patients. On the one hand, some authors have characterised these patterns with "trying to do a little of everything" (Honigbaum *et al*, 1995). On the other hand, that does not mean that it is necessary to define a basic package of health care. Examples from the Netherlands (Ham, 1997) and Israel (Chinitz *et al*, 1998) show how difficult it is to do so. However, a public discussion about the overall level of public expenditure on health care could result in more realistic expectations. Furthermore, the assumption that the health service is comprehensive may force policy-makers to think about how to dilute the resource to achieve cost containment instead of excluding the service and offering support to those who cannot afford to buy the service privately. This may result in a more honest account of health service and enable policy-makers and clinicians to talk openly about the options, which are not offered on the NHS but which nevertheless could provide benefit for patients.

In summary, possible ways forward are

- to include ethicists and sociologists in the decision-making committees;
- to offer training for policy makers;
- to address explicitly values at stake and the definition of need in the policy document;
- to develop methods to assess and implement patient experience in and views about using the technology in question.

6.3.2 Implications for individual decision-making

In terms of individual clinical decisions the findings highlight the need to improve the dialogue between HCPs and patients and explore ways in which consultations of hospital in- and outpatients can be organised in order to support the dialogue.

Implications for the use of IC: Hospital out- and especially inpatients were not aware of the side effects of their medicines and the potential treatment options, which indicates that they were unable to make an informed decision. The understanding of risks and treatment alternatives is a central requirement for IC, which is now regarded as the core issue of quality in health care (Doyal, 2001). If – according to the strong ethical argument - the standard of IC is applied to the prescription and administration of medicines, it is necessary to improve the information provision to hospital in- and outpatients.

One could argue – as some of the physicians did and some of the patients believed – that, in particular, hospital inpatients have to accept that health care providers are unable to afford the time to inform patients about all the medicines they are receiving during their stay. However, if this is the case, this needs to be explicitly stated to the individual being admitted to hospital enabling him or her to make a decision in the light of this knowledge. In some hospitals in the US, it is standard practice that patients sign a consent form for the medicines which will be administered to them during their stay in hospital (Rich, 2001). This is perhaps a development HCPs in the UK would like to avoid as revealed by the views of the doctors and nurses in this study sample.

Alternatively, a formal process of IC could be introduced for an agreed group of medicines, whereas the rest would be included in the group of medicines for which patients give their permission prior to their admission to hospital. However, it may be difficult to find consensus on the medicines which should be subject to a formal process of IC. HCPs mentioned chemotherapy most commonly as medicines for which they regard a formal process of IC as appropriate. This means that the potential severity, probability and nature of harm would form the basis of the inclusion criteria. In contrast, according to the outpatients' account importance was also placed on medicines, which had impact on their ability to drive or work. This would mean that a more individual approach would be required to define the group of medicines for

which detailed information needs to be provided prior to administration of the medicines.

Apart from the absence of information disclosure, two other requirements of IC were commonly neglected: the patients' understanding and their intended authorisation. Instead, HCPs largely assumed the patients' permission to treat and that the patients' values accord with those of the HCPs. This is in contrast to the patients' accounts which talked about their dependency, vulnerability and feelings of being like a "guinea pig". Further, some patients felt coerced to give permission, which is at variance with the statements of some doctors that they regarded patients as partners. Achieving successful implementation of IC as a minimum required level of patient involvement might start with creating awareness among HCPs of such discrepancy in assumptions.

Implication for the organisation of the doctor-patient consultation: Overall, hospital in- and outpatients and HCPs did not consider IC as an ideal model to support patients in making an informed decision. This shows that there are significant difficulties in its application in practice, which may have implications not only for medicines. It raises the question of what are the minimum requirements of a good prescribing process. Should the aim be that both sides are happy and in agreement as suggested by the model of concordance or that a specific clinical outcome is achieved as suggested by the findings of the present study? Or should it be to put patients in a position to enable them to make an informed decision? How can any of these be achieved in practice? According to the accounts of the HCPs interviewed, one key barrier to supporting the patient to make an informed decision about all their medicines was the time required doing this. However, whether this is a real problem in practice or an excuse for not changing current practices is questionable. Braddock *et al* (1999) argued that the elements of informed decision-making required vary according to the decision complexity which then impacted on the time needed. In case of greater complexity more time would be required in the consultation (e.g. a laboratory test would be a decision of 'basic' complexity whereas a decision to change the dose or initiating a new medicine would be a decision of 'intermediate' and a surgical intervention a decision of 'high' complexity). A review of alternative models of the consultation of hospital inpatients and of ways of information provision within the time available in daily practice could support the development of novel counselling models.

Training and education of HCPs and patients: The reasons why implementing patients' views and their preferences in the choice of medicines is difficult seem to go beyond time issues. The results of this study show that the entire process of prescribing is not designed to support the incorporation of the patient's perspective as well as to offer treatment choices. This is partly due to the beliefs of the physicians and patients that it is the physicians' task to select the treatment and that there is usually only one "right" choice, which means that the process of selection was aimed at determining *one* treatment rather than a *potential range* of options. Deliberation or negotiation was almost absent in most prescribing situations. It was left to the patient to make a decision at home about taking the medicines prescribed or not which means that the decision was made outside the doctor-patient consultation.

Many authors have focused on improving training in communication skills (Hulsman *et al*, 1999). However, as the results of the research presented shows improving communication does not necessarily go along with offering patients therapeutic options and letting them decide about these options if they so desire. For example, some of the doctors interviewed thought that improving communication by sending out clinical letters to patients would replace IC.

This suggests that training of HCPs in identifying and communicating the therapeutic aims, the treatment options and the values involved in the treatment decisions could support the discussion about values during the consultation. The term 'value literacy' has been used to summarise aspects such as being aware, interested in and capable of identifying and discussing values in practising a health profession (Cribb and Barber, 2000). That it is very difficult to be aware of all values involved in a decision process has been discussed by Lelie (2000) who investigated values in decision-making about dialysis in a Dutch Teaching Hospital. Whereas non-medical decisions were easily recognised as value-laden and discussed with patients, values related to medical decisions were not acknowledged. However, more transparency in the way the physician selects a treatment may increase the honesty between physician and patient. "Thinking aloud" of the physician gives the patients the opportunity to obtain information about each step in the decision and potentially contribute with information. It might also give patients the opportunity to protest if the physician makes a wrong assumption.

As mentioned before outpatients regarded decisions about long-term medication and medicines which have impact on their life-style as very important, which means that especially for these decisions an involvement on the part of the patient is desirable. However, this would require that first, HCPs need to be well informed about potential side effects of the treatment in question and second, HCPs would need to elicit specific information about the patient's life-style to be able to tailor the information to the needs of the individual.

It has been pointed out that also patients need training and education in order to be able to make trade-offs or to discuss values and preferences if they wish to exercise choice (Greenfield *et al*, 1985; Cegala *et al*, 2000). Previous work has explored the skills that patients will need to take part in shared decision-making, for example to articulate feelings, expectations and beliefs (Towle *et al*, 1999; Elwyn *et al*, 2000). Decision-aids such as written leaflets, booklets and videos have been one way to support patients in the decision-making about health care interventions and increase the patient's perceived involvement in the decision (O'Connor *et al*, 2003). However, most of the decision-aids identified in the extensive review by O'Connor *et al* (2003) covered decisions about oncology treatment, hormonal replacement therapy, cardio-surgical interventions and some forms of screening and diagnostic testing. Day-to-day prescribing decisions are not covered by such aids. Perhaps a less resource demanding way of supporting patients to contribute to the selection of treatment options needs to be developed.

Implication for policy: If government agencies advocate the involvement of patients in decision-making it would be desirable to offer patients a range of treatment options, so as to give the patient the freedom in choosing their treatment rather forcing them to choose between a single treatment and no treatment. Offering a range of therapies means that on the micro level physicians need to have the experience and the knowledge about the options available. It would also have implications for the macro level in the way that policy-makers should provide access to a variety of therapies for the same condition if available.

6.4 Future research

The findings presented in this thesis suggest various areas in which further research on health policy and individual prescribing decisions can be undertaken.

New methods or different study settings could be used to further investigate health policy and individual decision-making such as observational methods or surveys gathering data from a larger sample size. For example, there is a lack of observational studies investigating decision-making concerning national and regional health policy in the UK. Observation of the NICE appraisal committees and the PCT prescribing committees could give valuable insights into the interaction between policy-makers during such meetings and the arguments they proposed. It could also demonstrate ways in which conflicts are resolved and compromises reached. In terms of individual decision-making about medicines the views of general practitioners about decision-making and the use of IC for medicines in primary care can complement the views of doctors in secondary care as elicited in the present study. The use of questionnaires in a larger sample of hospital in- and outpatients about the benefits, risks and feasibility of IC would improve the knowledge about the impact of IC in practice. Moreover, it would enable one to quantify various aspects and to measure effect size such as satisfaction with the process of obtaining IC.

Secondly, modifying the research agenda and investigating questions derived from the findings presented in this thesis could complement the knowledge of health policy and individual decision-making obtained to date. The results of the interviews with policy-makers and stakeholders showed that informal factors had considerable influence on the decisions made. More research is needed to investigate the type of factors which are thought to be relevant in different health policy setting and the circumstances in which they are believed to result in unfair decisions. One of the main findings from the analysis of individual decision-making was the discrepancy between the assumptions made by doctors and by patients. It is important to investigate whether common knowledge of theses assumptions by both the patient and the doctor could improve communication, create an awareness of values imbedded in the decisions made and eventually support the patients in making trade-offs between treatment options if they wish to do so. Research further exploring the components of a minimum standard of a

good prescribing practice could complement the model of IC or support the development of other decision-making models.

6.5 Conclusions

The key conclusions of the first aim of this thesis, to investigate authoritative decision-making in the NHS about access to medicines by using three drug therapies as an example, are as follows:

- Access to the three drug therapies was limited to a certain group of patients according to the budget available for funding instead of provision of access to all patients who could benefit. This meant to choose expenditure and accordingly, tighter criteria until the funding of the treatment for the number of patients who fulfil the criteria could be afforded.
- Data about benefit, safety and cost implications were used as the main legitimisation for the access criteria chosen, although there were considerable uncertainties about the medical and financial effects of the decisions. These uncertainties were used by stakeholders to lobby in favour of access with an appeal for solidarity and responsibility.
- Personal and organisational factors were commonly hidden behind the technical debate of making decisions including following a certain decision-making process.
- More research is needed to observe the actual decision-making process and to investigate factors which fall outside the commonly used rationales to justify decisions.

The main conclusions of the second aim of the thesis, to investigate in secondary care the HCPs' and the patients' views on decision-making about choices of medicines within the medical consultation, including the use of IC for medicines, are as follows:

- Due to the absence of information about side effects and treatment alternatives for the majority of medicines prescribed, patients were unable to make an informed decision about their medication.
- Since prescribing decisions were viewed as 'technical', physicians with their greater technical expertise were regarded as the main decision-makers.

- Informed consent was not regarded as a model to support patients in making informed decisions.
- Although the physicians described patients as a 'partner' in the decisions, eliciting of the patient preferences and values as well as an implementation of their preferences and values was largely absent, which is in contrast to the decision-making models recently advocated.
- The significant discrepancy in the assumptions of physicians and patients regarding decision-making resulted in misunderstandings and decisions which were not in conformity with patients' values. More transparency in explaining the therapeutic options and their related values could support informed decision-making and the dialogue between doctors and patients.

The key conclusions of the third aim of the thesis, to seek similarities and differences between the factors found to contribute to each type of decision-making investigated before, are as follows:

- On both decision-making levels technical aspects were very much in the foreground of the debate of the decision-makers.
- Procedural safeguards functioned as a legitimisation for the decisions made and undermined the importance of the outcome of the decisions.
- On both levels the full range of potential options of choice was often obscure.
- The decision-making models developed for the health policy and the individual level leave some key factors of decision-making unsolved.

The thesis provides insight into important aspects of health policy and individual decision-making and an informed basis to make suggestions of how to improve practice.

APPENDICES

APPENDIX 4.2.1: RECRUITMENT LETTER FOR THE INTERVIEWS WITH POLICY-MAKERS AND STAKEHOLDERS

Date

Dear

European research about availability of treatments

As you know there is considerable debate among health care professionals, managers and the public about health policy and priority settings in the NHS. One part of this is discussion about decisions regarding the availability of certain treatments and we would value your help in this.

Health policy and priority setting are not only being discussed in Britain. Other European countries are in a similar situation and searching for the best ways in which decisions about health policy and priority setting can be made.

Together with four other European countries (Sweden, the Netherlands, Poland and Italy) a team of British researchers based at the School of Pharmacy and King's College London, University of London are investigating how decisions about access to health care treatments are made and what influences these decisions. The project is coordinated by Dr Hoedemaekers, University of Nijmegen and funded by the government research council in the Netherlands.

We are currently collecting data to help us understand the ways in which decisions about new treatments are made, and as part of this process we would like to interview you. We would be most grateful if you were willing to participate. The interview would last about an hour and will be completely confidential. We might want to use some quotes in our final report but data will be anonymised and access restricted solely to the researchers working on the project.

We would like to contact you by phone in the next couple of days to ask you if you are able and willing to participate. After completion of the project we will send you a summary of the results.

Thank you for your time and consideration.

Yours sincerely

Veronika Wirtz
Research Pharmacist
Centre for Practice and Policy

Professor N Barber
Head,
Department of Practice and Policy

Dr A Cribb
Director
Centre of Public Policy Research

APPENDIX 4.2.2: TOPIC GUIDE FOR THE INTERVIEWS WITH PROFESSIONALS IN PRIMARY CARE

1. Introduction[1)]
Thank interviewee for agreeing to take part
Explain background and aim of the study
Confirm confidentiality (quotes might be used but data will be anonymised)
Ask for permission to tape-record the interview

2. *Questions concerning background of the interviewee*
Could you tell me something about your professional background, your current work and how it is related to decision-making about availability?
Are you personally involved in decision-making or working with people who are involved in decision-making?
What is the role of the people you are working with?
What is the role of the committee your are working in?
Could you explain the decision-making process in general?

3. Historical process of decision-making in the case of - name or drug or drug class -
In our project we are particularly interested in three treatments, two of them are drugs – sildenafil, rivastigmine – and one is a group of drugs – statins. Perhaps you became involved in one of the decisions more than in another.

Using one as an example how did the decision-process look like?

What were the criteria for or against funding?
What was the main criterion identified?

What was the evidence used? How was it weighted?

What were the perceived uncertainties regarding knowledge?
Probe: lack of evidence, less quality of evidence, how were dealt with this situation.
[What were the difficulties in assessment of benefits and harms of the treatment?][2)]

How was the need for treatment defined?
Probe: life prolonging, symptom alleviation, increasing quality of life, division in subgroup of patients according to severity.
Was the potential abuse of (drug name) discussed?
[*Probe: What kind of abuse was discussed? What were mechanisms introduced to prevent abuse?*]

Was there a threshold of cost-effectiveness defined? If yes, what was the threshold?
Probe: threshold per treatment course per year or per overall proportion of budget spent.
Against what was the treatment compared to? What benchmark was used?
How did the costs of other treatment options influence the decision?
Probe: What costs were included in the assessment, which ones excluded?

What were the major areas of disagreement within the committee?
Probe: strong views held by some members, which ones, why did they conflict with other views?
How were the conflicts dealt with?
Where do you see conflicting values/dilemmas in the decision to fund treatment?
[Where there any differences between drug treatment for life-threatening diseases and less severe conditions? What was the difference? What type of conflicts occurred?]
[Did the decision raise any personal questions about rationing and the access to treatment?]

What kind of other influences did the committee experience?
Probe: Influences of the industry, patient groups (e.g. The Impotence Association, Alzheimer's Society, GPs, senior clinicians)? Did the media influence the decision? In which way?
What were the consequences of these influences?

Is there any difference in making an individual treatment decision with a patient or a decision on health policy level? If yes, what are the differences?

What was the impact of the decision for or against funding of - *name of the drug/drug class* - ? [What health care can or cannot be funded because of the decision?]

Were there any differences between the decision regarding *name of the drug/drug class* & other decisions about access to medicines?

3.1 Questions concerning sildenafil (Viagra ®)
What were the consequences of the DoH statement not to prescribe sildenafil in September 1998?
What was used as guiding principles for the decision to fund treatment or not at that time?
Whose opinions might have influenced the action of the committee?
Probe: GP, senior clinicians, other parties.
Did the delay of the governmental final decision have impact? Which kind of impact?
What were the consequences of the Department of Health (DoH) guidelines in January 1999? What were the incentives to follow the guidelines or not?
How transparent was the decision-making process of the DoH for the committee?
What were the positive or negative aspects of the guidelines?
What influences/consequences had the DoH announcement to change Schedule 11 of the NHS Regulations?
Was there the attempt to define general criteria to distinguish between medical treatment and lifestyle drugs?
How much did the principle of 'individual responsibility' influence the decision?
What were potential other values that influenced the decision to fund the treatment for certain patients or not?

3.2 Questions concerning rivastigmine (Exelon ®)
What were the consequences of the absence of national guidelines between 1998 (licensing of the drug) and 2001 (NICE guidelines)?
What was used as guiding principles for the decision to fund treatment or not?
Were there other guidelines in place?

Probe: guidelines developed by local clinicians, other institutions e.g. Wessex Institute for Development and Evaluation.
Beside costs for rivastigmine or alternative drugs were there other costs you took into consideration?
Probe: nursing home costs, care costs outside nursing home
How was age of the majority of the patients taken into account?

What influence/consequences had the NICE guidelines in 2001? What were the incentives to follow NICE guidelines or not?
How transparent was the process of NICE for the committee?
What are the positive or negative aspects of the NICE guidelines?

3.3. Questions specific about statins (simvastatin, pravastatin, atorvastatin, fluvastatin)
How was the funding regulated between licensing of statins and arrival of the SMAC guidelines in 1997?
What was used as guiding principles for the decision to fund treatment or not?
Were there local guidelines?
Probe: guidelines developed by senior clinicians, Trend Institute recommendation.
What influence/consequences had the SMAC guidelines 1998/NSF 2000? What were the incentives to follow SMAC or NSF?
How transparent was the process of SMAC regarding the reasons to decide for or against funding?
What are the positive or negative aspects of the SMAC/NSF guidelines?
What are the options of access to treatment for patients below the threshold for which treatment is not offered within the NHS?
P*robe: promotion of possible treatment on private prescription.*

4. Winding up
We have now covered many issues regarding the decision to fund treatment with *name of the drug or drug class.*
Looking backwards what do you think could be improved about the decision-making process?
[*Probe: Did NICE influence how decisions are made? What do you think how it affected the decision-making? What were the advantages and disadvantages?*]
Where do you see the possible difficulties experienced in the future in making rationing decisions?
Where do you see the potential chances in deciding about availability to contribute to better health care in Britain?

Anything else?

Thank you for your time.

[1] *italic* – Words in italic indicate instructions for the interviewer.
[2] [ ] Questions in brackets indicate changes of the topic guide made during data collection.

APPENDIX 4.2.3: TOPIC GUIDE FOR THE INTERVIEWS WITH PROFESSIONALS IN SECONDARY CARE

1. Introduction[1)]
Thank interviewee for agreeing to take part
Explain background and aim of the study
Confirm confidentiality (quotes might be used but data will be anonymised)
Ask for permission to tape-record the interview

2. Questions concerning the background of the interviewee
Could you tell me how long you have been working as a consultant and how long have you been working in this hospital?
Are you involved in the care of hospital inpatients as well as outpatients?

How is the use of drugs regulated in your hospital?
Who does decide about prescribing guidelines?
Who can prescribe specific drugs?

The researcher in the Netherlands chose three different drug treatments as an example to illustrate some aspects of decision-making: sildenafil, rivastigmine and statins.
Are you involved in prescribing all three treatments?

3. Questions concerning rivastigmine and sildenafil therapy
When did you become first aware that the drug was available and what did happen until you were able to prescribe it for your patients?
What did influence the suggestion to introduce it in the hospital formulary?
How long did it take to introduce?
How did you perceive the time while you could potentially use a drug but the drug was not included in the formulary? Is that a problem? Why or why not?
Do national guidelines influence the availability of treatments? In what way?

What are the criteria under which it can be prescribed?
What did influence the definition of these criteria? *Probe: other than evidence.*
Do you think they are appropriate? *Probe: MMSE score, underlying condition, which causes ED.*
Do you think they are necessary?
[Do they hinder or facilitate decision-making?][2)]
Are there criteria missing in the guidelines? *Probe: factors not or less well defined in the guidelines.*
[Are there exceptional circumstances where guidelines can be overruled?]
Are there patients who could potentially benefit from treatment but are not eligible according to the criteria defined?
What does usually happen in such circumstances?
Are there other conflicts, which arise as a result out of these criteria? *Probe: withdrawal of treatment, assessment of MMSE.*
What does happen if a patient does not fall under the criteria but would like to receive treatment?
Probe: Private treatment, exceptional circumstances.

Does it raise personal questions or conflicts?
What does happen if you see some patients benefiting from treatment but there is not enough evidence to support wider use of the drug?

4. Questions concerning statins therapy
What are the criteria for treatment with statins?
Do you think there are patients who are disadvantaged in terms of access to treatment with statins? Why or why not?
Are there any uncertainties regarding decision-making in prescribing statins? Which ones? What are the consequences? *Probe: safety issues, lack of evidence about efficacy.*

5. General questions
What do you think is the difference between individual decision-making and making decisions on a policy level?
Are there conflicts? Why do you think they occur? How are they solved?
Are there conflicts in being advocate for the patient and at the same time restricted by the hospital drug policy?
What do you think are the influences on decision-making about availability? *Probe: industry, media, politicians.*
Do you think you have influence on the design of guidelines?

Did decision-making change over time? Individual clinical decisions as well as policy decisions?
What do you think changed and why?
Do you think it has improved over time? Why or why not?
Did NICE influence decision-making? In what way?
Are there other bodies outside the hospital, which influence decision-making?

Where do you see potential areas of improvement regarding decision-making about availability of drug treatment?
In your view where do you think policy-makers should focus on in setting priorities?
Where do you see potential difficulties in the future?
How do you think access to treatment should be regulated?

Anything else?

Thank you very much.

[1] *italic* – Words in italic indicate instructions for the interviewer.
[2] [ ] Questions in brackets indicate changes of the topic guide made during data collection.

APPENDIX 4.2.4: TOPIC GUIDE FOR THE INTERVIEWS WITH REPRESENTATIVES FROM THE PHARMACEUTICAL INDUSTRY

1. Introduction[1)]
Thank interviewee for agreeing to take part
Explain background and aim of the study
Confirm confidentiality (quotes might be used but data will be anonymised)
Ask for permission to tape-record the interview

2. Questions concerning the background of the interviewee
What is your role in the company?
Could you tell me something about your professional background?
How long have you been working in public affairs and how long have you been working with this company?
How is your current work related to decision-making about access to treatment in the NHS or outside?

3. Influence of the industry on decision-making about access to treatment
What do you think are factors restricting the access of certain drugs in the NHS?
Probe: Lack of evidence, low budget, disinvestments, safety, 'anti' industry/ science movement, general policy.

How does the company try to influence the decisions made by the government or regional bodies if the drug will be funded by the public health service?
In what ways?
Probe: Price, trial data, promotion, direct negotiation, use of lobbyists.

Does the company have the opportunity to take active part in the decision-making?
Probe: Member of committees, presenting cases in person
What are the ways of interaction between national and regional bodies of decision-making in the NHS and the company?
Probe: NICE, SMAC or the National Specialist Commissioning Advisory Group.

What type of information do you usually provide?
Do the national bodies actively request information?
Does the company have the legal obligation to make all data available?
What kind of information do you think is needed to make a decision about access to drug therapy in the NHS?

Do you think clinical evidence is enough to make a sensible decision?
What type of other factors should be taken into account?

Do you think the restructuring of the NHS under the New Labour government influenced decision-making about access to drug therapies?

What do you think has changed since the establishment of NICE regarding the companies' action to promote the use of drugs?

Do you think it changed the interest in developing certain drugs? *Probe: less interest in developing life-style drugs since they will be unavailable in the NHS.*
Has the way of providing evidence changed or the type of evidence? Are trials designed in certain ways to be more acceptable to authoritative bodies in the NHS?
Probe: design of trials including specific patient groups or economic evaluation.
What do you think are the advantages of NICE and what are the disadvantages?

Does the company have influence on the availability of drugs over the counter?
In what ways?
Probe: Application, time of application, duration of the process, safety of the product.

Are there areas where you think that the access to the drug should be restricted? Which areas? How should access be regulated?
Regarding the restriction of access to treatment: What do you think should be funded by the NHS as essential therapies?

4. Examples of three drug therapies
The researchers in the Netherlands who coordinate the study chose some examples to illustrate decision-making about access to treatment within and outside public funded health services.

4.1 Questions concerning sildenafil (Viagra ®)
What can you tell me about the case?

Did the company know that the government would advice to restrict prescription of Viagra ® on the day it was licensed?
What was the reaction of the company?
Was a direct negotiation between the government and the company about access to Viagra ® possible?

What do you think were factors that influenced the governmental decision to fund the treatment for certain patients and not for others?

The company won the court case against the Minister of Health in May 1999. Do you think the case influenced the decisions which will be made in the future about ED treatment?
What were the consequences of the restricted access to Viagra ® in terms of future development of drugs or the promotion of them?

Do you think Viagra ® will be available over the counter in the near future? What do you think is needed to achieve that it becomes available?
Probe: Data about safety, five-year-period of testing.

Do you think there were any differences in the decision regarding sildenafil than other decisions about access to treatment?
Why? Did the interest of the media play a role?
What were other influencing factors?

Do you think there should be general criteria to distinguish between medical treatment and lifestyle drugs? Should it be taken into account for what purpose the drug is used?

Where do you see conflicting values in the decision to fund treatment?

4.2. Questions concerning statins
There are some criteria laid down in the NSF to guide prescribing of statins (use in primary prevention if the patient has an annual risk of >3% to develop CHD). Do you think the criteria are appropriate? Do you think they are necessary to regulate access? Some argue that statins should become available over the counter. What do you think? How should the safety be assured?
Do you think lowering the price would have effect on the decision about access to treatment?

4.3 Questions concerning rivastigmine (Exelon®)
What can you tell me about the case?

Did your company submit documents to NICE? What happen after submission? What do you think about the decision of NICE? What do you think were the factors influencing the decision of NICE?
Probe: Media, clinicians, professional bodies, patient's organisations.
What was the reaction of the company?
Do you think the decision will be reviewed and perhaps changed?

Do you think there was any difference between the decision made about rivastigmine and other decisions?
What do you think are the consequences of the NICE guidelines for your company?
Do you think the decision has impact on the development and marketing of other products?

5. Winding up
We have now covered many issues. Looking backwards what do you think could be improved in the decision-making about access to certain drugs in the UK?

Where do you see the possible difficulties regarding access to treatment in the future?

Where do you see the potential chances of your company to contribute to better health care in Britain?

Anything else?

Thank you for your time.

[1] *italic* – Words in italic indicate instructions for the interviewer.

APPENDIX 4.2.5: TOPIC GUIDE FOR THE INTERVIEWS WITH LAY REPRESENTATIVES (LR) OR REPRESENTATIVES OF PATIENT ORGANISATIONS (RPO)

1. Introduction[1)]
Thank interviewee for agreeing to take part
Explain background and aim of the study
Confirm confidentiality (quotes might be used but data will be anonymised)
Ask for permission to tape-record the interview

2. Questions about the background of the interviewee

Could you tell me something about your professional background and your current work? How long have you been in the current position?

Is your work related to decision-making about availability of treatments? In what way?

3. Questions for representatives of patient organisations (RPO):
What kind of role does your organisation have in decision-making about availability of certain treatments in the NHS?

Could you explain the process in general? How do you become aware that a specific decision will be made? *Probe: Media, internal informants, government.*

Where do you seek information?

Who does decide about the strategy of involvement?
What types of documents are submitted?
How can you make a 'good case'?

Are there representatives of your organisation present when these decisions are made? *Probe: nationally, regionally, any difference? Have you got direct contact to decision-makers?*
Do you contact drug companies to obtain information?
Do you contact patients?
Do you perform research? What type of research? Why is that important?

How did you perceive the discussion about costs? *Probe: What types of costs were discussed?*

4. Questions for lay representatives (LR):

What is the role of the committee you are/were working in? Could you tell me a little bit about the work of the committee and the number and function of its members?
If yes: Could you explain the decision-making process in general?

What is the role of the LR in the committees you are involved in or you know about? *Probe: Democratic representation of patients or public, observer, advisory role, full member of committees with the right to vote in decisions.*
Who did you represent? *Probe: patients, carers, public, etc*
What role(s) do you think would be appropriate? Why?
How many LRs are involved? What do you think about the number of the LR involved? *Probe: Should there be more? Why?*
What do you think about the degree of involvement, which you have as a LR? What should be the ideal?

Have you been present while decisions about availability of certain treatments have been made? What was your impression of the decision-making in general? Did you expect it to be like this?

Did you receive some training? What kind of training?

What type of information was used to make the decision?
What do you think about the information?
Was it sufficient? Were there things that you wanted to be included which were absent? *Probe: personal experience of the patients and the carers, cost of the care at home, more about the side effect of the treatment.*

How did you perceive the discussion about costs? *Probe: What types of costs were discussed?*

[Where uncertainties about the benefit of the treatment discussed? Were there other areas of uncertainties? *Probe: cost implications, side effects.*][2)]

What types of consequences of the decisions were discussed? What did you think about the consequences of making the treatment available or denying access?

5. Questions for LR and RPO
What other factors were taken into account while making the decision? *Probe: influence from the industry, media, clinicians.*

What do you think were the most important influencing factors?

What is excepted as evidence and what is the difference between the evidence contributed by LR/RPO and the clinical evidence?

[How transparent is the decision-making process?]

What do you think LR/RPO can contribute? *Probe: personal experience, feedback and concerns from other patients, unpublished studies etc.*

What do you think is a 'good' health policy decision?
Where did you experience good examples of involvement?
Where did you come across difficulties?

Were there situations where you voted for restricting the availability of a specific therapy? Could you give me an example for a situation where you had a different opinion from non-LR/ non-RPO? Why was that?

What do you think are the difficulties of being a LR/RPO? What kind of difficulties did you experience?

Did it result in any personal conflicts or questions? What experience did you make? Why do you think there were conflicts? How was dealt with them?

What are the differences between the decisions made nationally and regionally?

We have now covered many issues regarding LR/RPO:

What kind of treatment would you like to see implemented which is rationed in the NHS (nationally or locally) at the moment?

Should there be a basic package of health care, as it is common in some other countries? *Probe: What are the advantages? What are the disadvantages?*

Looking backwards what do you think could be improved about decision-making? *Probe: Number of LR/RPO, training, the weight, which is given to their opinion.*

Where do you see the possible difficulties in the future in making rationing decisions?

Where do you see the potential chances for LR/RPO in deciding about availability to contribute to better health care in Britain?

Anything else?

Thank you for your time.

[1] *italic* – Words in italic indicate instructions for the interviewer.
[2] [ ] Questions in brackets indicate changes of the topic guide made during data collection.

APPENDIX 4.3.1: TRANSCRIBING CONVENTION

Sign and Abbreviation	**Meaning**
...	Pause of the interviewee
Italics	Emphasis If it is the emphasis of the author of the thesis it is stated at the bottom of the quote in brackets, e.g. [my emphasis]
(---)	Words unclear
[ ]	Words in brackets added by the researcher
[...]	Sentence or more taken out which was regarded as irrelevant for the quotation

APPENDIX 5.2.1: INFORMATION SHEET FOR RESEARCH PARTICIPANTS RECRUITED ON THE WARDS

Informed consent for medicines

You are being invited to take part in a research study. Before you decide it is important to understand why the research is being done and what it will involve. Please take time to read the following information carefully. Ask if there is anything that is not clear or if you would like more information.

What is the purpose of the study?
You are usually asked to sign an 'informed consent' form before you have an operation or before you take part in research studies such as this one. This is a record of you having agreed to allow the doctor to do the surgery or to take part in a research study. For routinely used medicine you are not, at present, invited to sign an informed consent form. This study will help us to find out if asking patients to give informed consent for medicines is a better way for us to tell patients about their medicines, and to involve them more in their care. This study will take place over a period of six months at XX[1)] Hospital.

Why have I been chosen?
You have been chosen because you are an inpatient at XX[1)] Hospital, cared for by a consultant who supports this study, and you have been newly prescribed one of the following drugs: amiodarone, ciclosporin, digoxin or warfarin. We plan to include around 200 patients in the study.

Do I have to take part?
It is up to you to decide whether or not to take part. If you do decide to take part you will be given this information sheet to keep and be asked to sign a consent form. If you decide to take part you are still free to withdraw at any time, without giving a reason. A decision to withdraw at any time, or a decision not to take part, will not affect the standard of care you receive.

What will happen to me if I take part?
[You will be asked to fill in two questionnaires. One will be given to you two days after starting your new medication, the other questionnaire will be sent to your home two to four weeks after your discharge from hospital. The questions on the forms will ask you about your medicines, your wishes for information, your satisfaction with the information you have received, and how often you are taking your medicines. No name will be on the questionnaires; no one outside the research team will know your identity.]

You may also be one of 10 to 20 patients who will be asked to give an interview to the research pharmacist. The pharmacist will ask you in more detail for your opinion about the information you have regarding your medicines. You can participate in the study without giving an interview to the research pharmacist if you prefer.

Since we do not know which is the best way of informing patients about their medicines, and involving them in their care, we will use the results of the interviews to

prepare information to help us offer informed consent to future patients. We will then find out what patients thought about having been given this extra information.

What do I have to do?
[Patients who agree to take part will be asked to fill in the two questionnaires. A smaller number of patients will be invited to give an interview to the researcher. However, you can participate in the study without giving an interview if you prefer.

It will take you around 10 to 20 minutes to fill in the questionnaire you receive in hospital. The second one sent to your home will take a couple of minutes to fill in.]

Only those patients who also agree to give an interview to the researcher will be asked in more detail for their opinion about medicines and the information they received. These participants will have an interview normally lasting 30-40 minutes. We would like to tape-record this, and will ask your permission to do so, however you can give the interview without being taped if you like. If your interview is tape-recorded, you will have the opportunity to listen to the tape and to have it erased if you wish. After the study is finished, all tapes will be destroyed.

What is the procedure that is being tested?
The study will test whether improving patients' understanding of their medicines is better for patients than current practice.

What are the possible disadvantages and risks of taking part?
There are no clinical risks for you in taking part in this study. However, you may not wish to answer all the questions on the forms or in the interview, and you have the right not to answer these questions.

What are the possible benefits of taking part?
There may be no direct benefit for you. The aim of the study is to find a better way to inform patients about their medicines. With your participation you might help to improve the way we inform patients about their medicines in the future.

Will my taking part in this study be kept confidential?
[The information on the questionnaires will be kept strictly confidential. No name will appear on the questionnaires and no one will know your identity. Your GP will be informed about the study, its purpose and will receive copies of the questionnaires (but will not know your answers).] If you have been interviewed and tape-recorded, the contents of the tape will be typed out, however you will only be identified by a code. When we write up the research in scientific journals we may use quotes from your interview, but they will be anonymous. All the information from the study will be stored at the University of London and only the researchers will have access.

What will happen to the results of the research study?
The results will be used to improve the way patients in hospital are informed about their medicines. They will also help us understand if giving informed consent allows patients to be more involved in their care. The results of the study will be published in the future. If you wish to receive a copy of the results, please contact one of the researchers at the address below.

Who is organising and funding the research?
Researchers from the XX[1)] Hospitals NHS Trust and from the School of Pharmacy, University of London, will organise the study. Both institutions are funding the research.

Who has reviewed the study?
The Research Ethics Committee XX[1)] Hospital (Project Registration Number: YY[1)]).

Contact for Further Information
For further information you can contact XY[1)] Pharmacist at XX[1)] hospital, bleep ZZ[1)] or phone ZZ[1)] or contact Veronika Wirtz, one of the research pharmacists at the School of Pharmacy, University of London: 020 7753 5956. Address: Centre for Practice and Policy, 29/39 Brunswick Square, London WC1N 1AX.

You will be given a copy of the information sheet and a signed consent form to keep.

Thank you for your time.

[1)] Data omitted for reason of confidentiality and anonymity of the study site.

[ ] The data from the questionnaires will not be presented in the thesis.

APPENDIX 5.2.2: RECRUITMENT LETTER SENT TO CARDIOLOGY AND UROLOGY OUTPATIENTS

School crest XX[1)] Hospitals NHS Trust sign

Dear Mr/Mrs/Miss/Ms

Re: Study of decision-making about choices of medicines

You are being invited to take part in a research study and we would like to ask you if you are interested in participating.

Decision-making about medicines is one of the most common decisions that doctors and patients have to make. However, it is not clear how patients can best be supported to make decisions about their medicines. We would like to understand the way in which decisions about medicines are made, and find out what patients and doctors think is influencing them. We would therefore like to ask patients and doctors about their opinions.

We have enclosed an information sheet about the study and a consent form to bring along with you when you come to your outpatient appointment. We will ask you whether you would like to take part when you attend for your outpatient appointment. Please do not hesitate to contact us if you have any questions.

It is up to you whether or not you chose to take part, and your decision will not affect the medical care that you receive.

Thank you for your time.

Yours sincerely

Veronika Wirtz

Research Pharmacist
Dept for Practice and Policy
School of Pharmacy
University of London
29/39 Brunswick Square
London WC1N 1AX
Tel: 020 7753 5963

XY[1)]

Pharmacist
Pharmacy Department
XX Hospital

Tel: ZZ[1)]

The School of Pharmacy
University of London

Pharmacy
XX[1)] Hospital

Information sheet

Decision-making about choices of medicines
You are being invited to take part in a research study. Before you decide it is important to understand why the research is being done and what it will involve. Please take time to read the following information carefully. Ask if there is anything that is not clear or if you would like more information.

What is the purpose of the study?
Decision-making about medicines is one of the most common treatment decisions that doctors and patients have to make. However, it is not clear how patients can best be supported to make decisions about their medicines and what is important to them. We would like to understand the way in which decisions about medicines are made and find out what patients and doctors think is influencing them. We would therefore like to ask patients and doctors about their opinion and views.

Why have I been chosen?
You have been chosen because you are a cardiology or an urology outpatient at XX[1)] Hospital, cared for by a consultant who supports this study. We plan to include around 30 patients and 10 doctors in the study. This study will take place over a period of three months at XX[1)] Hospital.

Do I have to take part?
It is up to you to decide whether or not to take part. If you do decide to take part you will be given this information sheet to keep and be asked to sign a consent form. If you decide to take part you are still free to withdraw at any time, without giving a reason. A decision to withdraw at any time, or a decision not to take part, will not affect the standard of care you receive.

What will happen to me if I take part?
You will be asked to give an interview to the research pharmacist. The pharmacist will ask you about your views on making decisions about your medicines, how you usually make decisions and what you think is the best way to make a decision.

What do I have to do?
The patients who agree to take part will be interviewed by a research pharmacist at a time and place convenient for you. The interview will last for about 30-40 minutes.

We would like to tape-record the interview, and we will ask you for your permission to do so. However you can give the interview without being taped if you like. If your interview is tape-recorded, you will have the opportunity to listen to the tape and to have it erased if you wish. After the study is finished, all tapes will be destroyed.

What is the procedure that is being tested?
The study will find out how patients and doctors think about decision-making about medicines, and what they regard as important in making decisions about treatment with medicines.

What are the possible disadvantages and risks of taking part?
There are no clinical risks for you in taking part in this study. However, you may not wish to answer all the questions in the interview, and you are free to answer only the questions that you want to answer.

What are the possible benefits of taking part?
There may be no direct benefit for you. The aim of the study is to find a better way for patients and doctors to make decisions about medicines. With your participation you might help to improve the way patients and doctors decide about medicines in the future.

Will my taking part in this study be kept confidential?
The contents of the interviews will be typed out. No name will appear on the tapes or typed interview. No one will know your identity and you will only be identified by a code. When we write up the research in scientific journals we may use quotes from your interview, but they will be anonymous. All the information from the study will be stored at the University of London and only the researchers will have access.

What will happen to the results of the research study?
The results will be used to improve the way in which patients and doctors make decisions about medicines. They will also help us understand how patients can be involved in decisions about their medicines. The results of the study will be published in the future. If you wish to receive a copy of the results, please contact one of the researchers at the address below.

Who is organising and funding the research?
Researchers from the XX[1)] Hospitals NHS Trust and from the School of Pharmacy, University of London, will organise the study. The University of London is funding the research.

Who has reviewed the study?
The Research Ethics Committee at XX[1)] Hospital (Project Registration Number: RREC ZZ[1)]).

Contact for further information
For further information you can contact Veronika Wirtz, one of the research pharmacists at the School of Pharmacy, University of London: 020 7753 5963. Address: Centre for Practice and Policy, 29/39 Brunswick Square, London WC1N 1AX or contact XY[1)] pharmacist at XX[1)] hospital.

You will be given a copy of the information sheet and a signed consent form to keep.
- Thank you for your time. -

[1)] Data omitted for reasons of confidentiality and anonymity of the study site

Pharmacy Department

RESEARCH CONSENT FORM

Title of Project: Decision-making about choices of medicines

(The patient/volunteer should complete the whole of this sheet him/herself)

Have you read the Information Sheet?	Yes	No
Have you had the opportunity to ask questions and discuss the study?	Yes	No
Have you received satisfactory answers to all of your questions?	Yes	No
Have you received enough information about the study?	Yes	No
Whom have you spoken to? (write name)		
Do you understand that you are free to withdraw from the study, at any time, without having to give a reason, and without affecting the quality of your present or future medical care?	Yes	No
Do you agree to take part in this study?	Yes	No

I understand that the Local Ethics Committee may review this form as part of a monitoring process.

NAME IN BLOCK LETTERS:

Signature: Date:

SIGNATURE OF PERSON OBTAINING CONSENT Ms Veronika Wirtz

Signature: Date:

APPENDIX 5.2.3: TOPIC GUIDE FOR THE INTERVIEWS WITH HOSPITAL INPATIENTS

Hello, My name is Veronika Wirtz, and I am a researcher in the study into informed consent (IC) for medicines. You have been given information about the study and said you would be prepared to take part – are you still happy to take part?

I would like to ask you a few questions about medicines; this will probably take 30-40 minutes – is that OK? I find it helps me if I can record these conversations, so it allows me to concentrate on what you are saying instead of having to try and make notes while you are talking. The tapes would be anonymous and held in a secure place. Is it OK for me to record you, or would you prefer I did not? It is up to you.

When I have analysed these interviews I may use some quotes from them in a report, but it would be done in such a way that you could not be identified. Is that OK?

First, I'd like to know a bit about you – why have you come into hospital?

What medicines where you taking before you came into hospital?
Prompt from drug chart.[1)]

Tell me a bit about how you have got on with your medicines before you came into hospital –
Do you know what they all did?
Do you think they all worked?
Did any seem more important to you than any others? If so, why?
What do you think were the benefits of each medicine?
Did you know of any risks that they had, such as that they may give you side effects, or may not work?
Where did you get the information about your medicines?
Probe: from more than one source.

Many people adjust the dose, or stop taking some of their medicines. Did you do that for any of yours?
Probe: which, why and how.

Since you have come into hospital, do you know if there are any other medicines that you have been prescribed?
Check with chart, include IVs etc. Record which ones known about/not known about.
If yes – probe to see if know actions, benefits and risks of each.

Let's look at *name of the newly prescribed medicine* in more depth. Can you tell me anything you know about (*name of the medicine*)? How often you take it, why you should take it, what it does, if there are any risks [anything which can happen when you take the medicine][1)], etc.?
Keep probing 'anything else'.

How did you get to know these things?

Is there any more information you would like about this medicine?
Do you know what would happen if you choose not take the medicine at all?
Do you know if there are any alternatives to it?
Do you feel you had any choice in the decision that you have this medicine?

There is a wide range of views that people have on whether they should be involved in choosing the medicines they have, or even whether they need a medicine or not. Some people trust the doctor and leave the decision to them; others want as much information as possible and to make the decision themselves. Where are you on that range of views?

Probe with respect to understanding and to control of the decision.

[Before you undergo an operation the doctor usually explains the operation, the benefit and risks to you. At the end you need to sign a form saying that you received the information, understood the relevant facts and that you want to receive surgery. What do you think about that? Do you think it is necessary? Why? Do you have any concerns about it? What do you think are the benefits and the risks?]

We are going to study a new method of giving patients information about certain medicines such as (*name the newly prescribed medicine*). You would receive some written information and then a pharmacist would come and check whether you had any questions and that you understood the risks and benefits of the drug and the alternatives to taking it. When you were happy that you understood what the drug would do, you would be asked whether you wanted to take it or not. If you did not, an alternative, or no therapy could be given, depending what you wanted. The final decision would be yours.
What do you think about this idea?
Is any of it attractive to you – if so, what and why?
Do you have any concerns about the idea – if so what and why?
What would be the best thing to do from your perspective?

Anything else?

Thank you. Finally, can I ask you a few details about yourself?

When were you born?
How old were you when you left full time education?
Have you done any further qualifications since then? *Probe, if yes.*
How would you describe your ethnic group?

Thank you very much.

[1] *italic* – Words in italic indicate instructions for the interviewer.
[2] [ ] Questions in brackets indicate changes made to the topic guide during the study.

APPENDIX 5.2.4: TOPIC GUIDE FOR THE INTERVIEWS WITH HEALTH CARE PROFESSIONALS RECRUITED ON THE WARD

My name is Veronika Wirtz, and I am a researcher in the study into informed consent (IC) for medicines. As part of this we wish to know what a group of health service staff thinks about the idea. I'd like to ask you some questions about IC – this will probably take 20-30 minutes, is that OK?

I'd also like to record our conversation so that I can concentrate on your answers rather than on making notes – is that OK? You will not be identified in any write up of the findings; when reporting our findings we may use quotes from what you have said, however there will be no way you can be identified from these. Is that OK?

What do you understand IC to mean?

Probe: e.g. understanding, competence, formal decision-making, patient involvement, risk sharing, legal issues.[1)]

Where have you come across its use?

Seek examples.

Why do you think it is done?

What do you think of it as a process?

Probe: 'competence' and 'voluntary action of the patient.'

How could it be improved?

Probe: examples of good and bad practice, difficulties in obtaining IC.

What do you think its effects are on the patient?

We are going to conduct a trial of getting patients to give IC before taking certain medicines. This interview is part of our preparation, trying to design the best process.

What, if any, do you think are the reasons for having IC for medicines?

We are trying IC on four drugs first – amiodarone, ciclosporin, digoxin and warfarin.

What do you think will be the practical problems in giving IC for medicines?

How do you think it could be made to work on your ward(s)?

Probe: information presentation, time to consider giving IC, presents of relatives.

What do you think would specifically important to tell the patients about their medicines?

Why do you think this information would be important?

What do you think would be the implications for staff and patients of introducing IC for medicines?

People have suggested the following benefits of IC for medicines. Please tell me what you think about each of them:

It formally involves patients in the decision-making process about their own treatment.

It makes the patient accept the risk of therapy.

It provides the hospital with legal cover if the patient is harmed by the drug.

It is a way of carefully informing patient about benefits and risks of the new medicines and also the alternatives.

When the patient gets home they are more likely to take their medicines if they consented to them.

Do you think patients want to be involved in the choice of which medicines they take?
Probe: for examples of cases where would and where would not.

Some patients told me that they do not want to make the final decision. Even so, do you think it would be important to inform them about risk and benefit of the treatment as well as alternatives?
Probe: why do they give this answer.

Do you think all patients should give IC for some medicines?
Probe: why they give their answer.
For which medicines do you think it would be particularly useful?
If IC for medicines were introduced into routine practice, who do you think should obtain IC? Do you think there would be any training requirements for that person?

To finish, having now explored several aspects of IC: Do you think IC for at least some medicines would have any benefits - to the patient, to you and to the organisation?

Do you think there are risks associated with IC for medicines – to the patient, to you and to the organisation?

Anything else?

Finally, can I ask you a little about yourself?
What is your grade?
How old are you?
When did you qualify?
How long have you been in your current position? How long have you been on this ward?

Thank you very much.

[1)] *italic* - Words in italic indicate instructions for the interviewer.

APPENDIX 5.2.5: TOPIC GUIDE FOR THE INTERVIEWS WITH CARDIOLOGY AND UROLOGY OUTPATIENTS

1. Introduction[1)]
Thank interviewee for agreeing to take part
Explain background and aim of the study
Confirm confidentiality (quotes might be used but data will be anonymised)
Ask for permission to tape-record the interview

2. Questions
Could you tell me what type of medicines you are taking at the moment?
Do you think they are effective? What makes you think so?
Do you have any problems with them?
Do you know what would happen if you do not take them?
Did that ever happen to you?

There are several ways how doctors and patients can decide about prescribing medicines. What do you think is the best way? Why? Does it depend on the situation how it should be made?

What do you think would be necessary to make a decision about your medicine? *Probe: type of information, written information, advice, explanation, time to consider, friends to be present while decisions are made.*

What type of information do you think the doctor uses to decide which medicine to prescribe? *Probe: how the medicine works, the side effects, the patient's opinion etc.*

What do you think would be important for the doctor to know when he/she prescribes a medicine for you?

What would be important for you to know from him/her to make a decision if you take the medicine or not? What are the important factors for you to decide about your medicines? Under what circumstances do you think you would not take the medicines?

What information should the doctor use to make a decision about prescribing the medicine? *Probe: how effective the medicine is, information about side effects, information about the costs of the medicine, your experience of the illness.*

Some medicines are not paid by the NHS. Should the doctor mention all the alternatives including the medicines, which are not funded? Did that ever happen to you that the doctor mentioned a medicine, which you needed to obtain privately?

When the doctor tells you that he/she is thinking about prescribing a medicine would you like that he/she is asking you what you think? Why or why not?

Sometimes the doctor prescribes a medicine and the patient decides at home if he/she does want or doesn't want to take the medicine. In other cases the doctor and the patient talk in the consultation about their thoughts about the medicine.

Who do you think should make a decision about the choice of your medicine? Who do you think has the responsibility for the decision? Would you like to discuss the medicines with your doctor?

Should the reasons for the decision been discussed between patient and doctor? Why or why not?

Some people say that it is important that you make an informed decision about your medicine. What do you think is an informed decision? Would that be important for you to make up your mind about the medicines if you take it or not? How do you think that could be achieved?

Do you think it is important that you and your doctor agree on the treatment choice? Why or why not? What would happen if you think different about the choice of medicine than your doctor?

In some cases – for example when you have an operation – the doctor explains all relevant information to you (risks, benefit, alternatives) and ask you if you decide to undergo surgery or not. Sometimes you need to sign a consent form or verbally stating that you want the operation and that you received all the relevant information. Why do you think that is done? What do you think are the advantage? What are the risks? Do you think it is important?

What do you think about introducing this for medicine: the doctor would explain all the information (risks, benefits, alternatives) about your medicine and you would give your final 'yes' or 'no'?
Have you ever given informed consent for a medicine? If yes, how do you think about it? *Probe: advantages, disadvantages, etc.*

Do you think there is a difference between seeing your GP and seeing a doctor here in the outpatient clinic regarding your medicines? *If yes, probe: why?*

What do you think 'involvement in decision-making' would mean? What kind of involvement do you think of? What would be the benefits? What would be the risks? What type of involvement would you prefer?

Anything else?

3. Questions about personal details of the interviewee
Finally, can I ask you a little about yourself?
How old you are?
When did you leave full-time education? What is/was your profession?
How would you describe your ethnic background?
How would you rate your current health status according to the following scale (Euro-QoL)?
How would you describe you current health status according to the following questions?
Thank you for your time.

[1] *italic* - Words in italic indicate instructions for the interviewer.

APPENDIX 5.2.6: TOPIC GUIDE FOR THE INTERVIEWS WITH DOCTORS WORKING IN THE OUTPATIENT CLINICS

1. Introduction[1)]
Thank interviewee for agreeing to take part
Explain background and aim of the study
Confirm confidentiality (quotes might be used but data will be anonymised)
Ask for permission to tape-record the interview

2. General questions about prescribing decisions
How would you define 'good' decision-making about medicines?
What is the aim of decision-making about the choice of medicines?
Why do you think this is important or not?

Who should make a decision about the choice of medicines? *Probe: The patient? The doctor? Both? Why do you think so?*
When should the decision be made: during the consultation or the physician prescribes and the patient should decide afterwards, or ideally both together at the same time?
What do you think is informed decision-making? Is that important for you or for the patient? How could that be achieved?

You are prescribing for hospital inpatients and for outpatients. What do you think are the differences regarding the decision-making?

3. Questions regarding information provision
What type of information do you think is important for you to make a decision about the choice of the medicine? *Probe: Evidence of efficacy, cost-effectiveness, the medical history of the patient.*
What type of information do you think is important for the patient? Why do you think so? Does it depend if the patient is an inpatient or an outpatient?
What happens if they are different treatment alternatives? What information should be given to the patient? Do you think it is important to discuss why certain treatments are not available? Why do you think so?
In what circumstances did you experience conflicts of interests? What happened and how was it solved? Is there a difference between hospital inpatients and outpatients?

4. Questions regarding the decision-making process
What do you think would be important for you to take into account to decide, which medicine is best for the patient? Do you need to balance that against other factors? What are these factors?
Do you think the patient expects you to tell him/her what you think? Would you like that the patient tells you what he/she thinks?
What do you think about discussing the reasoning for or against the choice of a medicine? What do you think are the benefits and the risks?
What do you think you can or should do to support the patient to make a decision about their medicines?
Is there a difference between hospital inpatients and outpatients regarding discussing the prescription? What is your experience?

5. Questions regarding the role of the doctor and the patient in the decision-making process
Who do you think should make the final choice what medicine to use for treatment?
Would that be different for hospital inpatients than for outpatients?

Is it important that the doctor and the patient agree during the consultation about the choice of medicine? What would happen if they do not agree with each other?
Do you make compromises between what the patient wants and what you want? When do you think you can or should prescribe a medicine which it is not your first choice for this patient?
Does it raise conflicts? How do you deal with them?

6. Questions regarding informed consent
There are various models of ideal types of decision-making:
One type – informed consent – is typically used in surgery. There is some discussion how far informed consent - written, verbal or implied - can be used for medicines.
What do you think about it? What do you think are the benefits and the risks?

What do you think could be improved about decision-making regarding medicines and prescribing?

Anything else?

7. Questions about personal details of the interviewee:
Finally, can I ask you a little about yourself?
How old you are?
When did you qualify?
What is your grade?
Since when have you been in your current position?

Thank you for your time.

[1] *italic* - Words in italic indicate instructions for the interviewer.

APPENDIX 5.2.7: EURO-QOL EQ-5D QUESTIONNAIRE

Euro-QoL Description

By placing a tick in one box in each group below, please indicate which statement best describes your own health status today.

Mobility

I have no problems in walking about ☐
I have some problems in walking about ☐
I am confined to bed ☐

Self-care

I have no problems with self-care ☐
I have some problems washing and dressing myself ☐
I am unable to wash and dress myself ☐

Usual activities (e.g. work, study, housework, family or leisure activities)

I have no problems with performing my usual activities ☐
I have some problems with performing my usual activities ☐
I am unable to perform my usual activities ☐

Pain/discomfort

I have no pain or discomfort ☐
I have moderate pain or discomfort ☐
I have extreme pain or discomfort ☐

Anxiety

I am not anxious or depressed ☐
I am moderately anxious or depressed ☐
I am extremely anxious or depressed ☐

Euro-Qol rating

To help people say how good or bad a health status is, we have drawn a scale on which the best state you can imagine is marked 100 and the worst state you can imagine is marked 0.
We would like you to indicate on this scale how good or bad your own health is today, in your opinion.
(*Drawing of a scale from 0 to 100*)

LIST OF REFERENCES:

Abraham J (1995). Partical progress? The development of American and British drug regulation. In: *Science, politics and the pharmaceutical industry. Controversy and bias in drug regulation.* London: UCL Press.

Adams RJ, Smith BJ and Ruffin RE (2001). Impact of the physician's participatory style in asthma outcomes and patient satisfaction. *Annals of Allergy Asthma and Immunology* **86**, 263-271.

Agid Y, Dubois B, Anand R and Gharabawi G (1998). Efficacy and tolerability of rivastigmine in patients with dementia of the Alzheimer type. *Current Therapeutic Research, Clinical and Experimental* **59**, 837-845.

Alderson P and Goodey C (1998). Theories in health care: Theories of consent. *British Medical Journal* **313**, 1313-1315.

Aldridge J and Measham F (1999). Sildenafil (Viagra ®) is used as a recreational drug in England. *British Medical Journal* **318**, 669.

Altman IL, Wheeler R and Avery J (2002). Self-administration of medicines in Brighton. *Hospital pharmacist* **9**, 305-307.

Alzheimer's Society (2000). *Appraisal of the drugs for Alzheimer's disease. Submission to the National Institute for Clinical Excellence (NICE).* London: Alzheimer's Society.

Andalo D (2002). Why the government accepts the hit and miss nature of prescription charges. *Pharmaceutical Journal* **268**, 833-834.

Annas G (1998). Introduction. In: *Some choice. Law, medicine and the market.* New York: Oxford University Press.

Armstrong K, Schwartz JS and Asch D (1999). Direct sale of sildenafil (Viagra ®) to consumers over the Internet. *New England Journal of Medicine* **341**, 1389-1392.

Arora NK and McHorney CA (2000). Patient preferences for medical decision making: who really wants to participate? *Medical Care* **38**, 335-341.

Ashton T, Cumming J and Devlin N (2000). Priority-setting in New Zealand: translating principles into practice. *Journal of Health Service Research and Policy* **5**, 170-175.

Ashworth M, Golding S, Shephard L and Majeed A (2002). Prescribing incentive schemes in two NHS regions: cross sectional survey. *British Medical Journal* **324**, 1187-1188.

Association of British Pharmaceutical Industry (ABPI) (2000). *Pharma, Facts and Figures. How the pharmaceutical industry contributes to the health and wealth of the nation.* London: ABPI.

Association of British Pharmaceutical Industry (ABPI) (2002). *Information and statistics.* http://www.abpi.org.uk/ (accessed in August 2002).

Association of the British Pharmaceutical Industry (2003). *The NHS expenditure per person in the UK.* http://www.abpi.org.uk/statistics/ (accessed May 2002).

Aveyard H (2001). *Does informed consent theory inform nursing practice? An exploration of the application of informed consent prior to nursing care procedures.* Thesis. London: University of London.

Aveyard H (2002). Implied consent prior to nursing care procedures. *Journal of Advanced Nursing* **39**, 201-207.

Balint M (1964). *The doctor, his patient and the illness.* London: Pitman Medical.

Banta D (2003). The development of health technology assessment. *Health Policy* **63**, 121-132.

Barnes DM (1998). Informed consent in a multicultural cancer patient population: implications of nursing practice. *Nursing Ethics* **5**, 412-423.

Barnes JC and Thomas J (1998). Participation and influence of individuals and committees that decide on drug availability in HMOs. *American Journal of Health-System Pharmacy* **44**, 56-61.

Barnett D (2002). *Memorandum. Select Committee on Health.* www.parliament.the-stationery-office.co.uk (accessed June 2003).

Barrett S and McMahon L (1990). Public management in uncertainty: a micro-political perspective of the health service in the United Kingdom. *Policy and Politics* **18**, 257-268.

Barry CA, Bradley CP, Britten N, Stevenson FA and Barber N (2000). Patients' unvoiced agendas in general practice consultations: qualitative study. *British Medical Journal* **320**, 1246-1250.

Baxter C, Jones R and Corr L (1998). Time trend analysis and variations in prescribing lipid-lowering drugs in general practice. *British Medical Journal* **317**, 1134-1135.

Bayer T (1999). Commentary: Another piece of the Alzheimer's jigsaw. *British Medical Journal* **318**, 633-640.

Beauchamp TL and Childress JF (1994). Respect for autonomy. In: *Principles of biomedical ethics.* 4th edition. New York: Oxford University Press.

Becker MH and Maiman LA (1975). Sociobehavioral determinants of compliance with health and medical care recommendations. *Medical Care* **13**, 10-24.

Beisecker AE and Beisecker TD (1990). Patient information-seeking behaviors when communicating with doctors. *Medical Care* **28**, 19-28.

Bellingham C (2002). Overhaul of POM-to-P reclassification could mean big changes for pharmacy. *Pharmaceutical Journal* **268**, 131-132.

Bellinghiere EG, Santoro D, Lo Forti B, Mallamace A, De Santo RM and Savica V (2001). Erectile dysfunction in uremic dialysis patients: diagnostic evaluation in the sildenafil era. *American Journal of Kidney Disease* **38** (Suppl 1), S115-S117.

Benbassat J, Pilpel D and Tidhar M (1998). Patients' preferences for participation in clinical decision making; a review of published surveys. *Behaviour Medicine* **24**, 81-88.

Bernfort L (2003). Decisions on inclusion in the Swedish basic health care package – roles of cost-effectiveness and need. *Health Care Analysis* **11**, 301-308.

Berwick D, Davidoff R, Hiatt H and Smith R (2002). Refining and implementing the Tavistock principles for everybody in health care. In: New B and Newberger J (eds). *Hidden Assets. Values and decision-making in the NHS.* London: King's Fund.

Biggs H and Mackenzie R (2000). Where is the boundary between clinical and social need for Viagra ®? *Journal of Health Service Research and Policy* **5**, 64.

Blanchard CG, Ruckdeschle JC, Blanchard EB, Avena JG, Saunders NL and Malloy ED (1983). Interactions between oncologists and patients during rounds. *Annals of Internal Medicine* **99**, 694-699.

Blanchard CG, Labrecque MS and Ruckdeschel JC (1988). Information and decision-making preferences of hospitalised adult cancer patients. *Social Science and Medicine* **27**, 1139-1145.

Bloom BS (1986). Controlled studies in measuring the efficacy of medical care: a historical perspective. *International Journal of Technology Assessment in Health Care* **2**, 299-310.

Bochner F, Martin E, Burgess N, Somogyi A and Misan G (1994). Controversies in treatment: How can hospitals ration drugs? *British Medical Journal* **308**, 901-8.

Bodenheimer T (1997). The Oregon health plan – lessons for the nation. First part. *New England Journal of Medicine* **337**, 651-656.

Bosanquest N (1999). Dobson decision fuels drugs debate. *British Journal of Health Care Management* **5**, 65.

Boseley S (1999). Dobson bows to reality of rationing. *Guardian*, Friday 22nd January.

Bowling A (1999a). Qualitative and combined research methods, and their analysis. In: *Research methods in health. Investigating health and health services.* 3rd edition. Buckingham: Open University Press.

Bowling A (1999b). Unstructured interviewing and focus groups. In: *Research methods in health. Investigating health and health services.* 3rd edition. Buckingham: Open University Press.

Bowling A and Ebrahim S (2001). Measuring patient's preferences for treatment and perceptions of risk. *Quality in Health Care* **10** (Suppl1), i2-i8.

Brabbins C, Butler J and Pentall R (1996). Consent to neuroleptic medication for schizophrenia: clinical, ethical and legal issues. *British Journal of Psychiatry* **168**, 540-544.

Braddock CH (1998). Advancing the cause of informed consent: Moving from disclosure to understanding. *American Journal of Medicine* **105**, 354-355.

Braddock CH, Kelly AE, Hasenberg NM, Laidley TL and Levinson W (1999). Informed decision making in outpatient practice, time to get back to basics. *Journal of American Medical Association* **282**, 2313-2320.

Bradley CP (1992). Uncomfortable prescribing decisions: A critical incident study. *British Medical Journal* **304**, 294-296.

Bradley JG, Zia MJ and Hamilton N (1996). Patient preferences for control in medical decision-making: a scenario-based approach. *Family Medicine* **28**, 496-501.

Bradshaw N and Walker R (1997) "Equity of prescribing: a pharmaceutical issue?" *Pharmaceutical Journal* **259**, (Suppl) R32.

Brearly S (1990). *Patient participation. The literature.* London: Scutari Press.

Brenner J, Glynn RJ, Mogun H, Neumann PJ, Weinstein MC and Avorn J (2002). Long-term persistence in use of statin therapy in elderly patients. *Journal of the American Medical Association* **288**, 455-461.

Brindle P, Emberson J, Lampe F, Walker M, Whincup P, Fahey T and Ebrahim S (2003). Predictive accuracy of the Framingham coronary risk score in British men: prospective cohort study. *British Medical Journal* **327**, 1267-1272.

British Cardiac Society, British Hyperlipidaemia Association, British Hypertension Society, endorsed by the British Diabetic Association (1998). Joint British

recommendations on prevention of coronary heart disease in clinical practice. *Heart* **80** (supplement 2) S1-S29.

British Medical Association (1999). *General Practitioners Committee Viagra statement.* www.bma.org.uk/pressrel.nsf/ (accessed Feb 2002).

British Medical Association and Law Society (1995). *Report: assessment of mental capacity—guidance for doctors and lawyers.* London: BMA.

Britten N (2001). Prescribing and the defence of clinical autonomy. *Sociology of Health and Illness* **23**, 478-496.

Britten N, Stevenson FA, Barry CA, Barber N and Bradley CP (2000). Misunderstandings in prescribing decisions in general practice; qualitative study. *British Medical Journal* **320**, 484-488.

Britten N, Jenkins L, Barber N, Bradley C and Stevenson F (2003). Developing a measure of appropriateness in prescribing. *Quality and Safety in Health Care* **12**, 246-250.

Brock D (2002). Choice and equity. In: New B and Neuberger J (eds). *Hidden assets. Values and decision-making in the NHS.* London: King's Fund.

Brody DS (1980). The patient's role in clinical decision-making. *Annals of Internal Medicine* **93**, 718-722.

Brown N, Rappert B and Webster A (2000). Introducing contested futures: From looking into the future to looking at the future. In: *Contested futures. A sociology of prospective techno-science.* Aldershot: Ashgate.

Bryan J (1998). Three-score years and then? *Health Service Journal* **27** Aug, 4-8.

Buist A (1992). *The Oregon Experiment. Combining expert opinion and community values to set health care priorities.* Health Economic Research Group Report 12. Middlesex: Brunel University.

Burke K (2002). NICE may fail to stop "postcode prescribing", MPs told. *British Medical Journal* **324**, 191.

Burns A, Lawlor B and Craig S (2002). Rating scales in old age psychiatry. *British Journal of Psychiatry* **180**, 161-167.

Bury M (2001). Illness narratives: fact or fiction? *Sociology of Health and Illness* **23**, 263-285.

Butler CC, Kinnersley P, Prout H, Rollnick S, Edwards A and Elwyn G (2001). Antibiotics and shared decision-making in primary care. *Journal of Antimicrobial Chemotherapy* **48**, 435-440.

Butler J (1999). Ethics and the process of health care rationing. In: *The ethics of health care rationing. Principles and practices.* London: Cassell.

Byrne P and Long B (1976). Analysing detailed behaviour and discovering basic styles. In: *Doctors telling to patients.* London: HMSO.

Calabresi G and Bobbit P (1978a). Introduction. In: *Tragic choices.* New York: Norton.

Calabresi G and Bobbit P (1978b). Pure allocation approaches. In: *Tragic choices.* New York: Norton.

Campbell G and Sprague KL (2001). The state of drug decision-making: Report on a survey of P & T committee structure and practices. *Formulary* **36**, 644-647.

Cantrill J and Leese B (2002). An exploration of the potential impact of primary care groups on pharmaceutical practice. *Pharmaceutical Journal* **266**, 857-859.

Cappuccio FP, Oakeshott P, Strazzullo P and Kerry SM (2002). Application of Framingham risk estimates to ethnic minorities in United Kingdom and implications for primary prevention of heart disease in general practice: Cross sectional population based study. *British Medical Journal* **325**, *1271-1274.*

Caress AL, Luker K, Woodwock A and Beaver K (2002). A qualitative exploration of treatment decision-making role preference in adult asthma patients. *Health Expectations* **5**, 223-235.

Cassileth B, Zupkis RV, Sutton-Smith K and March V (1980a). Informed consent -- why are its goals imperfectly realized? *New England Journal of Medicine* **302**, 896-900.

Cassileth B, Zupkis RV, Sutton-Smith K and March V (1980b). Information and participation preferences among cancer patients. *Annals of Internal Medicine* **4**, 506 - 511.

Cayton H (2000). Only a last resort. *Nursing Standard* **14**, 24.

Cegala DJ, McClure L, Marinelli TM and Post DM (2000). The effects of communication skills training on patients' participation during medical interviews. *Patient Education and Counselling* **41**, 209-222.

Chalmers I, Dickersin K and Chalmers TD (1992). Getting to grips with Archie Cochrane's agenda. *British Medical Journal* **305**, 786-788.

Chaplin C (2002). *What is best for the patient? The ethical experiences, reasoning and decision-making of nurses.* PhD thesis. London: University of London.

Charavel M, Bremond A, Mounmjid-Fredjaoui N, Mignotte H and Charrere MO (2001). Shared decision-making in question. *Psycho-oncology* **10**, 93-102.

Charles C, Gafni A and Whelan T (1997). Shared decision-making in the medical encounter: what does it mean? (Or it takes at least two to tango) *Social Science and Medicine* **44**, 681-692.

Charles C, Gafni A and Whelan T (1999a). Decision-making in the physician-patient encounter: revisiting the shared treatment decision-making model. *Social Science and Medicine* **49**, 651-661.

Charles C, Gafni A and Whelan T (1999b). What do we mean by partnership in making decisions about treatment? *British Medical Journal* **319**, 780-782.

Charles J and Lewis J (1994). Requiring elderly patients to give signed consent for influenza vaccine. Does it affect acceptance? *Canadian Family Physician* **40**, 474-477.

Childress JF (1982). Consents. In: *Who should decide: paternalism in health care.* New York: Oxford University Press.

Chinitz D, Shalev C, Galai N and Israeli A (1998). Israel's basic basket of health services: the importance of being explicitly implicit. *British Medical Journal* **317**, 1000-1007.

Clark CM, Sheppard L, Fillenbaum GC, Galasko D, Morris JC, Koss E, Mohs R and Heyman A (1999). Variability in annual Mini-Mental State Examination score in patients with probable Alzheimer disease: a clinical perspective of data from the Consortium to Establish a Registry for Alzheimer's disease. *Archives of Neurology* **56**, 857-862.

Clarke S (2003). Informed consent without bureaucracy. *Journal of Clinical Neuroscience* **10**, 35-36.

Cleemput I and Kesteloot K (2002). Economic implications of non-compliance in health care. *Lancet* **359**, 2129-2130.

Clegg A, Bryant J, Nicholson T, Gerrad K, McIntyre L, de Broe S and Waugh N (2000). *Clinical and cost effectiveness of donepizil, rivastigmine and galantamine for Alzheimer's disease.* Southampton: Wessex Institute for Health Research and Development.

Cochrane A (1972). *Effectiveness and efficiency, random reflection on health services.* Leeds: Nuffield Provincial Hospitals Trust.

Colhoun H and Fuller J (1999). Drug therapy for coronary heart disease: the Sheffield table. *Lancet* **350**, 1853.

Collier J and Foster J (1985). Management of restricted drugs policy in hospital: the first five years experience. *Lancet* **i**, 333-335.

Committee for Proprietary Medicinal Products (CPMP) (1998). *EMEA opinions on sildenafil citrate (Viagra). Public statement.* London: The European Agency for the Evaluation of Medicinal Products, Human Medicines Evaluation Unit.

Commons Hansard (1999a). Criteria for appraisal by the National Institute for Clinical Excellence. In: *House of Commons Hansard Debates for 14 June 1999* (pt 24, 27). London: HMSO.

Commons Hansard (1999b). Prescriptions. In: *House of Commons Hansard Debates for 13 April* (pt 33). London: HMSO.

Corey-Bloom J, Anand R and Veach J (1998). A randomised trial evaluating the efficacy and safety of ENA 713 (rivastigmine tartrate), a new acetylcholinesterase inhibitor, in patients with mild to moderately severe Alzheimer's disease. *International Journal of Geriatric Psychopharmacology* **1**, 55-65.

Coulter A (1997). Partnerships with patients: the pros and cons of shared clinical decision-making. *Journal of Heath Service Research and Policy* **2**, 112-121.

Cox K (2002). Informed consent and decision-making: patients' experiences of the process of recruitment to phases I and II anti-cancer drug trials. *Patient Education and Counseling* **46**, 31-38.

Cribb A and Barber N (2000). *Developing pharmacy values. Stimulation the debate.* London: Royal Pharmaceutical Society of Great Britain.

Daniels N and Sabin J (1998). Ethics of accountability in managed care reform. *Health Affairs* **17**, 50-64.

Daniels N (1985). *Just Health Care.* Cambridge: Cambridge University Press.

Daniels N (2001). Justice, Health, and Healthcare. *American Journal of Bioethics* **1**, 2-16.

Davidson MH (2001). Safety profiles for the HMG-CoA reductase inhibitors. Treatment and Trust. *Drugs* **61**, 197-206.

Davis AJ (1988). The clinical nurse's role in informed consent. *Journal of Professional Nursing* **4**, 88-91.

Dean M (2003). UK health secretary promises more choice for NHS patients. *Lancet* **362**, 458.

Deber RB, Kraetschmer N and Irvine J (1996). What role do patients wish to play in the treatment decision-making? *Archives of Internal Medicines* **156**, 1414-1420.

Degner LF and Sloan JA (1992). Decision-making during serious illness: what role do patients really want to play? *Journal of Clinical Epidemiology* **45**, 941-950.

Degner LF, Kristjanson LJ, Bowman D Sloan JA, Carriere KC, O'Neil J, Bilodeau B, Watson P and Mueller B (1997). Information needs and decisional preferences in women with breast cancer. *Journal of the American Medical Association* **277**, 1485-1492.

Den Broeder L (2000). *State of Affairs, Concepts, Approaches, Organizations in the Women's Health Movement, Country Report The Netherlands.* Hannover, Germany: Association for Health Promotion in Lower Saxony.

Dent THS and Sadler M (2002). From guidance to practice: Why NICE is not enough? *British Medical Journal* **324**, 842-845.

Denzin N (1989). The sociological interview. In: *The research act. A theoretical introduction to sociological methods.* Engelwood Cliffs, NJ: Prentice Hall.

Department of Health (1998a). *Prescribing of sildenafil (Viagra ®),* CEM/CMO/98/15. London: Department of Health.

Department of Health (1998b). *Standing medical advisory committee on the use of Viagra ® (sildenafil) in the treatment of impotence (erectile dysfunction).* London: Department of Health.

Department of Health (1999a). *Health Service Circular 1999/065.Clinical governance: Quality in the new NHS.* London: HMSO.

Department of Health (1999b). *Pharmaceutical Price Regulation Scheme 1999.* doh.gov.uk/pprs.htm (accessed November 2002).

Department of Health (1999c). Statistics of prescriptions dispensed in the community: England 1988 to 1998. *Statistical Bulletin 1999/17.*

Department of Health (1999d). *Viagra ® – NHS Prescription Proposals Announced.* London: Department of Health.

Department of Health (1999e). *Health Service Circular (HSC) 1999/042. Sildenafil (Viagra).* London: Department of Health.

Department of Health (1999f). *Health Service Circular (HSC) 1999/115. Treatment for impotence.* London: Department of Health.

Department of Health (1999g). *Health Service Circular (HSC) 1999/148. Treatment for impotence.* London: Department of Health.

Department of Health (1999h). *Health Service Circular (HSC) 1999/177. Treatment for impotence. Patients with severe distress.* London: Department of Health.

Department of Health (2000a). *The NHS plan.* London: HMSO.

Department of Health (2000b). *The National Service Framework for Coronary Heart Disease.* London: HMSO.

Department of Health (2000c). *Primary care trusts: Financial framework.* London: HMSO.

Department of Health (2001a). *National Service Frameworks.* http://www.doh.gov.uk/nsf/about.htm (accessed August 2002).

Department of Health (2001b). *Reference guide to consent for examination or treatment.* London: HMSO.

Department of Health (2001c). *National Service Framework for older people.* London: HMSO.

Department of Health (2001d). *Press release: reference 2001/0475. No change for guidelines on GP prescribing of NHS impotence treatments.* London: Department of Health.

Department of Health (2002a). *Drug Tariff.* London: HMSO.

Department of Health (2002b). *Standing Medical Advisory Committee.* http://www.doh.gov.uk/smac.htm (accessed August 2002).

Department of Health (2002c). *Press release: 2002/0205 Wider access to medicines to allow patients to manage their own healthcare.* London: Department of Health.

Department of Health (2003a). *NHS leaflet HC12: Charges and optical voucher values 1 April 2002 / April 2003.* http://www.doh.gov.uk/nhscharges (accessed December 2003).

Department of Health (2003b). *Acute inpatient survey. National Overview 2001/02.* http://www.doh.gov.uk/acuteinpatientsurvey (accessed September 2003).

Development and Evaluation Committee (1998). *Rivastigmine (Exelon ®) in the treatment of senile dementia of the Alzheimer type (SDAT).* Bristol: The Wessex Institute.

Dewar S (1999). Viagra ®: the political management of rationing. In: Appleby J and Harrison A (eds). *Health Care UK 1999/2000.* London: King's Fund.

Dewing J (2002). Older people with confusion: capacity to consent and the administration of medicines. *Nursing Older People* **14**, 23-28.

Dinsmore W and Evans C (1999). Erectile dysfunction. ABC of sexual health. *British Medical Journal* **318**, 387-390.

Dixon M (2001). NHS Organisations – Primary Care Groups and Trusts. In: Merry P. *Wellard's NHS Handbook 2001/02*. 16th edition. Tunbridge Wells: JMH Publishing.

Dobrow MJ, Goel V and Upshur REG (2003). Evidence-based health policy: context and utilisation. *Social Science and Medicine* **58**, 207-217.

Donnelly M (2002). Developing a culture of consent. In: *Consent: bridging the gap between doctor and patient*. Cork University Press.

Dood MJ and Mood DW (1981). Chemotherapy: helping patients to know the drugs they are receiving and their possible side effects. *Cancer Nursing* **4**, 311-318.

Doraiswamy PM and Kaiser L (2000). Variability of the MMSE in dementia. *Neurology* **54**, 1538-1539.

Dowell J, Jones A and Snaddan D (2002). Exploring medication use to seek concordance with 'non-adherent' patients: a qualitative study. *British Journal of General Practice* **52**, 24-32.

Downie G, Hind C and Kettle J (2000). The abuse and misuse of prescribed and over the counter medicines. *Hospital Pharmacist* **7**, 242-250.

Doyal L (1995). Needs, rights and equity: moral quality in healthcare rationing. *Quality in Health Care* **4**, 273-283.

Doyal L (2001). Informed consent: moral necessity or illusion? *Quality in Health Care* **10** (Suppl I): i29-i33.

Dranove D, Hugdes FX and Shanley M (2003). Determinants of HMO Formulary adoption decisions. *Health Service Research* **38**, 169-189.

Drug and Therapeutic Bulletin (1998). Sildenafil for erectile dysfunction. *Drug and Therapeutic Bulletin* **11**, 81-84.

Drug and Therapeutic Bulletin (2000). Rivastigmine for Alzheimer's disease. *Drug and Therapeutic Bulletin* **38**, 15-16.

Dunn WN (1983). Introduction: *Values, ethics and the practice of policy analysis.* Lexington, Mass.: Lexington books.

Dunning AJ (1992). *Report of the Government Committee in Choices in Health Care.* Rijswijk: The Netherlands, Ministery of Welfare and Cultural Affairs.

Dyer C (1997). Ruling on interferon beta will hit all health authorities. *British Medical Journal* **315**, 143-148.

Dyer C (1999). Viagra ® guidance declared unlawful. *British Medical Journal* **318**, 1509.

Earl-Slater A (1997). Regulating the price of the UK's drug; second thoughts after the government's first report. *British Medial Journal* **314**, 365.

Ebrahim S, Davey Smith G, McCAbe C, Payne N, Pickin M, Sheldon TA, Lampe F, Sampson F, Ward S and Wannamethee G (1999). What role for statins? A review and economic model. *Health Technology Assessment* **3**, I-91.

Eijkman MA and Goedhart H (1996). The opinions of Dutch dentists on the introduction of new legislation in the field of informed consent. *Patient Education and Counseling* **28**, 45-50.

Elit L, Charles C, Gold I, Gafni A, Farrell S, Tedford S, Dal Bello D and Whelan T (2003). Women's perceptions about treatment decision making for ovarian cancer. *Gynecologie Oncology* **88**, 89-95.

Elwyn G , Gwyn R, Edwards A and Grol R (1999). Is shared decision-making feasible in consultations for upper respiratory tract infections? Assessing the influences of antibiotic expectations using discourse analysis. *Health Expectations* **2**, 105-117.

Elwyn GJ, Edwards A, Kinnersley P and Grol R (2000). Shared decision making and the concept of equipoise: The competences of involving patients in healthcare choices. *British Journal of General Practice* **50**, 892-899.

Elwyn G, Edwards A, Wensing M, Hibbs R, Wilkinson C and Grol R (2001a). Shared decision making observed in clinical practice: visual displays communication sequence and patterns. *Journal of Evaluation in Clinical Practice* **7**, 211-221.

Elwyn G, Edwards A, Mowle S, Wensing M, Wilkinson C, Kinnersley P and Grol R (2001b). Measuring the involvement of patients in shared decision-making: a systematic review of instruments. *Patient Education and Counseling* **43**, 5-22.

Elwyn G, Edwards A, Wensing M, Hood K, Atwell C and Grol R (2003). Shared decision-making: developing the OPTION scale for measuring patient involvement. *Quality and Safety in Health Care* **12**, 93-99.

Emanuel EJ and Emanuel LL (1992). Four models of the physician-patient relationship. *Journal of the American Medical Association* **267**, 2221-2226.

Ende J, Kazis L, Ash A and Moskowitz MA (1989). Measuring patients' desire for autonomy. Decision making and information-seeking preferences among medical patients. *Journal of General Internal Medicine* **4**, 23–30.

Faculty of Public Health Medicine of the Royal College of Physicians (1997). *Lipid lowering – how to draw the line. Guidance for directors of public health on drawing up local guidelines for cholesterol testing.* London: Royal College of Physician.

Faden RR and Beauchamp TL (1980). Decision-making and informed consent: a study of the impact of disclosed information. *Social Indicators Research* **7**, 313-336.

Faden RR and Beauchamp TL (1986). The concepts of informed consent and competence. In: *A history and theory of informed consent.* New York: Oxford University Press.

Faden RR, Becker C and Lewis C (1981). Disclosure of information to patients in medical care. *Medical Care* **19**, 718-733.

Fahey T (1998). Assessing heart disease risk in primary care. Editorial. *British Medical Journal* **317**, 1093-1094.

Feinmann J (2001). Arrested developments: The UK has a shocking record on cardiac disease. But why? *Guardian*, 30th September.

Ferguson D (2001). Informed consent –who knows? Results of a hospital audit project. *Health Education Journal* **60**, 371-377.

Ferner RE (1996). Newly licensed drugs. *British Medical Journal* **313**, 1157-1158.

Fleming TR and DeMets DL (1996). Surrogate end points in clinical trials: are we being misled? *Annals of Internal Medicines* **125**, 605-613.

Folstein MF, Folstein SE and McHugh PR (1975). Mini-mental state: A practical method for grading the cognitive state of patients for the clinician. *Journal of Psychiatric Research* **12**, 189-198.

Food and Drug Administration (1998). *First oral therapy for erectile dysfunction.* New York: Food and Drug Administration.

Ford S, Schofield T and Hope T (2003). What are the ingredients for a successful evidence-based patient choice consultation? A qualitative study. *Social Science and Medicine* **56**, 589-602.

Foretter F, Anand R and Gharabawi G (1999). A phase III study in patients with Alzheimer's disease to assess the preliminary efficacy and maximum tolerated dose of rivastigmine (Exelon registered). *European Journal of Neurology* **6**, 423-429.

Formulary (1995). Current formulary decision-making strategies and new factors influencing the process. *Formulary* **30**, 462-470.

Foy R, So J, Rous E and Scarffe H (1999). Perspectives of commissioners and cancer specialists in prioritizing new cancer drugs: impact of the evidence threshold. *British Medical Journal* **318**, 456-459.

Freemantle N, Barbour R, Johnson R, Marchment M and Kennedy A (1997). The use of statins: a case of misleading priorities? *British Medical Journal* **315**, 826-828.

Frith L (1999). Evidence-based medicine. In: Dowrick C and Frith L (eds). *General practice and ethics. Uncertainty and responsibility.* London: Routledge.

Fulop N, Allen P, Clarke A and Black N (2003). From health technology assessment to research on the organisation and delivery of health services: addressing the balance. *Health Policy* **63**, 155-165.

Galasko D, Corey-Bloom J and Thal LT (1991). Monitoring Progression in Alzheimer's Disease. *Journal of the American Geriatric Society* **39**, 932.

Gattellari M, Butow PN and Tattersall MH (2001). Sharing decisions in cancer care. *Social Science and Medicine* **52**, 1865-1878.

Gattellari M, Voigt KJ, Butow PN and Tattersall HN (2002). When the treatment goal is not cure: are cancer patients equipped to make informed decisions? *Journal of Clinical Oncology* **20**, 503-513.

General Medical Council (1998). *Seeking patients' consent: the ethical considerations.* London: General Medical Council.

Giacomini M (1999). The Which-Hunt: assembling health technologies for assessment and rationing. *Journal of Health Politics, Policy and Law* **24**, 715-758.

Gilbert D, Walley T and New B (2000). Lifestyle medicines. *British Medical Journal* **321**, 1341-1344.

Gillespie R and Florin D (2002). *Changing relationships.* London: King's Fund.

Glaser BG and Strauss AL (1968). *The discovery of the grounded theory. Strategies of qualitative research.* London: Weidenfeld & Nicolson.

Golin C, DiMatteo MR, Duan N, Leake B and Gelberg L (2002). Impoverished diabetic patients whose doctors facilitate their participation in medical decision making are more satisfied with their care. *Journal of General Internal Medicine* **17**, 857-866.

Gotler RS, Flocke SA, Goodwin MA, Zyzanski SJ, Murray TH and Stange KC (2000). Facilitating participatory decision-making. What happens in real-world community practice? *Medical Care* **38**, 1200-1209.

Grbich C (1999). Interviewing. In: *Qualitative research in health. An introduction.* London: Sage Publications.

Green RM (2001). Access to health care: going beyond fair equality of opportunity. *American Journal of Bioethics* **1**, 22-23.

Greenfield S, Kaplan S and Ware J (1985). Expanding patient involvement in care-effects on patient outcome. *Annals of Internal Medicine* **102**, 502-528.

Greenhalgh P (1997). More convincing evidence of efficacy needs to be cited. *British Medical Journal* **315**, 1623.

Guadagnoli E and Ward P (1998). Patient participation in decision-making. *Social Science and Medicine* **47**, 329-339.

Gwyn R and Edwards G (1999). When is a shared decision not (quite) a shared decision? Negotiating preferences in a general practice encounter. *Social Science and Medicine* **49**, 437-447.

Hall C (1998). Viagra ® abuse 'will add £1bn to NHS bill'. *Daily Telegraph* July 8th.

Hall JA and Roter DL (2002). Do patients talk differently to male and female physicians? A meta-analytic review. *Patient Education and Counseling* **48**, 217-224.

Halpern JJ and Stern RN (1998). Beneath the social science debate: economic and social notions of rationality. In: *Debating rationality.* London: Cornell University Press.

Halpern S (1999). Does Dobbo realise what he's done? *British Journal of Health Care Management* **5**, 64.

Ham C (1993). Priority setting in the NHS: reports from six districts. *British Medical Journal* **307**, 435-438

Ham C (1997). Priority setting in health care: Learning from international experience. *Health Policy* **42**, 49-66.

Ham C (1999a). New Labour and the NHS. In: *Health Policy in Britain. The politics and organisation of the national health service.* Hampshire: Macmillan.

Ham C (1999b). Tragic choices in health care: lessons from the child B case. *British Medical Journal* **319**, 1258-1261.

Ham C and Pickard S (1998). *Tragic choices. The case of child B.* London: King's Fund Institute.

Ham C and Coulter A (2003). International experience in rationing. In: Ham C and Robert G (eds.). *Reasonable rationing.* Buckingham: Open University Press.

Hammersley M and Atkinson P (1995). The process and analysis. In: *Ethnography: Principles in practice*. 2nd edition. London: Routledge.

Hansson MO (1998). Balancing the quality of consent. *Journal of Medical Ethics* **24**, 182-187.

Harris L, Boyle JM and Braunstein PJ (1982). Views of informed consent and decision-making: parallel surveys of physicians and the public. In: Presidential's Commission, *Making Health Care Decisions* **2**, 17-316.

Hasman A (2003). Eliciting reasons: empirical methods in priority setting. *Health Care Analysis* **11**, 41-58.

Haynes RB, McDonald H and Garg AX (2002). Helping patients follow prescribed treatment. Clinical applications. *Journal of the American Medical Association* **288**, 2880-2883.

Heart Protection Study Collaborative Group (2002). MRC/BHF Heart Protection Study of cholesterol lowering with simvastatin in 20,536 high-risk individuals: a randomised placebo-controlled trial. *Lancet* **360**, 7-22.

Heisler M, Bouknight RR, Hayward RA, Smith DM and Kerr EA (2002). The relative importance of physician communication, participatory decision making and patient understanding in diabetes self-management. *Journal of General Internal Medicine* **17**, 243-252.

Heitman E (1998). Ethics in technology assessment. *International Journal of Technology Assessment in Health Care* **14**, 544-566.

Henderson M (1998). The health guidelines that will disappoint. *The Times*, 14th September.

Herxheimer A (2002). Comment on: the influence of the pharmaceutical industry on the off-label use of its medicines. *Journal of Medical Ethics* **28**, 277.

Holmer AF (2002). Direct-to-consumer advertising – Strengthening our health care system. *New England Journal of Medicine* **346**, 526-528.

Holmes S (2000). Treatment of male sexual dysfunction. *British Medical Bulletin* **56**, 798-808.

Honigbaum F, Richard J and Lockett T (1995). *Priority setting in action – purchasing dilemmas.* Oxford: Recliff Medical Press.

Hope T, Hicks N, Reynolds DJM, Crisp R and Griffiths S (1998). Rationing and the health authority. *British Medical Journal* **317**, 1067-1069.

Hope T (2001). Rationing and life-saving treatments: Should identifiable patients have higher priority? *Journal of Medical Ethics* **27**, 179-185.

Hopper KD and Matthews YL (1993). Patient choice and non-ionic contrast media. *Investigative Radiology* **28**, 303-307.

Horne R and Weinman J (1999). Patients' beliefs about prescribed medicines and their role in adherence to treatment in chronic physical illness. *Journal of Psychosomatic Research* **47**, 555-567.

Hughes D and Griffiths L (1997). 'Ruling in' and ruling out': two approaches to the micro-rationing of health care. *Social Science and Medicine* **44**, 589-599.

Hulsman RL, Ros WJG, Winnubst JAM and Bensing JM (1999). Teaching clinically experienced physicians communication skills. A review of evaluation studies. *Medical Education* **33**, 655-668.

Hunter DJ (1980). Conclusion: Policy implication for the NHS. In: *Coping with uncertainty. Policy and politics in the NHS.* New York: Research Studies Press.

Hunter DJ (1995). Rationing: the case for "muddling through elegantly" *British Medical Journal* **311**, 811.

Iles A (2003). Given patients choice puts pressure on GPs, survey shows. *British Medical Journal* **327**, 527.

Illich I (1976). The epidemic of modern medicine. In: *Limits to medicine. Medical nemesis, the expropriation of health.* London: Boayrs.

Jackevicius CA, Mamdani M and Jack V (2002). Adherence with statin therapy in elderly patients with and without acute coronary syndromes. *Journal of the American Medical Association* **288**, 462-467.

Jacobs DR, Hebert B, Screiner PJ, Sidney S, Iribarren C and Hulley S (1997). Reduced cholesterol is associated with recent minor illness: the CARDIA Study. Coronary Artery Risk Development in young Adults. *American Journal of Epidemiology* **146**, 558-564.

Jadad AR, Moore RA, Carroll D, Jenkinson C, Reynolds DJM, Gavaghan DJ and McQuay HJ (1996). Assessing the quality of reports of randomized clinical trials: Is blinding necessary? *Controlled Clinical Trials* **17**, 1-12.

Jan S, Dommers E and Mooney G (2003). A politico-economic analysis of decision making in funding health service organisations. *Social Science and Medicine* **57**, 427-435.

Janesick V (1994). The dance of qualitative research design: metaphor, methodology and meaning. In: Denzin N and Lincoln Y (eds). *Handbook of qualitative research.* Thousand Oaks (Calif.): Sage Publications.

Jarvis MP (2001). *Nottingham Health Authority. Guidance on low priority. Restricted treatment and drugs.* http://www.shef.ac.uk/ebc/nha.doc (accessed May 2003).

Jenkings N and Barber N (2003). What constitutes evidence in hospital new drug decision making? *Social Science and Medicine*, in press.

Johri M and Lehoux P (2003). The great escape? Prospects for regulating access to technology through health technology assessment. *International Journal of Technology Assessment in Health Care* **19**, 179-193.

Jonsen AR (1998a). The Miracle of Modern Medicine: The ethics of organ transplantation and artificial organs. In: *The birth of bioethics.* Oxford: Oxford University Press.

Jonsen AR (1998b). Bioethics as a discourse. In: *The birth of bioethics.* Oxford: Oxford University Press.

Joshi MP, Williams A and Petrie JC (1994). Hospital formularies in 1993: Where, why and how? *Pharmaceutical Journal* **253**, 63-64.

Jubb V and Norris C (1993). Traditions in document analysis. In: Hammersley M (ed). *Social research: philosophy, politics and practice.* London: Sage.

Jubelirer S, Linton J and Magnetti S (1994). Reading versus comprehension: implications for patient education and consent in an outpatient oncology clinic. *Journal of Cancer Education* **9**, 26-29.

Kaplan SH, Greenfield S and Ware JE Jr. (1989) Assessing the effects of physician-patient interactions on the outcomes of chronic disease. *Medical Care* **27**, (3 Suppl) S110-S127.

Kassirer JP (1994). Incorporating patients' preferences into medical decisions. *New England Journal of Medicine* **330**, 1895-1896.

Katz JN, Daltroy LH, Brennan TA and Liang MH (1992). Informed consent and the prescription of non-steroidal anti-inflammatory drugs. *Arthritis and Rheumatism* **35**, 1257-1263.

Kaufman CL (1983). Informed consent and patient decision-making: two decades if research. *Social Science and Medicine* **17**, 1657-1664.

Kee F, Patterson CC, Wilson EA, McConnell JM, Wheeler S and Watson DJ (2000). Stewardship or clinical freedom? Variations in dialysis decision-making. *Nephrology, Dialysis, Transplantation* **15**, 1647-1657.

Keeley D (1997). General practice fundholding and health care costs. *British Medical Journal* **351**, 139.

Kelly CA, Harvey RJ and Cayton H (1997). Drug treatments for Alzheimer's disease. *British Medical Journal* **314**, 603.

Kennedy I and Grubb A (2000). *Medical Law*. 3rd edition. London: Butterworths.

Kerrigan DD (1993). Who's afraid of informed consent? *British Medical Journal* **306**, 298-300.

Khan F (2002). Using medicines wisely. The place of the formulary in medicines management. *Hospital Pharmacist* **9**, 159-163.

Kind P, Dolan P, Gudex C and Williams A (1998). Variations in population health status: results from a United Kingdom national questionnaire survey. *British Medical Journal* **316**, 736-741.

Kitzinger J (1995). Qualitative research: introducing focus groups. *British Medical Journal* **311**, 299-302.

Kjellgren KI, Svensson S, Ahlner J and Saljo R (2000). Antihypertensive treatment and patient autonomy--the follow-up appointment as a resource for care. *Patient Education and Counseling* **40**, 39-49.

Klein R (1993). Rationality and rationing. In: Tunbridge M (ed). *Rationing of health care in medicine*. London: Royal College of Physicians.

Klein R (1995). Priorities and rationing: pragmatism or principles? *British Medical Journal* **311**, 761-762.

Klein R (2001). Politics of the technocratic change. In: *The new politics in the National Health Service*. 4th edition. Harlow, Essex: Prentice Hall.

Klein R (2002). A babel of voices: values, policy-making and the NHS. In: New B and Newberger J (eds). *Hidden assets. Values and decision-making in the NHS.* London: King's Fund.

Kleinert S (2000). The next phase of priority setting in health care. *Lancet* **354**, 1869-1870.

Kmietowicz Z (2001). Government insists NHS pays for drugs approved by NICE. *British Medical Journal* **323**, 1386.

Koopmans PP (2002). Clinical end points in cancer trials: time to rethink? *British Medical Journal* **324**, 1389-1391.

Laine C and Davidoff F (1996). Patient-centered medicine: a professional evolution. *Journal of the American Medical Association* **375**, 152-156.

Lamb HM and Goa KL (2001). Rivastigmine. A pharmacoeconomic review of its use in Alzheimer's disease. *PharmacoEconomics* **19**, 303-318.

Langtry HD and Markham A (1999). Review of its use in erectile dysfunction. *Drugs* **57**, 967-989.

Le Fanu J (2002). The pill could guard against Alzheimer's in sickness and in health. *The Sunday Telegraph*, Jan 20.

Leach RH and Leach SJ (1994). Drug and therapeutic committees in the United Kingdom in 1992. *Pharmaceutical Journal* **253**, 61-62.

Lehoux P and Blume S (2000). Technology assessment and the sociopolitics of health technologies. *Journal of Health Politics, Policy and Law* **25**, 1083-1120.

Leighl N, Gatterlari M, Butow P, Brown R and Tattersall MHN (2001). Discussing adjuvant cancer therapy. *Journal of Clinical Oncology* **19**, 1768-1778.

Leino-Kilpi H (2000). Informed consent in the literature. In: *Patient's autonomy, privacy and informed consent.* Amsterdam: Biomedical Health Research.

Lelie A (2000). Decision-making in nephrology: shared decision-making? *Patient Education and Counseling* **39**, 81-89.

Lewis C and Allen D (2002). General prescribing principles – choosing a drug. *Nursing Standard* **17**, 32-33.

Lidz CW, Meisel A, Osterweis M, Holden JL, Marx JH and Munetz MR (1983). Barriers to informed consent. *Annals of Internal Medicines* **99**, 539-543.

Lidz CW, Meisel A and Munetz M (1985). Chronic disease: the sick role and informed consent. *Culture, Medicine and Psychiatry* **9**, 241-255.

Lidz CW, Appelbaum PS and Meisel A (1988). Two models of implementing informed consent. *Archives of Internal Medicine* **148**, 1385-1389.

Light D and Hughes D (2001). Introduction: A sociological perspective on rationing. *Sociology of Health and Illness* **23**, 551-569.

Lilleyman J (2002). Consent in the practice of haematology. *British Journal of Haematology* **115**, 782-785.

Lincoln YS (2002). Emerging criteria for quality in qualitative and interpretive research. In: Denzin NK and Lincoln YS (2002). *The qualitative inquiry reader.* Thousand Oaks (Calif.), London: Sage Publications.

Lincoln YS and Guba EG (1985). Establishing trustworthiness. In: *Naturalistic inquiry.* Thousand Oaks (Calif.), London: Sage Publications.

Livingston G and Katona C (2000). How useful are cholinesterase inhibitors in Alzheimer's disease? A number needed to treat analysis. *International Journal of Geriatric Psychiatry* **15**, 203-207

Llodys A, Hayes P, Bell PRF and Naylor AR (2001). The role of risk and benefit perception in informed consent for surgery. *Medical Decision Making* **21**, 141-149.

Lovestone S, Graham N and Howard R (1997). Guidelines on drug treatments for Alzheimer's disease. *Lancet* **350**, 232-233.

Luce BR and Brown RE (1995). The use of technology assessment by hospitals, health maintenance organizations, and third-party payers in the United States. *International Journal Technology Assessment in Health Care* **11**, 79-92.

Lyles A, Luce BR and Rentz AM (1997). Manged care pharmacy, socio-economic assessments and drug adoption decisions. *Social Science and Medicine* **45**, 511-521.

Makoul G, Arntson P and Schofield T (1995). Health promotion in primary care: physician-patient communication and decision making about prescription medications. *Social Science and Medicine* **41**, 1241-1254.

Malin N, Wilmot S and Wanthorpe J (2002). Prioritising and rationing. In: *Key concepts and debates in health and social policy.* Buckingham: Open University Press.

Manneback MA, Ascione FJ, Gather CA, Bagozzi RP, Cohen IA and Ryan ML (1999). Activities, function and structure of pharmacy and therapeutics committees in large teaching hospitals. *American Journal of Health-System Pharmacy* **56**, 622-628.

Mansell D, Poses R, Kazis L and Duefield C (2000). Clinical factors that influence patients' desire for participation in decision about illness. *Archives of Internal Medicine* **160**, 2991-2996.

Marelich WD, Johnston Roberts K, Murphy DA and Callari T (2002). HIV/AIDS patient involvement in antiretroviral treatment decisions. *AIDS Care* **14**, 17-26.

Mariner WK (1994). Patients' rights after health care reform: Who decides what is medically necessary? *American Journal of Public Health* **84**, *1515-1520.*

Marks P (2003). The evolution of the doctrine of consent. *Clinical Medicine* **3**, 45-46.

Mark JS and Spiro H (1990). Informed consent for colonscopy. A prospective study. *Archives of Internal Medicine* **150**, 777-780.

Martin DK, Pater JL and Singer PA (2001). Priority-setting decisions for new cancer drugs: a qualitative case study. *Lancet* **358**, 1676-1681.

Martin DK, Giacomini M and Singer PA (2002). Fairness, accountability for reasonableness, and the views of priority setting decision-makers. *Health Policy* **61**, 279-290.

Martin DK, Hollenber D, MacRae S, Madden S and Singer P (2003). Priority setting in a hospital drug formulary: a qualitative case study and evaluation. *Health Policy* **66**, 295-303.

Marumo K and Murai M (2001). Aging and erectile dysfunction; the role of aging and concomitant chronic illness. *International Journal of Urology* **8**, S50-S57.

May T (1997). Document analysis. In: *Social research.* Buckingham: Open University Press.

Mayeux RM and Sano M (1999). Treatment of Alzheimer's disease. *The New* England *Journal of Medicine* **341**, 1670-1679.

Mays N and Pope C (2000). Qualitative research in health care: Assessing quality in qualitative research. *British Medical Journal* **320**, 50-52.

McConnachie A, Hunt K, Emslie C, Hart C and Watt G (2001). Unwarranted survivals" and "anomalous deaths" from coronary heart disease: prospective survey of general population. *British Medical Journal* **323**, 1487-1491.

McDonald R (2002). Street-level bureaucrats? Heart disease, health economics and policy in a primary care group. *Health and Social Care in the Community* **10**, 129-135.

McDonald R, Burrill P and Walley T (2001). Managing the entry of new medicines in the National Health Service: health authority experience and prospects for primary care groups and trusts. *Health and Social Care in the Community* **9**, 341-347.

Mechanic D (1995). Dilemmas in rationing health care services: the case for implicit rationing. *British Medical Journal* **310**, 1655-1659.

Medicines Act 1968, section 58A.

Medicines Control Agency (1996). *Towards safe medicines. A guide to the control of safety, quality and efficacy of human medicines in the United Kingdom.* London: MCA.

Medicines and Healthcare products Regulatory Agency (MHRA) (2003). *Consultation document: ARM 18. Reclassification summary for simvastatin POM to P.* London: Medicines and Healthcare products Regulatory Agency.

Michie S, Miles J and Weinman J (2003). Patient-centredness in chronic illness: what is it and does it matter? *Patient Education and Counseling* **51**, 197-206.

Miles MB and Huberman AM (1994). Making good sense: Drawing and verifying conclusions. In: *Qualitative data analysis.* 2nd edition. Thousand Oaks (Calf.), London: Sage Publications.

Mitton C, Patten S, Waldner H and Donaldson C (2003). Priority setting in health authorities: a novel approach to a historical activity. *Social Science and Medicine* **57**, 1653-1663.

Moore W (2002). 21th century heart solution may have a string in the tail. *British Medical Journal* **325**, 184.

Moore DS and McCabe GP (2000). Appendix, table E. In: *Introduction to the practice of statistics.* New York: W.H. Freeman & Company.

Moore RA, Edwards JE and McQuay HJ (2002). Sildenafil (Viagra) for male erectile dysfunction: a meta-analysis of clinical trial reports. *BMC Urology* **2**, 6.

Morgan M, Calnan M and Manning N (1993). The hospital as a social organisation. In: *Sociological Approaches to Health and Medicine.* 3rd edition. London: Routledge.

Mulldoch MF, Manuck SB and Matthews KA (1990). Lowering cholesterol concentrations and mortality. *British Medical Journal* **301**, 815.

Murden RA, McRae TD, Kaner S and Buckham ME (1991). MMSE State Examination Scores vary with education in blacks and whites. *Journal of the American Geriatric Society* **39**, 149-155.

Murphy E, Dingwall R, Greatbatch D, Parker S and Watson P (1998). Qualitative research methods in health technology assessment: a review of the literature. *Health Technology Assessment* **2**, 89-163.

Murray E, Lo B, Pollack L, Donelan K, Catania J, White M, Zapert K and Turner R (2003). The impact of health information on the Internet on the physician-patient relationship. *Archives of Internal Medicines* **163**, 1727-1734.

Muss HB, White DR, Michielutte R, Richard F 2nd, Cooper MR, Williams S, Stuart JJ and Spurr CL (1979). Written informed consent in patients with breast cancer. *Cancer* **43**, 549-556.

National Association of Health Authorities and Trusts (NAHAT) (1995). Health committee report on priority setting in purchasing. *NAHAT briefing No* **78**.

National Audit Office (2001). *Handling clinical negligence claims in England.* London: HMSO.

National Institute for Clinical Excellence (NICE) (2001). *Technology appraisal guidance on 19. Guidance on the use of donepezil, rivastigmine and galantamine for the treatment of Alzheimer's disease.* London: NICE.

National Institute for Clinical Excellence (NICE) (2003a). *Recommendation from citizens council on clinical need have immediate impact on NICE.* http://www.nice.org.uk/pdf/2003_003_Press_release_citizenscouncil.pdf (accessed May 2003).

National Institute for Clinical Excellence (NICE) (2003b). *Press release: NICE asked to develop guidance on treating eczema, support programmes for parents dealing with conduct disorder in children, use of statins for heart disease, and when hysterectomy is appropriate.* http://www.nice.org.uk/pdf/9pressrelease8thwave.pdf (access April 2003).

National Service Framework (2000). *National Service Framework for Coronary Heart Disease.* London: Department of Health.

National Service Framework (2001). *National Service Framework for Older People.* London: Department of Health.

New B (1996). The rationing agenda in the NHS. *British Medical Journal* **312**, 1593-1601.

Neuberger J (2002). The value project. In: New B and Neuberger J (eds.). *Hidden assets. Values and decision-making in the NHS.* London: King's Fund.

Newdick C (1995). Statutory regulation of standards. In: *Who should we treat? Law, patients and resources in the NHS.* Oxford: Clareton.

Newdick C (1998). Primary care groups and the right to prescribe. *British Medical Journal* **317**, 1361-1365.

NHS (General Medical Service) Regulations 1992.

NHS Centre for Reviews and Dissemination (1998). Cholesterol and coronary heart disease: screening and treatment. *Effective Health Care Bulletins* **4**, 1-16.

NHS Centre for Reviews and Dissemination (2003). *Health technology assessment. Frequently asked questions.* http://nhscrd.york.ac.uk (accessed June 2003).

NHS Executive South and West, R&D Directorate (1996). *Who benefit from statins? Revisiting the evidence from a purchasing perspective.* Bristol: NHS Executive South and West.

Nordheim OF (2002). The role of evidence in health policy making: a normative perspective. *Health Care Analysis* **10**, 309-317.

Novartis (2001). *Exelon. Summary of product characteristics.* Surrey: Novartis.

Nursing and Midwifery Council (NMC) (2002). *Guidelines for the administration of medicines.* London: Nursing and Midwifery Council.

O'Brien (1986). *'What are my chances doctor?' A review of clinical risks.* London: Brunell University.

O'Connor AM, Stacey D, Entwistle V, Llewellyn-Thomas H, Rovner D Holmes-Rovner M, Tait V, Tetroe J, Fiset V, Barry M and Jones J (2003). Decision aids for people facing health treatment or screening decisions. In: *The Cochrane Library* **3**. Oxford: Update Software.

O'Neill O (2003). Some limits of informed consent. *Journal of Medical Ethics* **29**, 4-7.

Oates L (2000). The courts' role in decisions about medical treatment. *British Medical Journal* **321**, 1282-1284.

Oberlander J, Marmor T and Jacobs L (2001). The Oregon Health Plan: rhetoric, rationing and reality. *British Journal of Health Care Management* **7**, 358-362.

Olver JN, Turrell SJ, Olszewski NA and Willson KJ (1995). Impact of an information and consent form on patients having chemotherapy. *Medical Journal of Australia* **162**, 82-83.

Omar MA, Wilson JP and Cox TS (2001). Rhabdomyolysis and HMG-CoA reductase inhibitors. *Annals of Pharmacotherapy* **35**, 1096-1097.

Ong LM, de Haes JC, Hoos AM and Lammes FB (1995). Doctor-patient communication: a review of the literature. *Social Science and Medicine* **40**, 903-918.

Paice E, Aitken M and Moss F (2001). Informed consent and the preregistration house officer. *Hosptial Medicine* **62**, 699-701.

Patel MG, Wright DJ, Gill PS, Jerwood D, Silcock J and Chrysten H (2002). Prescribing of lipid lowering drugs to South Asian patients: ecological study. *British Medical Journal* **325**, 25-26.

Pelligrino E (1992). Is truth-telling to the patient a cultural artifact? *Journal of the American Medical Association* **268**, 1734-1735.

Pharmaceutical Journal (2000). Managing the entry of expensive new drugs. *Pharmaceutical Journal* **265**, 496-497.

Pharmaceutical Journal (2001a). National Service Framework for old people. *Pharmaceutical Journal* **266**, 415-416.

Pharmaceutical Journal (2001b). Money-back guarantees pass ABPI code. *Pharmaceutical Journal* **266**, 846.

Pharmaceutical Journal (2002). NICE defends its work to the Health Select Committee. *Pharmaceutical Journal* **268**, 125.

Pickin DM, McCabe CJ, Ramsay LE, Payne N, Haq IU, Yeo WW and Jackson PR (1999). Cost effectiveness of HMG-CoA reductase inhibitor (statin) treatment related to the risk of coronary heart disease and cost of drug treatment. *Heart* **82**, 325-332.

Pilling D and Timmins N (2001). NHS set to extend use of Alzheimer's drug. *Financial Times*, Jan 19.

Pollock K, Blenkinsopp A and Grime J (2002). Concordance – A valuable contribution to make to debate. *Pharmaceutical Journal* **268**, 837-838.

Pope C, Ziebland S and Mays N (2000). Analysing qualitative data. *British Medical Journal* **320**, 114-116.

President's Commission for the Study of the Ethical Problems in Medicine and Biomedical and Behavioral Research (PCEMR) (1982). Making health care decisions. The ethical and legal implications of informed consent in the patient-practitioner relationship. *President's Commission for the Study of Ethical Problems in Medicine and Biomedical and Behavioural Research* 3, Appendices: studies on the foundations of informed consent. Washington: USGPO.

Prior L (2001). Rationing through risk assessment in clinical genetics: all categories have wheels. *Sociology of Health and Illness* **23**, 570-593.

Psaty BM, Weiss NS, Furberg CD, Koepsell TD, S Siscovick D, Rosendaal FR, Smith NL, Heckbert SR, Kaplan RC, Lin D, Fleming TR and Wagner EH (1999). Surrogate end points, health outcomes, and the drug-approval process for the treatment of risk factors for cardiovascular disease. *Journal of the American Medical Association* **282**, 786-790.

Quaid KA, Faden RR, Vining EP and Freeman JM (1990). Informed consent for a prescription drug: Impact of disclosed information on patient understanding and medical outcomes. *Patient Education and Counseling* **15**, 249-259.

Quintiliani R, Grousse GC and Chow MS (1990). P & T committee perspectives: Achieving rational therapeutics through consensus-based protocols and follow-up. *Hospital Formulary* **25**, 283-294.

Ramsey P (1970). Consent as a canon of loyalty with special reference to children in medical investigations. In: *The patient as person. Explorations in medical ethics.* New Haven and London: Yale University Press.

Raynor T and Britten N (2001). Why medicines information leaflets do nothing to promote concordance. *Pharmaceutical Journal* **267**, 120.

Reed J and Payton VR (1997). Focus groups: issues of analysis and interpretation. *Journal of Advanced Nursing* **26**, 765-777.

Reid FDA, Cook DG and Whincup PH (2002). Use of statins in the secondary prevention of coronary heart disease: Is treatment equitable? *Heart* **88**, 15-19.

Reynolds TM (1997). Standing Medical Advisory Committee should reconsider advice to use Sheffield risk table. *British Medical Journal* **315**, 1615.

Rhodes R (2001). Justice, medicine and medical care. *American Journal of Bioethics* **1**, 32-33.

Rich DS (2001). Ask the Joint Commission. Required consent for medications. *Hospital Pharmacy* **36**, 337-342.

Richards H and Emslie C (2000). The 'doctor' or the 'girl from the University'? Considering the influence of professional roles on qualitative interviewing. *Family Practice* **17**, 71-75.

Richardson A (1983). The concepts and forms of participation. In: *Participation. Concept in social policy.* London: Routledge.

Richardson NG and Jones MG (1996). Should house officers obtain consent for operation and anaesthesia? *Health Trends* **28**, 56-59.

Robinson A and Thomson R (2001). Variability in patients preferences for participating in medical decision making: implication for the use of decision support tools. *Quality and Safety in Health Care* **10**, i34-i38.

Roche Pharmaceuticals (2001). *Roaccutance. Summary of Product Characteristics.* Hertfordshire: Roche Pharmaceuticals.

Rodwin MA (2001). The politics of evidence-based medicine. *Journal of Health Politics, Policy and Law* **26**, 439-445.

Rogers WA (1999). Beneficence in general practice: an empirical investigation. *Journal of Medical Ethics* **25**, 388-393.

Rogers WA (2002). Whose autonomy? Which choice? *Family Practice* **19**, 140-145.

Rosen R and Gabbay J (1999). Linking health technology assessment to practice. *British Medical Journal* **319**, 1-3.

Rosen RC (1996). Erectile dysfunction: the medicalization of male sexuality. *Clinical Psychology Review* **16**, 497-519.

Rosenberg W (2001). Evidence-based patient choice in secondary care. In: Edwards A and Elwyn G (eds). *Evidence-based patient choice.* Oxford: Oxford University Press.

Rösler M, Anand R, Cicin-Sain A, Gauthier S, Agid Y, Dal-Bianco P, Stähelin H, Hartman R, Gharabawi M and Bayer T (1999). Efficacy and safety of rivastigmine in patients with Alzheimer's disease: International randomised controlled trial. *British Medical Journal* **318**, 633-640.

Royal College of Psychiatrists (Section for the Psychiatry of Old Age) (1997). *Interim Statement on Anti-Dementia Drugs - Implications, Concerns and Policy Proposals.* London: Royal College of Psychiatry.

Royal Pharmaceutical Society of Great Britain and Merck Sharp & Dohme (1997). *From compliance to concordance. Achieving shared goals in medicine taking.* London: Royal Pharmaceutical Society and Merck, Sharpe and Dohme.

Rucker TD and Schiff G (1990). Drug formularies: Myths-in-formation. *Medical Care* **28**, 928-942.

Rucker TD and Visconti JA (1976). Hospital formularies: organizational aspects and supplementary components. *American Journal of Hospital Pharmacy* **33**, 912-917.

Sackett D, Rosenberg WMC, Gray JAM, Haynes RB and Richardson WS (1996). Evidence based medicine: what it is and what it isn't. *British Medical Journal* **312**, 71 - 72.

Sacks FM, Pfeffer MA, Moye LA, Rolleau JL, Rutherford JD, Cole TG, Brown L, Warnica JW, Arnold JMO, Wun C, Davis BR and Braunwald E (1996). The effect of pravastatin on coronary events after myocardial infarction in patients with average cholesterol levels. *New England Journal of Medicine* **335**, 1001-1009.

Salmon DP, Thal LJ, Butters N and Heindel WC (1990). Longitudinal evaluation of dementia of the Alzheimer type: A comparison of 3 standardised mental status examinations. *Neurology* **40**, 1225-1230.

Salt B (2003). *Medicines: do they work?* The Association of the British Pharmaceutical Industry. Publications. www.abpi.org.uk/publications. (accessed August 2003).

Sanders T and Skevington S (2003). Do bowel cancer patients participate in treatment decision-making? Findings from a qualitative study. *European Journal of Cancer Care* **12**, 166-175.

Savage R and Armstrong D (1990). Effect of general practitioner consultation's style on patients' satisfaction: a controlled study. *British Medical Journal* **301**, 968-970.

Scandinavian Simvastatin Study Group (1994). Randomised trial of cholesterol lowering in 444 patients with coronary heart disease: The Scandinavian Simvastatin Survival Study (4S). *Lancet* **344**, 1383-1389.

Schachter M (2001). Statins, drug interactions and cytochrome P450. *British Journal of Cardiology* **8**, 311-317.

Schneider C (1998). The reluctant patient: Can adjuring autonomy make sense? In: *The practice of autonomy. Patients, doctors, and medical decisions.* Oxford: Oxford University Press.

Schouten BC, Eijkman MA, Hoogstraten J and den Dekker J (2001). Informed consent in Dutch dental practice: knowledge, attitudes and self-efficacy of dentists. *Patient Education and Counseling* **42**, 185-192.

Scott PA, Valimaki M, Leino-Kilpi A, Dassen T, Gasull M, Lemonidou C and Arndt M (2003). Autonomy, privacy and informed consent 3: elderly care perspective. *British Journal of Nursing* **12**, 158-168.

Sculpher M, Gafni A and Watt I (2002). Shared treatment decision making in a collectively funded health care system: possible conflicts and some potential solutions. *Social Science and Medicine* **54**, 1369-1377.

Seale C (1999). Accounting for contradiction. In: *The quality of qualitative research.* Thousand Oaks (Calf.), London: Sage Publications.

Secretary of State for Health (1997). *The new NHS: modern, dependable.* London: The HMSO.

Seedhouse D (1993). Paper two: Need. In: *Fortress NHS. A philosophical review of the National Health Service.* Chichester: J. Wiley.

Segal R and Pathak DS (1988). Factors that influence P & T committee drug evaluations and decision making. *Hospital Formulary* **23**, 174-178.

Select Committee Report (1999). *House of commons – Health – Minutes of Evidence, Examination of Witnesses (Questions 796) 29 April.* London: HMSO.

Shalanshy SJ, Virk R, Ackman M, Jackevicius C, Kertland H, Tsuyuki R and Humphries K (2003). Access to new cardiovascular therapies in Canadian hospitals: A national survey of the formulary process. *Canadian Journal of Cardiology* **19**, 173-179.

Sharpe VA and Faden AI (1998). Democratization of medical morality. In: *Medical harm.* Cambridge: Cambridge University Press.

Sheperd J, Cobbe SM, Ford I, Isles CG, Lorimer AR, MacFarlane PW, McKillop JH and Packard CJ (1995). Prevention of coronary heart disease with pravastatin in men with hypercholesterolemia. *New England Journal of Medicine* **333**, 1301-1307.

Shepherd MD and Salzman RD (1994). Formulary decisions in the HMOs. *PharmacoEconomics* **5**, 29-38.

Shriner DL, Wagner RF, Weedn VW, Winslade WJ and Aragona JM (1992). Informed consent and risk management in dermatology: to what extent do dermatologists disclose alternate diagnostic and treatment options to their patients? *Journal of Contemporary Health Law Policy* **8**, 137-162.

Shubra M and Taylor D (2002). Adherence to NICE guidance for the use of anticholinesterases for Alzheimer's disease. *Pharmaceutical Journal* **269**, 680-681.

Shulman D (2001). Expertise in Law, Medicine, and Health Care. *Journal of Health Politics, Policy and Law* **26**, 117-126.

Shuster E (1997). Fifty years later: the significance of the Nuremberg Code. *New England Journal of Medicine* **337**, 1436-1440.

Silverman D (1987). Decision-making discourse: part 1. In: *Communication and medical practice - Social relations in the clinic.* London: Sage Publications.

Silverman D (1993). Ethnography. In: *Interpreting qualitative data. Methods for analysing talk, text and interaction.* London: Sage Publications.

Silvers A (2001). No basis for justice: equal opportunity, normal functioning, and the distribution of healthcare. *American Journal of Bioethics* **1**, 35.

Singer P, Choudhry S and Armstrong J (1993). Public opinion regarding consent to treatment. *Journal of the American Geriatric Society* **41**, 112-116.

Singer PA, Martin DK, Giacomini M and Purdy L (2000). Priority setting for new technologies in medicine: qualitative case study. *British Medical Journal* **321**, 1316-1318.

Skene L and Smallwood R (2002). Informed consent: lessons from Australia. *British Medical Journal* **324**, 39-41.

Smith F (2002). Qualitative interviews. In: *Research methods in pharmacy practice.* London: Pharmaceutical Press.

Smith GD, Shipley MJ, Marmot MG and Rose G (1992). Plasma cholesterol concentration and mortality. The Whitehall Study. *Journal of the American Medical Association* **267**, 2740-2741.

Smith GD, Song F and Sheldon TA (1993). Cholesterol lowering and mortality: the importance of considering initial level of risk. *British Medical Journal* **306**, 1367-1373.

Smith R (1991). Rationing: The search for sunlight. Editorial. *British Medical Journal* **303**, 1561-1562.

Staffa JA, Chang J and Green L (2002). Cerivastatin and reports of fatal rhabdomyolysis. *New England Journal of Medicine* **346**, 539-540.

Stamler J, Stamler R, Neaton J, Wentworth D, Daviglus ML, Garside D, Dyer A, Liu K and Greenland P (1999). Low risk-factor profile and long-term cardiovascular and noncardiovascular mortality and life expectancy: findings for 5 large cohorts of young adult and middle-aged men and women. *Journal of the American Medical Association* **282**, 2012-2018.

Standing Medical Advisory Committee (1997). *The use of statins.* www.doh.gov.uk/cmo/statins2.htm (accessed May 2002).

Standing Medical Advisory Committee (1998). *Advice from the SMAC on the use of Viagra ® (sildenafil) in the treatment of impotence (erectile dysfunction).* www.doh.gov.uk/cmo/cmo9814.htm (accessed Feb 2002).

Stein K, Milne R and Best L (1997). BMJ should require advertisements to detail actual state of evidence. *British Medical Journal* **315**, 1623.

Stevens A, Milne R and Burls A (2003). Health technology assessment: history and demand. *Journal of Public Health Medicine* **25**, 98-101.

Stevenson FA (2001). The strategies used by general practitioners when providing information about medicines. *Patient Education and Counseling* **43**, 97-104.

Stevenson FA (2003). General practitioners' views on shared decision-making: a qualitative analysis. *Patient Education and Counseling* **50**, 291-293.

Stevenson FA, Barry CA, Britten N, Barber N and Bradley C (2000a). Doctor-patient communication about drugs: the evidence for shared decision making. *Social Science and Medicine* **50**, 829-840.

Stevenson FA, Britten N, Barry CA, Barber N and Bradley CP (2000b). Qualitative methods and prescribing research. *Journal of Clinical Pharmacy and Therapy* **5**, 317-324.

Stocking B (1991). Patient's charter. New rights issue. *British Medical Journal* **303**, 1148-1149.

Stolk EA, Brouwer WBF and Busschbach JJV (2002). Rationalising rationing: Economic and other considerations in the debate about funding of Viagra. *Health Policy* **59**, 53-63.

Strull WM, Lo B and Charles G (1984). Do patients want to participate in medical decision making? *Journal of the American Medical Association* **252**, 2990-2994.

Stuffaford T (1998). Every day another loss to grieve. *The Times* Dec 3.

Stuttaford T (1999). Partial to pizza? Stock up on statins. *The Times*, January 28th.

Stuttaford T (2000). The drugs that fight cholesterol. *The Times*, November 16th.

Sugarman J, McCrory DC, Powell D, Krasny A, Adams B, Ball E and Cassell C (1999). Empirical research on informed consent. *Hastings Center Reports* **29** (Suppl): S1-42.

Sullivan M (2003). The new subjective medicine: taking the patient's point of view on health care and health. *Social Science and Medicine* **56**, 1595-1604.

Sulmasy D, Lehmann LS, Levine DM and Faden RR (1994). Patients' perception of the equality of informed consent for common medical procedures. *Journal of Clinical Ethics* **5**, 189-194.

Summers KH and Szeinbach SL (1993). Formularies: The role of pharmacy and therapeutics committees. *Clinical Therapeutics* **15**, 433-441.

Szasz TS and Hollender MH (1956). The basis models of the doctor-patient relationship. *Archives of Internal Medicine* **97**, 585-592.

Tauber AI (2001). Historical and philosophical reflections on patient autonomy. *Health Care Analysis* **9**, 299-319.

Taylor D, Shubhra M and Fry C (2001). The use of anticholinesterases for Alzheimer's disease in Britain. *Pharmaceutical Journal* **266**, 263.

The Impotence Association (2001). *Consultation on the Current Statutory Framework for the Treatment of Impotence on the National Health Service by GP's.* London: The Impotence Association.

Thistlethwaite JE (2002). Making and sharing decisions about management with patients: the views and experiences of pre-registration house officers in general practice and hospital. *Medical Education* **36**, 49-55.

Tingle J and Cribb A (1995). *Nursing law and ethics.* Oxford: Blackwell Science.

Tolman KG (2002). The liver and lovastatin. *American Journal of Cardiology* **89**, 1374-1380.

Tombaugh TN and McIntyre NJ (1992). The mini-mental state examination: a comprehensive review. *Journal of the American Geriatrics Society* **40**, 922-935.

Towle A and Godolphin W (1999). Framework for teaching and learning informed shared decision-making. *British Medical Journal* **319**, 766-771.

Tuckett D, Boulton M, Olson C and Williams A (1985). Sharing ideas and its importance in medical consultations. In: *Meetings between experts. An approach to sharing ideas in medical consultations.* London: Tavistock.

Ubel PA (1996). Informed consent. From bodily invasion to the seemingly mundane. *Archives of Internal Medicine* **156**, 1262-1263

Ubel PA and Loewenstein G (1997). The role of decision analysis in informed consent: choosing between intuition and systematicity. *Social Science and Medicine* **44**, 647-656.

United Kingdom Central Council for Nursing (UKCC) (2001). *UKCC position statement on the covert administration of medicines – Disguising medicine in food and drink.* London: United Kingdom Central Council for Nursing.

Van den Heuvel WJA, Wieringh R and van den Heuvel LPM (1997). Utilisation of medical technology assessment in health policy. *Health Policy* **42**, 211-222.

Varekamp I, Meiland FJM, Hoos AM, Wendte JF, de Haes, JCJM and Krol LJ (1998). The meaning of urgency in the allocation of scarce health care resources; a comparison between renal transplantation and psychogeriatric nursing home care. *Health Policy* **44**, 135-148.

Veatch RM (2000). Organ allocation. In: *Transplantation ethics.* Washington: Georgetown University Press.

Verhaak CM, Kraaimaat FW, Staps ACJ and van Daal WAJ (2000). Informed consent in radiotherapy. *Patient Education and Counseling* **41**, 63-71.

Vick S and Scott A (1998). Agency in health care. Examining patients' preferences for attributes of the doctor-patient relationship. *Journal of Health Economics* **17**, 587-605.

Watkins EJ, Milligan LJ and O'Beirne HA (2001). Information and consent for anaesthesia: a postal survey of current practice in Great Britain. *Anaesthesia* **56**, 879-882.

Wear S (1998) The value of patient participation in medical decision-making. In: *Informed consent: patient autonomy and physician beneficence within health care.* 2nd edition. Dordrecht: Kluwer Academic Publisher.

Weeks LM and Brooks C (1996). Drug and Therapeutic Committees in Australia: Expected and actual performance. *British Journal of Clinical Pharmacology* **42**, 551-557.

Weston WW and Brown JB (1995). Overview of the patient-centred clinical method. In: Stewart M, Brown JB, Weston WW, McWhinney IR and McWillaim CL (eds). *Patient-centered medicine – transforming the clinical method.* Thousand Oaks, California: Sage Publications.

Whynes DK and Baines DL (2002). Primary care physicians' attitudes to health care reform in England. *Health Policy* **60**, 111-132.

Wilkin D, Gillam S and Smith K (2001). Primary care groups: Tackling organisational change in the new NHS. *British Medical Journal* **322**, 1464-1467.

Williamson PN (1980). Law reform survey – Part 7. Explaining the risks. *Canadian Doctor* **46**, 40-48.

Wilt TJ, Fink HA, Macdonal R, Rutks IR and Schow D (1999). *Treatment options for male erectile dysfunction: a systematic review of published studies of effectiveness.* Boston: Management Decision and Research Center, Health Services Research and Development.

Woodward S and Roberts C (2001). *Consent to treatment – do doctors really understand? Conference presentation "Risk Management".* London: One Great George Street.

Working Group on Acute Purchasing (1996). *Statin therapy/ HMG-CoA reductase inhibitor treatment in the prevention of coronary heart disease.* Sheffield: Trent Institute for Health Services Research Working Group on Acute Purchasing, University of Sheffield.

World Health Organisation (2002). *The European Health Report 2002*. Geneva: World Health Organisation, Regional Office for Europe.

World Health Organisation, Regional Office for Europe (1994). *A declaration on the promotion of patients' rights in Europe. European consultation on the rights of patients.* Copenhagen, Denmark: World Health Organisation Regional Office for Europe.

Wright D (2002). Medication administration in nursing homes. *Nursing Standard* **16**, 33-38.

Wu W and Pearlman RA (1988). Consent in medical decision-making - The role of communication. *Journal of General Internal Medicine* **3**, 9-14.