1. The Australian Health Care System and High Cost Medications (HCMs)

“The extent to which beliefs are based on evidence is much less than believers suppose”. Bertrand Russell

This chapter describes the Australian health care system and mechanisms to access medicines within that system. It also provides a discussion of the definition of High Cost Medication.

1.1 Introduction

Approaches to health care funding differ from country to country. There are three main ways of funding health care in developed countries. 1) General taxation, in countries such as Australia, UK, Canada and Sweden. 2) Social Insurance in Germany, France and the Netherlands. 3) Large contributions from voluntary private insurance, such as in the United States. (4-6)

In developed countries that are members of the Organisation for Economic Co-operation and Development (OECD) the public sector is the main source of funds. In 2001 the public share of health expenditure for OECD countries accounted for 73% of total expenditure on health. (7) In the United States a relatively low share (44%) of the total health expenditure comes from the public sector. Of the members
of the OECD, USA has the highest percent of Gross Domestic Product (GDP) allocated to health (15%). (7, 8)

Expenditure associated with health care delivery has increased widely throughout the developed countries over recent decades. (9) In these countries the proportion of GDP spent on health is approximately 10% (See Table 1.1), with this proportion rising steadily. (8, 10-12) This increase in health care expenditure is largely due to new medical technologies and the introduction of new and more expensive medications. The cost of new medications is increasing and consuming a growing percentage of the total health care expenditure in many countries. (6, 13)

Pharmaceuticals account for over 20% of the total health spending in France and Italy. (13) Expenditure in pharmaceuticals is also one of the fastest growing components of health care cost in the United States. (11, 12)

1.2 The Australian Health Care System

The average GDP ratio spent on health for all OECD countries was 8.6% in 2003. In this same period Australia expended an estimated 9.3% of GDP on health care, (see Table 1.1). (8, 14)

The Australian health care system funding is a combination of private and public sector spending. (15, 16) One of the main characteristics of this system is the division of responsibilities between the Commonwealth and State governments. The financing of medical services, pharmaceutical benefits and aged care is a Commonwealth responsibility. State governments, with diverse levels of financial assistance from the Commonwealth, are primarily responsible for the funding and
operation of public hospitals and other state-based programs such as providing or purchasing ambulance, dental and community health services. (17, 18)

Table 1.1 Health expenditure as a percentage of GDP in 2003. (8)

<table>
<thead>
<tr>
<th>Country</th>
<th>% of GDP</th>
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<tbody>
<tr>
<td>United States</td>
<td>15.0</td>
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<tr>
<td>Switzerland</td>
<td>11.5</td>
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<tr>
<td>Germany</td>
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<td>Canada</td>
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<td><strong>Australia</strong></td>
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<tr>
<td>Sweden</td>
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<tr>
<td>Denmark</td>
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<tr>
<td>Italy</td>
<td>8.4</td>
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<tr>
<td>Japan</td>
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<td>United Kingdom</td>
<td>7.7</td>
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<td>OECD Average*</td>
<td>8.6</td>
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*2002
^2001
#Average of all OECD countries

During 2001-2002, the Commonwealth provided 46.3% of the public funding for health expenditure; states and territories and local governments funded 22.3% and non-government sources such as health insurance funds and patients’ contributions provided 31.4% of funding. (18)

The Australian health care system is based on the premise that residents should have access to health care, regardless of their ability to pay. (15) Introduced in 1984, Medicare is the major part of the national health care system. Funded and
administered by the Commonwealth Government, Medicare is financed largely through taxation. This universal publicly financed system includes benefits such as access to public hospitals at no charge for all citizens and permanent residents. (15-17, 19)

The private sector is also an important component of the health care system. It operates in the insurance and delivery of, health services, receiving both indirect and direct government subsidies. (19) Private health insurance is one of the components of the Australian health care system, which can cover part or all of the direct hospital charges to private patients. (19) In 1997 the Commonwealth Government introduced a series of incentives to increase private health insurance coverage. The reforms included a 30% subsidy to insurance premiums, a tax surcharge for the high-income uninsured and Lifetime Health Cover”. (20) Currently private health insurance is subsidised by indirect payments from individuals in the form of premiums; which are themselves subsidised by a 30 per cent rebate from the Commonwealth Government. (18)

Private insurance provides coverage for a range of services, with accommodation in private hospitals being the largest constituent. (16) In 2003 approximately 44% of Australians had private health insurance. (21)
1.3 Pharmaceuticals

1.3.1 Availability of Pharmaceuticals in Australia

Australia has a two-stage system that distinguishes access and subsidised access to approved pharmaceuticals. (22, 23) The first is the application for registration (licensing approval) made to the Therapeutic Goods Administration (TGA). The second is reimbursement for or subsidisation of the cost of pharmaceuticals. (23)

1.3.2 Registration

Under the *Therapeutic Goods Act 1989*, before a new medication is marketed in Australia it must be evaluated by the Australian regulatory agency the Therapeutics Goods Administration (TGA). The TGA is a unit within the Commonwealth Department of Health and Ageing. (24, 25) Based on assessment of quality, safety and efficacy the Australian Medication Evaluation Committee (ADEC), advises the TGA regarding the registration of a new product. This procedure is followed for every new indication of an already approved medication. (25) After registration, the medication may be marketed for the approved indication. Medical practitioners are able to prescribe a medication once it has been marketed.

1.3.3 Subsidy

1.3.3.1 The Pharmaceutical Benefits Scheme (PBS)

1.3.3.1.1 Section 85

The Pharmaceutical Benefits Scheme (PBS) is funded by the Commonwealth Government, and subsidizes certain medications prescribed and dispensed in the
Private Hospitals are supplied through community pharmacy models and medications accessed through the PBS and private prescriptions. In some states, selected public hospitals have now implemented the PBS as a funding mechanism for outpatient and discharge medications. (26, 27)

 Governed by the National Health Act, (1953) together with the National Health (Pharmaceutical Benefits) Regulations (1960) made under the Act, the PBS is the major source of subsidised medicines in Australia. (17) From 1 May 2004 the PBS included 605 pharmaceuticals, available in 1,508 forms and strengths and marketed as 2,613 different brands. (28)

In order for a medication to be listed in the Pharmaceutical Benefits Scheme there are several stages of evaluation. These are illustrated in Figure 1.1. Only medications approved for use by the TGA for an indication of interest are eligible for submission for consideration for listing on the PBS. The sponsor may submit an application to the Pharmaceutical Benefits Advisory Committee (PBAC) which then recommends which medications should be subsidised by the Commonwealth Government under this scheme. The PBAC considers cost effectiveness of medications as well as efficacy and safety issues in recommendations about PBS scheduling. Since 1993 economic analysis has been mandatory requirement for medications submitted for PBAC listing. (24, 29-32). There are two sub-committees that provide advice to the PBAC: the Economic Sub-Committee (ESC) and the Drug Utilisation Sub-Committee (DUSC). (33) The ESC reviews economic evaluations, cost effectiveness analysis conducted from the societal perspective being required for submissions to the PBAC. (34) The DUSC provides data on medication utilisation.
Under Section 85 of the PBS patients contribute to the cost of subsidised medicines. When a subsidised medication is dispensed, patients contribute a co-payment. As of January 2005, general patients pay A$28.60 per prescription. Patients eligible for concessions (elderly for example) pay A$4.60. (35) However once general patients spend $874.90 on PBS prescriptions in a given calendar year, they reach what is called the “Safety Net”. Once reached general patients pay the concessional rate of $4.60 for further PBS prescribed items for the remainder of

![Figure 1.1 Procedure for listing a medication on the Pharmaceutical Benefits Scheme.](image)
the calendar year. For concessional patients, the patient co-payment per prescription item is removed once they reached the Safety Net threshold of $239.20 or 52 scripts within a calendar year. Further PBS prescribed medicines are provided free for the remainder of the calendar year. (35)

1.3.3.1.2 Section 100 Highly Specialised Drug Program (S100-HSD)

Another section of the Pharmaceutical Benefits Scheme is Section 100. During the early 90s the highly specialised drug (HSD) program was introduced and Section 100 (S-100) of the National Health Act 1953, was established. (36) Section 100 medicines are used for certain chronic conditions, prescribed by specialists and dispensed through pharmacies associated with hospitals participating in the Section 100 "Highly Specialised Drug Program".

These medications are often expensive medications, and include ones for the treatment of cancer, HIV and organ transplantation. Administrative agreements are in place between the Commonwealth Government and the States and Territories to manage this program. In private hospitals, claims are administered by the Health Insurance Commission (HIC), now Medicare Australia, as for community pharmacy. This program is administered by the States and Territories for public hospitals. (31)

Public hospitals provide S100 medications to outpatients. These patients are community based patients, and the prescriptions are dispensed by the hospital pharmacy. (30) Public hospitals do not have access to PBS Section 85 or 100 for inpatients. (23)
The PBAC considers submission for inclusion under special funding and listing in the PBS of these highly specialised medications. In order for a medication to be included in the program it should meet all of the criteria below: (37, 38)

- Ongoing specialised medical supervision required.
- Treatment of longer term medical conditions not episodes of in-patient treatment or treatment of acute conditions.
- Medication highly specialised and an identifiable patient target group.
- Subject to marketing approval by the Therapeutic Goods Administration (TGA) and specific therapeutic indications covered by the terms of the marketing letter from TGA.
- High unit cost.

In 2003/2004 there were 58 medications subsidised under the HSD program and total expenditure increased 13.51% from previous year. (39)

1.3.4 Other Categories of Access

There are different mechanisms to access medications outside the PBS. These include: through a private prescription (where the patient pays the costs), orphan medications, life saving drugs, unapproved medications or supply from public hospitals (where the states may bear the cost). (32)
1.3.4.1 Orphan Medications

These medications are used to treat, prevent or diagnose rare diseases affecting less than or equal to 2000 individuals per year in Australia. Due to their low potential financial return compared to other medications, pharmaceutical companies do not often develop these kinds of medications. The Orphan Medication program aims to overcome this problem by encouraging sponsors to market orphan medications in Australia. The medication is given an “orphan medication” status allowing shorter TGA approval times and no fee is charged for application. (40)

In 2001, of the seventeen orphan medications that had marketing approval, seven had public funding. Two were listed in the Section 100 of the PBS, one was supported under the life saving medication program and the final four were funded on a 50:50 basis by the Commonwealth and the States/Territories. (23)

1.3.4.2 Lifesaving Medications

Life saving medications that do not meet the PBAC cost effectiveness criteria may be funded by the Commonwealth Government through the life saving drug program. Access to these medications is subject to certain conditions agreed by the Minister for Health and Finance and to specified eligibility criteria found on the Commonwealth Department of Health and Ageing website. (41)

The amount of funding for this program is limited and determined on a yearly basis. There are currently three medications to treat rare inherited enzyme deficiencies available through this special arrangement: (41)
- Imiglucerase (Cerezyme®), a medication for the treatment of Gaucher's disease;
- Agalsidase - beta (Fabrazyme®) and Agalsidase - alfa (Replagal®) for the treatment of Fabry's disease

1.3.4.3 Unapproved medications

To obtain an unapproved (unregistered) medicine in Australia there are three mechanisms.

1.3.4.3.1 Special Access Scheme (SAS)

Supply of an unapproved medicine under this scheme is done on a case by case basis for single patients. Circumstances in which an individual patient may have access are: terminally ill patients, products which have been withdrawn from the Australian market, products available overseas but not marketed in Australia. Access varies depending on the patient’s health status. Patients are classified in one of two categories which the prescriber determines. Category A is for patients who are seriously ill “with a condition from which death is reasonably likely to occur”. Category B is for all other patients. (42)

1.3.4.3.2 Clinical Trials (CTN and CTX Schemes)

Patients on clinical trials may have access to unapproved medications. Under the Clinical Trial Exemption (CTX) the sponsor is requested to have TGA approval before the trial begins. Under the Clinical Trial Notification (CTN) the sponsor needs to notify the TGA. (43)
1.3.4.3.3 Importation for personal use

As stated by its name, this can only be done for personal use and strict criteria apply for this importation process. Criteria are available from the Therapeutic Goods Administration website. (44)

1.3.5 Hospitals

There is a mix of private and public provision and funding in the hospital sector in Australia. (16) For example the 2003-2008 Australian Health Care Agreement (AHCA) requires that patients being admitted to public hospitals are given the choice of being a private or public patient, irrespective of their health insurance status or their ability to pay. (45)

Between 2002-2003, private patients accounted for 9.1% of public hospital admissions, an increase of 16.6% from the period between 1998-1999. (46) Private hospitals are a fast growing sector and are largely funded by non-government sources such as benefits paid by private health insurance and out-of-pocket expenditure by individuals. (18) Private hospitals account for 35% of all beds in the hospital sector (public and private hospitals combined). (19) Although the range of services provided in private hospitals is increasing, these are still far less comprehensive than those services provided by public hospitals. (47)

The foundation of hospital care in Australia is provided by public hospitals. (48) Public hospitals deliver the majority of complex and highly specialised services. They provide the major proportion of inpatient and emergency care for the Australian population and carry out almost all of the training of medical, nursing,
ancillary clinical students and specialists. (46, 47, 49-51) According to Duckett nearly all clinical research is conducted in large public sector institutions (teaching public hospitals) rather than private sector health institutions. (50)

1.3.5.1 Access to pharmaceuticals in private hospitals

The Australian government, the patient and third party insurers provide funding for medicines in private hospitals. (1) These medicines are dispensed by community pharmacies and there is access to the PBS. (19, 50) The National Health Strategy identified the PBS as a critically important source of funding for pharmaceuticals. (52) In 2001, Jackson conducted a study aiming at resolving inefficiencies associated with the use of PBS in five private hospitals. In this study PBS-listed medications represented 31.5% by wholesale cost of all medications and non-PBS listed medications 68.5%. Those were private scripts and over the counter medications. (53)

1.3.5.2 Access to pharmaceuticals in public hospitals

Medicines used for in-patients in public hospitals are primarily funded by the hospital under the Medicare Agreements between the States and Territories and the Commonwealth Government. There is no direct cost to public hospital inpatients. (17) The State-based public hospital medicines funding is included as part of the financial grants from the Commonwealth to the States. This means it depends on budgetary allocation decisions made at a number of levels including the health department, health district or area health service and individual hospital. Therefore this funding is capped. (1, 54) During 2002-2003, five percent of the total hospital expenditure in public hospitals was spent on medicines. (55)
The 1998-2003 Australian Health Care Agreement (AHCA) changed access to pharmaceuticals through some public hospitals. One of these reforms included piloting access to the PBS for outpatient, discharge and day patient medication costs. (45) PBS listed cancer chemotherapy medications have been transferred to Section 100 to facilitate access to admitted or non admitted patients in public hospitals. (56) However not all states or territories have entered into this reform agreement. Approximately 30% of the 239 public hospitals with a hospital pharmacy service offer medicines subsidised through the PBS for non-admitted and same-day patients. (57)

Victoria was the first state to introduce the PBS to public hospitals in 2001. (58) As at first of June 2004, 55 hospitals had implemented the reforms in Victoria, Queensland and Western Australia. (27) Inpatient medicines cost still remains the responsibility of the hospital.

Public hospital and private patients may also have access to medicines through clinical trials and SAS. (1)

1.4 High Cost Medications (HCMs)

As described previously, a significant part of health care spending in the developed world is associated with costs of medications. (59-61)

The rising price of pharmaceuticals is often attributed to the use of innovative medicines, some of which are treating diseases that had no treatment. (62) However, according to Henry, high prices could also be attributable to
pharmaceutical companies' patent protection. (63) Patents grant monopoly power which allows pharmaceutical companies to charge monopoly prices. (64) Pharmaceutical companies argue that high prices and patents are necessary to compensate for high development cost of innovative products. (63, Scherer, 1993 #3) Some consider these claims as controversial. (11, 65-67) The pharmaceutical industry is characterised by its extraordinary high profitability, (68) and it has been reported that a great deal of resources are devoted to marketing. (62) Even though the question as to why medicines are expensive is important, a detailed comprehensive review of this matter is outside the scope of this literature review.

“Hospital medication treatment” according to Fijn et al accounts for a large proportion of international medication expenditures. (61) This is due to the fact that hospital treatment may involve intensive and complex medication treatment with “expensive” medications. On the other hand hospitals are often involved in clinical trials of new medications and in off-label or unlicensed treatment. (61, 69)

The main focus of this thesis is the decision-making process used in the allocation of resources to HCMs in public hospitals in Australia. The definition of HCMs is pivotal to this work. The following examples demonstrate there may be several different definitions of high cost medications. These definitions may vary depending on the setting and the perspective of the person or group making the decision. Whether the issue is about adding a new high cost medication to the formulary; (70-72); rationing health care, or seeking government subsidy (73-79) the definition may vary.
More than ten years ago Powe, considered cyclosporine and erythropoietin as high cost medications. The cost of treatment with these medications in 1994 was U$4,000 to $6000 per patient per year in the USA. (80) Powe recognised the impact that expensive medications may have in the USA health care system and specifically on Medicare expenses (for the over 65 years age group). (80) Mason also considered treatment with erythropoietin as expensive, the annual cost to the US government being US $200-500 million. (81) In 1995 Poirier, a Professor of Clinical Pharmacy in the USA defined foscarnet, monoclonal antibodies (MAbs) for septic shock and granulocyte colony-stimulating factors as high cost medications. Poirier discussed the clinical and ethical principles regarding rationing of HCMs but no reference was made to the cost of these medications. The concept of rationing at the hospital level via the Pharmacy and Therapeutics Committee was introduced. (82) In that same year Crump in the UK defined “expensive treatments” as those costing more than £2,000 per patient per year. Examples of these treatments included growth hormone and cyclosporine. The context for this definition was the cost shifting between secondary and primary care in the UK. (83) In a recent paper on access to prescription medications in the USA, Kleinke listed Glivec®, Xigris®, Fuseon® as “supremely expensive”. (84)

Those who have attempted to give a definition have identified two different kinds of high cost medications 1) “modest acquisition cost but used in high volume” 2) “very high cost medication, whose even limited used might create budgetary pressure. (60, 85)
1.4.1 Definitions of HCMs in Australia

Definitions in Australia also vary according to who makes the definition and how they make it. Three points of view can be identified, that of the media, the Commonwealth Government and the States and Territories.

In the media’s hands, high unit cost medications make the headlines. The general public receives messages such as “Expensive medications could blow out scheme” (86) “Expensive arthritis medication to be subsidized” “A$25,000 a person each year”. (87) “Cancer medication gives hope, and a huge bill” the cost would be more than $40 million” or “Glivec® can cost up to $45,000 year”. (88) Robotham commented on funding for trastuzimab, “But it costs $1,031 a week”. (89)

For the Commonwealth Government there are two kinds of high cost medications. The first one is defined by Section 100 of the PBS “Highly Specialised Drug program” criteria, which states if medications are to be recommended for inclusion in the program, they have to have a “high unit cost”. (38)

On the other hand medications like atorvastatin, omeprazole or celecoxib are also considered high cost medications because they are used in high volume. (90) Lu also described these two types of high cost medications when discussing access in Australia. (91)

States such as New South Wales and Victoria have also provided definitions for high cost medication. In 1997 in New South Wales (NSW), high cost medications for outpatient use not funded by the Commonwealth Government were defined as:
those not listed for subsidy on the PBS Section 85 or 100 of the National Health Act, and which incur an acquisition cost equivalent to, or more than A$100 per week per medication per patient and require particular expertise for management of patient care. (92). This definition was only updated in October 2004 when the acquisition cost was changed to A$500 per week per medication per patient. (93)

In Victoria the Victorian Therapeutic Advisory Group (VicTAG) defined high cost medications for inpatient use in public hospitals as “those costing more than A$1000 per treatment episode and for outpatients as costing more than $100 per week per patient and not covered under Section 85 or 100 of the Pharmaceutical Benefits Scheme (PBS)”. (54)

A key issue for public hospitals is the cost of pharmaceuticals for inpatients. Public hospitals deal with capped budgets, scarce resources and cost shifting between different funding systems. (16, 49, 94, 95)

There may also be equity of access issues between public hospitals and the private sector. Private hospitals have access to medication through the PBS and this is an uncapped budget in contrast to the capped budget for state public hospitals. (96) According to the current AHCA agreement medications that are listed on the PBS (Section 85) solely for inpatient use (for example drotrecogin alpha – used to treat patients with sepsis in Intensive Care Units) cannot be subsidised for inpatient use in public hospitals. Therefore subsidised access to these medications can only be obtained through private hospitals. (54)
It was estimated that 25% of total expenditure on medicines in Victorian public hospitals was due to high cost medicines (as defined by VicTAG). Many of these medicines could not be accessed through Section 100 or 85 of the PBS, the main reasons being: a) they were not listed or not listed for all the indications used or b) not available to inpatients. (54)

While there is uniformity across Australia with the PBS, the same cannot be said for the availability of medicines to patients using public hospitals. (97) There are state based formularies in Queensland and Western Australia, (98, 99) hospital based Drug and Therapeutics Committees (DTCs) in public hospitals or local medical administrators. (97)

Cost shifting is a well established practice, but is reactive and will be practised more widely as regulatory requirements or agreements are changed. The level and decision making process to access medicines is unclear. There is a confusing mixture of rules and guidelines governing the availability of medicines in public hospitals. (1, 96, 97, 100)

Public hospitals are often exposed (via Clinical trials) to new medicines or new indications before PBS listing having to cover the gap between registration and subsidy. (1, 54)

Despite the recognition of these problems, limited work has been conducted regarding use and funding of High Cost Medications (HCMs) in public hospitals in
Australia. Furthermore there are no reported studies on how decisions are made to allocate resources to HCMs.
2. Decision-making and Priority Setting in Health Care

“The allocation of resources between competing demands is both an economic challenge and a political puzzle.” Chris Ham

This chapter describes the literature on levels of decision-making priority setting in health care and the role of the public.

2.1 Introduction

Carroll defined decision-making as “a process by which a person, group or organisation identifies a choice of judgment to be made, gathers and evaluates information about alternatives, and selects from among alternatives.” (101)

Under conditions of scarce resources, health care decision-making has been described as different and complex. (102-104) Decisions in health care could involve patients choosing between treatments. (104) It could also involve physicians or health care policy makers. (105) The focus of this section of the literature review is on decision-making about allocation of resources (priority setting) in health care at the institutional level, with a special focus on high cost medications.
Allocating resources or assigning priorities is also known as priority setting or rationing. The terms rationing or priority setting have been used interchangeably in the literature. Rationing has had a negative association, implying the denial of access to health care resources. (106, 107) It has also been defined as “societal toleration of inequitable access to services deemed necessary, as defined by reference to appropriate clinical guidelines”. (108) On the other hand the term priority setting has had a positive connotation. (107) Some consider that the language has evolved from “rationing” to “resource allocation” to “priority setting” and might move even more towards “sustainability”. (109) Whatever term is used, the concept relates to determining ‘who gets what and at whose expense’ and incorporates the notion of ‘winners’ and ‘losers’.

In general terms, as stated by Klein, it is important to understand how decision-making and priority setting in health care is being done (process) and the structure of decision-making. (110) Decision-making and priority setting occurs at different levels: the national or “macro” level, “meso” or institutional level and “micro”, individual patient level. (110-113)

There are also different levels of decision-making and priority setting about health care technologies such as HCMs. At the macro level, it includes assessment for reimbursement (for example decision-making regarding listing on the PBS). At the meso level, decisions are made by hospitals, through Drug and Therapeutics Committees (DTCs), whereas at the micro level, patients and doctors make choices about individual treatments. (114, 115)
2.2 Levels of Decision-making priority setting in health care

2.2.1 Macro – Government level

At this level policy decisions are made about the allocation of resources to specific sectors or groups within a health care system. (110)

In Australia, an example of decisions re HCMs at the macro level is the process for PBS subsidy of selected pharmaceuticals. Duckett has described the PBS as a “rationing” process. (116) More than 10 years ago, Australia was the first country to introduce cost effectiveness as a mandatory criterion for reimbursement of pharmaceuticals in 1993. (15, 117, 118) Hall noted that the PBS was not the first attempt to include economic evaluation in decision-making about health care resource allocation. (15) Even though the PBS has an uncapped budget, overall government resources to spend on health care are limited. Spending in one area may mean less allocation to other (health) areas. (119)

As previously described, the Pharmaceutical Benefits Advisory Committee (PBAC) considers cost effectiveness of medications in recommendations about PBS listing. (24, 29-31) The PBS exemplifies decision-making at the macro level in Australia. (120) For George, the public decision-making process for reimbursement of pharmaceuticals in Australia is consistent with economic efficiency. However the author also acknowledged that this is not the only factor that the committee considers when assessing medications proposed for listing on the PBS. (121)

In 2000, Cookson conducted a case study of the PBS. Despite being considered a success by stakeholders, there were several criticisms about the decision-making
process. These included the methodology, transparency and accountability. Findings from the interviews showed that some considered that the process lacked transparency, there was no consumer involvement in the design of the “fourth hurdle” and there was insufficient consideration of equity. (122)

The PBAC membership was not made public until 1973 and the first consumer representative joined the PBAC in the late 1990s. (123) In 2001 the Department of Health and Ageing started publishing on its website a quarterly summary of the PBAC’s positive recommendations, with a brief summary of the basis on which each approval was made. (124) Although welcomed as a positive move, Lopert and Henry considered there was still the need for more information on the decision rationales. They suggested that the information on those medications that had been rejected and the grounds for these rejections should also be available. (125, 126)

In 2004 Sansom (the chair of the PBAC) stated that the need for greater understanding and transparency of the process is essential. (33) However Sansom acknowledged there are limitations to full and open disclosure of reasons for decisions. (33) Two sides of the transparency issue are presented by the Editorial Executive Committee of the Australian Prescriber. One side is the fact that pharmaceutical companies have been hesitant to make public the information they have submitted to the PBAC. (127) Some of the data submitted by the pharmaceutical companies are “commercial in confidence”. (128) The second side is the release of information by the PBAC. The free trade agreement has enabled the PBAC to release information about how it reaches its decisions. (127)
Other countries use pharmacoeconomic and/or health economic information in health-care decision-making. Like Australia, the Canadian province of Ontario has set pharmacoeconomic guidelines for the pricing and reimbursement of pharmaceuticals. (120, 129) Pharmacoeconomic evaluation is also used in Portugal, Sweden, Belgium, Norway and Finland for national formulary listing. (125, 130, 131)

Despite the uptake of economic evaluation, authors such as Ubel criticize it for focusing on efficiency and not considering equity. (132) Holm and Ham et al have expressed that there are no simple or technical solutions that may help decision-makers in the allocation of limited health care resources. (133-135) The focus, according to Ham, should be on the decision-making process and securing legitimacy for decisions made. (112)

2.2.2 Meso – Hospital or Institution
An example of decisions made at the meso level includes those made by institutions/individual hospitals. (113) Decisions are made about the allocation of hospital budgets. (136) Ham and Coulter suggest that there is a need to strengthen the institutional processes in which decisions are taken. (135) The meso level is, according to Martin, where much of the priority setting within a health care system takes place. One-third of Canada’s public spending on health is related to hospitals. (113) However there is limited published literature regarding decision-making and priority setting at the institutional level. (113, 137)
Martin has used case study methods to describe the decision-making process for setting priorities for new technologies in public hospitals in Canada. (138-141) Priority setting for HCMs has also been conducted. (76, 142, 143)

At the public hospital level, most decisions to allocate resources to medications including HCMs are taken at hospital Drug and Therapeutic Committees (DTCs). Bochner claims the DTC seems the appropriate body for drug rationing. (137)

2.2.2.1 Hospital Drug and Therapeutics Committees (DTCs)

Hospital DTCs have been defined as “policy makers at the institutional level for ensuring expenditure on medicines provides good value”. (144) DTCs may have a number of roles and functions within the organisation. Determining what medicines are available in the hospital is one of the primary roles of the hospital DTC. (145),(146) Hospital DTCs have the task of constraining medication costs and ensuring the Quality Use of Medicines. By analysing drug usage trends, DTCs have become more involved in cost containment and predicting budgetary needs. (9)

Hasle-Pham et al conducted a study on the decision-making process for selecting medications in European public hospitals. Sixty-six per cent of the participants thought that, besides helping in pharmacy management, the hospital formulary was an educational and economic tool. (146) According to Jonsen, drug formularies should ensure patient welfare and organisational efficiency. (147)

In interviews conducted with Directors of Pharmacy Services in Managed Care Organisations (MCOs) in the US, the majority expressed increasing concerns about
the decision-making process for formulary listing. The leading reasons cited were the higher price of new medications and the increasing pressure for both cost cutting and improvement in quality of care. (148)

Different problems with regards to formulary listing have been identified in the literature. The unit cost of new medications may be higher than the medications they are replacing. (120) Medications are being developed for conditions that had no previous treatment. In some cases the biotechnology industry has produced designer medications for uncommon diseases. (149)

Reports show early concerns about allocating resources for HCMs and the complexities of the formulary decision-making process. (70, 150) In 1998, Sapienza showed that 66% of the formulary managers in the US expressed their concern about the impact of the cost of biotechnology medications. They thought that resources used for these medications could otherwise be available for alternative purposes. (72) Odedina found that less than half of the members of the Pharmacy and Therapeutics Committee (PT&C) (USA equivalent of DTC) considered formulary decisions re HCMs easy to make. (151)

One of the criticisms of formulary decisions has been a lack of transparency in decision-making and inadequate publicity given to the decision-making process. Daniels believes the decisions about contents of a drug formulary should be publicly accessible to clinicians, patients and citizens. (152)
2.2.2.2 Australian Hospital Drug and Therapeutics Committees.

In a study conducted to identify the most common ethical issues of concern in Australian hospitals, McNeill et al found that 51% of the respondents were concerned about the allocation of health care resources. Public hospitals reported these concerns more often than private hospitals. (153)

As previously described, hospitals deal with decision-making regarding the allocation of resources to medications through multidisciplinary DTCs. In 1982, 60% of Australian Hospitals had a DTC. (154) By 1995 the percentage had increased to 92.6%. (144) In New South Wales (NSW), it is mandatory for all public hospitals to have a DTC or have access to an Area Health Service committee. (155)

The main role of the DTC has been to advise on medicines to be listed in the hospital drug formulary. (144, 156, 157) However, according to a survey conducted by Tan these roles have expanded to include: (157)

- Promoting evidence based medicine
- Developing and maintaining formularies
- Governing clinical practice
- Acting as financial gatekeepers
- Ensuring patient safety

The DTC may also be involved in decisions about access to non formulary items (this may include HCMs). This is addressed in Chapter Three.
In NSW the roles for DTCs according to the Department of Health include: (155)

- Promotion of quality and cost effectiveness use of medicines
- Development and approval of drug policies
- Approval of drugs within the hospital
- Analysis of medication reports and development of strategies for medication error prevention.

Shenfield supported the use of DTCs in Australia for several reasons: resources are limited, expensive does not always mean better and “someone has to make an evaluation and decision about whether resources should be used for new drugs” (158, 159)

Studies of Australian DTCs have shown that besides quality drug use and drug policies, spending on high cost medications has been reported as an important issue. (160) The increasing expenditure and implications of a capped budget were also important for most of DTCs. (144, 157)

There are criticisms of Australian DTCs in the literature arguing that DTC decision-making lacks transparency and proper working procedure. (144) Bochner et al and Weeks et al showed that the formulary decision-making process was considered a complex task and it lacked transparency and consultation with community groups. (144) It also lacked an appeals mechanism, inability to properly measure the impact of decisions and the quality of the information used for formulary decisions was inadequate. (144, 161)
Different strategies for resource allocation have been applied in other countries. (61) Some of the possible approaches described by Shenfield include: (159)

- **Direct funding**: provision through PBS Section 100, transferring the cost to the Commonwealth Government. However, Shenfield stated this “totally removes the nexus between the fund provider and the prescriber and consequently makes rational prescribing more difficult to achieve”.

- **‘Absolute dictator’**: no purchase of new expensive drugs is permitted. This is not a feasible approach for tertiary referral institutions.

- **‘Police state’**: this is done by developing strict guidelines and policing the criteria for access to a medication.

- **‘Benevolent dictator’**: the DTC designates a fixed budget to a prescriber or a given unit.

Studies have noted that there are differences between public and private hospitals in their decision-making processes and formulary management, due to their different funding systems. (144, 157) For example, it is perceived that public hospitals have ownership of decision-making in their hospital since decisions happen at the local level. They also have “budgetary control”. (162)

Despite the fact that work has been conducted to measure the performance of DTCs (163) the decision-making process to allocate resources to HCMs by DTCs has not been explored extensively. (160, 164)
As stated in the report “The state of our public hospitals, June 2004”: “all governments are accountable for their expenditure on public hospitals. Each state and territory government is accountable for the performance of its own public hospitals and for making decisions about how it allocates dollars to get the best health outcomes for its community”. (46) However little is known about the decision-making process to allocate resources in public hospitals.

There is limited published work in Australia regarding health care decision-making at the meso or institutional level and/or priority setting for HCMs. Three researchers have published in this area: Bochner conducted a study on rationing of drugs in hospitals; (137, 161) Weekes undertook a case study about the process used by a DTC regarding the availability of a high cost medication to a specific patient group; (164) Tan conducted a national study of DTC members regarding the use of ‘importance’ of a DTC decision as a means of prioritising implementation of policy. One of the hypothetical scenarios in that study was related to access to HCMs. Surveyed DTC members considered that DTC decisions regarding access to high HCMs have high priority for implementation. (160)

2.2.3 Micro- Individual

The micro-level may relate to individually based decisions and that focus on individual patient-doctor relationships. At this level of decision-making and priority setting Klein considers there are two dimensions. These are decisions about how to prioritise access to treatment between patients and decisions on how much to spend on individual patients. (110)
Also known as bedside rationing, the doctor’s role in decision-making and priority setting has been discussed extensively. (165-173) Some commentators considered that cost should never enter into doctor’s decision-making at the bedside. (165-168) Those against bedside rationing suggest that health care rationing should occur at higher levels, such as DTCs, by restricting medications available to clinicians. (168)

An alternative view is that doctors have a responsibility towards society and should ration scarce resources. (173-176) Arnesen considered that individual level decisions not only affect patients, they also affect society, which bears the cost. Society and individuals may have conflicting values. Patients benefit from unrestricted use of resources whilst society benefits from restricted access. (177) Resources allocated to one patient may have an opportunity cost and therefore those resources will be unavailable within the health care system to treat other patients. (178) The doctor then has a double role: 1) as provider of care and patient advocate, 2) societal agent. (59, 105, 177, 179)

However, doctors may not be able to fully appreciate the societal cost. (174) By assuming their role as patients’ advocates they might not recognise just distribution of resources. (180). Coast considers that rationing is administered by doctors who are aware of limits on budgets. (181)

Doctors’ responses to resource constraints and access to HCMs varies. A study of physicians in the United States showed that 64% were concerned about the cost of expensive interventions such as chemotherapy. (180). In a Norwegian study, 68%
of the doctors reported having refrained from giving the best treatment to a patient because it was regarded as too expensive. (177)

Not all doctors deal with decisions about individuals. Those involved in management roles deal with decisions concerning populations. (182) They may also play a crucial role as a source of expertise and information on hospital formulary committees (DTCs). (172) Although formulary committees must think about the moral implications of their decisions (183), doctors working on formulary committees are not constrained by the same moral duties that apply when they work directly with patients. (172) As stated by Ubel “Physicians who limit the use of high cost medications through DTCs are not involved in bedside rationing but are making population based, organisational-level rationing”. They are involved in meso/institutional priority setting decisions.

Ubel acknowledges that doctors can influence patients’ decisions because of their knowledge and power. They make value judgments about which treatments are worth the cost. (172) However, patients are becoming less passive and more active by informing themselves and seeking more information from their health care providers. (184) As a consequence, decisions about treatment are more likely to be discussed and shared between patients and doctors. Patients’ values and goals may then be considered in the decision. (172)

Where this occurs the doctor acknowledges the legitimacy of the patients’ decision and the patient accepts the shared responsibility for the treatment decision. (185) This is important because as stated by Ham “ultimately resources used in most
cases are determined by the decisions and treatment choices made by individuals and their patients. “(112) People’s values, beliefs and preferences have a crucial influence on the choices that are made. (112) These values, according to Ubel, should play an important part in decision-making and priority setting in health care. (132)

In Australia there is little published work regarding doctors’ perceptions about decision-making and priority setting about HCMs at the micro level. Thompson surveyed Australian oncologists regarding factors influencing the discussion with patients regarding access to HCMs. (186) In this study, physicians avoided discussing the high cost of the medication with patients and families to avoid causing distress. In this study the circumstances where they would do so would be if the patient was privately insured, wealthy or if the patient specifically wanted to know their treatment options. (186)

2.3 Decision-making priority setting in health care and the general public

There is an increasing interest in involving the general public in health care priority setting. (187) In the early 1990’s, the public was involved in setting priorities in health care in New Zealand. (188-190) In 1992 in the Netherlands, the Committee on Choices in Health Care produced what is now known as the Dunning report. In the mid 90’s the state of Oregon in the United States made an unsuccessful attempt to make explicit health care rationing by involving members of the public. (191-193) The main problem with the Oregon experiment, from Hardon’s view point, was that the final list of priorities lacked specificity with regard to conditions and treatments.
However, this did not reduce the interest in including community preferences in priority setting in health care.

The New Zealand and Oregon cases are examples of decisions at the macro level; however the public could be involved in different levels of decision-making and priority setting from the macro through to the micro level. (194).

The importance of general public participation in decision-making and priority setting has been debated widely. (195) From Ubel’s perspective, fairness of decisions might be enhanced by public participation. This author claims the general public uses a more balanced range of principles on rationing compared to those used by professionals. (173) It is also perceived that community values play an important role in health care priority setting. (132)

The current shift towards including general public participation has been explained in different ways in the literature. One is the desire to make providers more accountable to the people they serve. In the United States, Health Maintenance Organisations (HMOs) are trying to reduce their liability by shifting decision-making responsibility from providers to patients. (104) Mossialos expresses that some of the burden of making difficult choices could be shifted to the public and as a consequence, make health care decision-making more transparent. (196) Citizens, according to Charles, can also bring different knowledge to the decision-making process. (197) Secondly, as previously expressed, members of the general public values and preferences differ from those of health care professionals. (198-200)
According to Peeters there seems to be a lack of community participation in prioritisation and rationing in health care in Australia. (201) There have been attempts to improve public participation at different decision-making levels. (202) The National Medicines Policy (NMP) recognises the fundamental role of consumers in achieving the objectives of the policy. One of the main four aims of the NMP is access to medicines. (203)

Initiatives by the Commonwealth Government to engage consumers in health care (in general) have included: the Consumer and Provider Partnerships in Health Project (CAPPS), the Consumer Participation Conference in May 2001 and the creation of The National Resource Centre for Consumer Participation in Health (NRCCPH). (34)

One of the publications from the NRCCPH, “The report on consumer participation in resource allocation”, (204) is a comprehensive summary of methodologies for eliciting consumers’ views and involving the public in health care resource allocation. (204)

A study by Johnson, also conducted through the NRCCPH, explored the community’s and consumer participation in Australia in health care services (205). Although this study focused on consumers’ input into health services and not decision-making, some of the findings are relevant to priority setting at the hospital level. Johnson showed that hospitals did not have strategies to involve consumer participation and lacked coordination of, and planning for, consumer and community feedback and participation. It also suggested that community and consumers
seemed to have a passive role in decision-making in hospitals. Few effective community working groups have been developed and there is a lack of shared decision-making. (205) To exemplify this Tan reported that of 124 hospital DTCs surveyed in Australia only nine had a consumer representative. (160)

In spite of the Commonwealth Government commitment to encourage a “stronger, more active role for consumers at all levels of the health system” (206) funding from the Commonwealth Government for the NRCCPH ended the 30th of June 2004.

At the state level, NSW developed a resource kit for community participation and consultation. This was meant to assist Area Health Service staff with community consultation. (207)

There are published studies on community participation in decision-making and priority setting in Australia. (195, 200, 202, 208-210) Nord conducted a postal survey of Australians regarding the importance of costs in prioritizing health care services (macro level). The survey addressed the following issue:

“In our society there is not enough money to give to all patients and all the health care they want. There is a shortage of donor organs for patients in need of organ transplantation. In practice this means that some patients get treated more quickly than others. It can also mean that some patients receive certain kinds of expensive treatment while others do not. In both cases we may say that some patients are given}
priority over others. On what basis should priority be given? This is the question that we ask you to consider in this study.” (209)

Nord found that the costs of treatment were not given high priority in determining priorities between different diagnostic groups. (208, 209) As follow up to the postal survey, personal interviews were also conducted and the same results were obtained. The methodology used by Nord in this study was the “person trade-off”. (208, 209) Alexander and Hicks used focus groups, community meetings and telephone interviews with hospital staff, managers, groups of consumers and community members to rank six possible policies for health care in order of preference for investment of funds in a public hospital in South Australia. Alexander and Hicks found that decisions about resource allocation were consistent across groups of consumers, hospital staff and the community. (210)

Mooney et al surveyed (postal and a door to door survey) South Australians to elicit preferences with respect to equity in resource allocation. Respondents were asked to express how they believed resources should be allocated across different groups in the community. (202) The methodology used by Mooney in this study was “conjoint-analysis”.

Wiseman conducted face to face interviews with patients attending two clinics in central Sydney (195) In Wiseman’s study, participants expressed a strong preference for general public involvement to inform priority setting decision in health care. However the desire of the participants to be involved in decision-making priority setting in health care varied according to the type of decision being made.
Participants were less willing to be involved in decisions regarding “specific medical procedures”. It is important to note that this study included patients and not members of the general public. (195)

Citizen’s juries have been trialled in Western Australia (WA) as way to introduce greater democracy into decision-making priority setting in health care. (200) One of the conclusions drawn by Mooney is that citizen’s juries are capable of dealing with complex concepts such as “equity” and can provide meaningful advice on issues regarding health care resource allocation. (200) Other published papers on community participation in decision-making and priority setting comment on ethical issues and methodologies like program budgeting and marginal analysis (PMBA). (194, 211, 212)

However, to date there have been no published studies in Australia regarding public involvement in decision-making priority setting for HCMs. This is despite the public considered to be a major stakeholder in decisions that will affect members of the public. (213)

In 1994 the Australian Consumers’ Health Forum (CHF) held a workshop on consumers and pharmaceuticals. Participants in the forum ranked access to HCMs as a problem. (214)

In Sweden Lindblad explored patients views of priority setting for high cost pharmaceuticals. (77) This study found that patients felt there should be no priority setting. Respondents also considered that patients might have reasons to refuse a
new treatment and patients’ opinion should be considered. (77) In the UK Richards
described that each high profile medication launch prompts public discussion about
what a national health service should provide and who should decide. (215) In the
USA Meli considered the public has a growing role in medication resource
allocation decisions. (213)

2.4 Decision-making priority setting in health care and ethics.
The literature on the ethics of priority setting in relation to health care resources is
extensive. In this section, only the ethical framework “accountability for
reasonableness” will be described. If health care resources are limited, should
people be given a fair chance to benefit or should decision-makers favour what
produces the best outcome based on evidence? (216) A utilitarian viewpoint will
consider maximizing the benefit by directing resources to the group of patients with
the greatest benefit. (217, 218) An egalitarian approach would favour random
allocation, such as the lottery where everyone has the same chance to benefit.
(219, 220)

According to Daniels et al, the methodology for resource allocation decisions in
health care should be ethically sensitive. The process should be fair and publicly
acceptable allowing decision makers to be confident. (216)

Accountability for reasonableness (A4R) is grounded on theories of justice and
emphasises democratic deliberation. It was developed in the United States within
HMOs. (221) Currently it is a framework used extensively in research conducted on
priority setting, including papers cited in this review. (139-141, 222-224)
Accountability for reasonableness is built on the premise that processes need to be fair. To make legitimate and fair decisions on priorities, four conditions must be met ([221, 225]):

- **Publicity**: the public should have access to the decisions being made. The reasons for decisions should also be transparent.
- **Relevance (reasons)**: decisions must rest on reasons that “fair minded” participants (managers, clinicians, patients, and consumers in general) can agree are relevant to deciding how to meet the diverse needs of a particular population given necessary resource constraints.
- **Appeals**: There should be mechanisms for challenging and disputing decisions in light of further evidence or principles.
- **Enforcement**: There must be voluntary or public regulation of the process to ensure that the first three conditions are met.

The aim of this thesis is related to HCMs, therefore it is important to describe the relevance A4R framework has in this area. The authors of this framework stated that “Managing access to pharmaceuticals is a microcosm of the limit setting problems of health care systems as a whole…. If we are right that accountability for reasonableness is a solution to the legitimacy problem in health systems as a whole, then it should be possible to illustrate what such accountability would mean in practical terms in pharmacy benefit management.” (216)

In summary, the literature shows there is difficulty in reaching an agreement on “what” decisions should be made in health care priority setting. (135) There is no consensus on “how” to reach decisions. As stated by Martin et al there is little
known about how people make decisions with real consequences. There is a need for exploration into real life decisions and how decision makers make them. (226)

There are gaps in the literature as to how decisions are made, and in particular, the decision-making process to allocate resources to HCMs in public hospitals. It is important to evaluate how individual perceptions influence and assist in the decision-making process. Attitudes and concerns among health care decision-makers and the perceptions of the community-at-large, about access and allocation of resources to HCMs should be explored.

What follows is a series of studies designed to explore and describe the decision-making process for resource allocation for HCMs in public hospitals; furthermore the views of the stakeholders involved in this process, the decision-makers and the general public are sought.
3. The Financial Impact of Approval of Medications for Individual Patient Use (IPU) in a Public Hospital

“Knowing man cannot choose but pay, how have we cheapened paradise?”
Freemantle N.

This chapter describes the Individual Patient Use (IPU) scheme in a public hospital and provides a review of the impact of this scheme on medication expenditure, which arises when there is a need to prescribe a non-formulary medication.

3.1 Introduction

In the early 90s, it was reported that Drug and Therapeutics Committees (DTCs) in Australia considered that formulary management was the most common function of the committee. (227) Formularies have been described as a method for controlling medication expenditure, reducing waste and promoting rational prescribing. (9, 183, 228) There has been a variety of responses to formularies, as some may be seen as restrictive or limited and others as open. (156, 229, 230) However, there is no such thing as an ideal formulary that can be applicable to every patient situation. (231) In Packer’s words “even the best formulary cannot be expected to meet the needs of every patient in all situations”. (232) Therefore the need to prescribe non-formulary medications may arise. (233)
Structured non-formulary requests have been described in different hospitals in the USA and elsewhere. (231, 232, 234-236) The process that each hospital uses varies. For example, at Saint Vincent Hospital in Massachusetts (USA), decisions on individual requests are made at the level of pharmacy supervisor. Non-formulary medication use is documented and tracked for prescribing trends and financial impact. (237) At the Howard’s University Hospital in Washington, in order to obtain a medication not listed in the formulary, prescribers fill out a special request form. (238) At the Göttingen University Hospital in Germany, non-formulary requests are made by filling out a form, including the reason for requesting the medication, patient’s name and signature of the responsible physician. (239)

Analyses of non-formulary medication requests have been used as indicators to assess the success of a hospital formulary. (240) Restrictive and effective formularies should have fewer non-formulary requests, according to Green. (240) However it has also been reported that open access to non-formulary medications improves clinician satisfaction with the formulary process and support for important cost containment policies. (230)

Drug formularies are primarily aimed at improving quality use of medicines however they may be employed to allow control of medication expenditure. (228, 230, 241) In Sloan’s study in the US, three-fifths of the hospitals spent less than 5% of total medication expenditure on non-formulary medications. (236) However continuous intervention is necessary for the formulary to achieve the objective of reducing costs. (242)
To give a specific example, at St. Vincent’s Hospital (SVH) in Sydney, Australia, the formulary is a list of medications approved for use within the institution under approved guidelines for use. (243, 244) However if a hospital doctor wishes to access a medication which is not listed in the formulary he/she may submit an application through the Individual Patient Use (IPU) approval program. The IPU scheme is coordinated by the SVH DTC, and aims to provide access for patients who may require medication therapy that is not included in the SVH Formulary. (244)

The request for a non-formulary item may occur in a number of possible scenarios. This includes but is not limited to:

- A high cost medication with a low level of evidence to support use in the specific indication.
- A high cost medication with a high level of evidence to support use in the specific indication.
- A low cost medication with a low level of evidence to support use in the specific indication.

Whatever the circumstance, the medication is not available on the hospital formulary, nor has it been approved by the hospital for the intended indication. The IPU scheme is used to allow prescribers to request access to non-formulary medications in exceptional cases.
There is anecdotal evidence that some hospitals in Australia have developed guidelines for use and approval of IPUs, (162) however there are no published data. The outcomes of the IPU schemes have not been assessed. Outcomes of interest include the clinical consequences for the individual patients, as well as the health care costs involved. Comprehensive data may be difficult to collect and interpret. There are limitations in documentation, follow up, and availability of comparative data. Thus this study was designed to explore how these issues were approached in one Sydney teaching hospital.

3.2 Aim

The aims of this study were to:

- document and describe the IPU Scheme at St. Vincent’s Public Hospital, Sydney
- assess the financial impact of the IPU Scheme on the hospital medication expenditure.

3.2.1 Methods

3.2.1.1 Setting

The project was conducted at St. Vincent’s Public Hospital (SVH) in New South Wales (NSW), a 300-bed university-affiliated, principal referral metropolitan hospital. This hospital specialises in heart/lung transplantation; bone marrow transplantation; cardiology; HIV medicine; respiratory medicine; mental health; and medication and alcohol services. (245)
3.2.2 Data Collection

Documents such as IPU scheme records, Department of Pharmacy and DTC records were reviewed and, where appropriate, clarification was sought from staff to document and describe the IPU scheme. All submissions for IPU received between Jan 1997 and Dec 2001 were reviewed. Submissions were collated on a calendar year basis (January through December). Sources of data included Department of Pharmacy records, Medication Use Evaluation data records, DTC records, dispensing systems such as MEDRECORD (Version 5.36 DOS) for medications dispensed before November 1999 and STOCCA (Paramedical STOCCA for Windows Version 3.7) for medications dispensed after November 1999.

Acquisition prices of medications were obtained from the 2001 inventory catalogue provided by STOCCA in which all prices are expressed in Australian dollars. Information regarding the status of the medication indication being approved was obtained from a range of sources such as the Schedule of Pharmaceutical Benefits (PBS), MIMS and the Therapeutics Goods Administration (TGA) 1800 contact line. (30, 246-250) Data collection included: medications, approved indications, status of the indication being approved (see below), prescriber, prescribing team, outcome of the therapy, person reviewing the submission, approval date duration and expiry, cost of therapy. The proportion of overall medication expenditure was calculated. All patient data were de-identified.

IPU approvals were excluded from this analysis if details such as course of therapy and duration were not available, or if cost was less than $100 per year per patient.
3.2.3 Data Analysis and Statistics

A Microsoft Excel spreadsheet (Microsoft Excel Microsoft Corp., Redmon WA; Version 2000) was used. Data were entered and validated by performing manual checks against original sources. Data for submissions and approvals were grouped by calendar year. Each year was grouped separately in terms of medications and status of the indication being approved at the time of the approval. The following categories were used to differentially describe approval status:

- Special Access Scheme (SAS): unregistered, unapproved medication made available under the provisions of sections 19 and 18 of the Therapeutic Goods Act. (32)
- Non-TGA approved indication: medication marketed in Australia but not for the intended indication (off-label).
- TGA approved indication and not listed under Section 100 of Pharmaceutical Benefit Scheme subsidy criteria.
- TGA approved indication and listed in Section 100 PBS subsidy criteria.

Data collection also included the prescriber’s specialty group (medical/surgical team), outcome of the therapy, the person assessing the submission, the amount of medication actually dispensed, the cost per unit of medication dispensed, the annual cost and percentage of the total medication expenditure.
Data were summarised and proportions for medication expenditure and annual cost were compared using the chi-square test for linear trend. Significance was set at the 5 per cent level.

3.3 Results

The process for IPU submission at SVH is outlined in Figure 3.1. The prescriber filled out an IPU request form. This form was then sent to the DTC secretary who is the Director of Pharmacy (DoP), who made the initial assessment. Depending on the complexity and urgency of the request, the DoP would either review the request or ask for further evidence, such as literature review, to support the indication. Following this, the request may be approved or forwarded to the chair of the DTC. For seven IPU submissions (1999-2001) the DoP made a recommendation to the next meeting of the full committee of the DTC. There were circumstances where acute conditions needed immediate attention and decisions were made out of session of DTC meetings.

The following criteria were necessary to fulfil the IPU submission:

- The medication requested was not on the SVH formulary.
- There were no clinically relevant alternative formulary medications

In the IPU submission required information included: proposed dose; duration of treatment; cost of treatment and plans to follow up; relevant patient history details and previous treatments; evidence to demonstrate that any relevant formulary medication(s) had been tried and/or was (were) inappropriate; evidence of efficacy and safety.
If the medication was approved an annotation was entered in the STOCCA dispensing system, including the date of approval, indication and duration of the therapy. Details of the decision and reasons were communicated to the prescriber.

A total of 340 IPU submissions (resulting in 323 approvals) between 1997-2001 were reviewed. From the documents reviewed, once a medication was approved for a certain indication, submissions for other patients with the same indication were always approved. A total of 33 literature reviews were supplied, for 8.8% of the total submissions.

There was an apparent trend that IPU submissions and approvals have increased through recent years as shown in Figure 3.2. However there were a number of medications that despite being approved were never dispensed to the patient, 13 in total. This was due to changes in the clinical status of the patient. Two submissions were withdrawn after being submitted.

IPU approvals for 1997 (40) and 1998 (61) were excluded for further analysis since data such as course of therapy and duration were not available due to limitation in data retrieval from MEDRECORD (Version 5.36 DOS). The number of IPUs excluded due to medication costs less than $100 was unavailable.
Figure 3.1 Process of submission and approval of IPU at St. Vincent's Hospital
From 63 submissions in 1999, 61 new IPUs were approved for 31 different medications and 51 patients. Mycophenolate (11 approvals) accounted for 18% of the approvals, followed by octreotide, erythropoietin and cyclosporine with 8.2% each.

In 2000 there were 77 approvals, for 37 medications in 75 patients. In this same year mycophenolate was the most frequently approved IPU request with 22.9% of the approvals, followed by pamidronate (12%), cyclosporine and tacrolimus with 4.8% each. By 2001, the number of approvals had increased to 87, based on 20 medications in 79 patients. The medication most frequently approved in 2001 was pamidronate with 18.4% of the approvals followed by mycophenolate with 17.2% and gabapentin 10.3%. Table 3.1 lists the approved medications in order of frequency between 1999-2001.
The status of the indications at the time of approval is shown in Table 3.2. More than half of the medications (67.1%) were approved for non-TGA approved indications ("off-label"). The next most commonly approved with 12.4%, were medications with indications listed on the Section 100 of the PBS but patients did not meet eligibility criteria.

Twelve percent of the medications had TGA approval for the indication of interest but were not funded through Section 100 of the PBS. Six point seven per cent of IPU approvals for medications that were accessible through the access scheme (SAS). The “other” classification in year 2000 represents a single approval of a tensoactive agent.

### Table 3.1 Most frequently approved agents shown by year 1999-2001

<table>
<thead>
<tr>
<th>Year</th>
<th>1999</th>
<th>2000</th>
<th>2001</th>
</tr>
</thead>
<tbody>
<tr>
<td>Medication</td>
<td>Mycophenolate</td>
<td>Mycophenolate</td>
<td>Pamidronate</td>
</tr>
<tr>
<td></td>
<td>Octreotide</td>
<td>Pamidronate</td>
<td>Mycophenolate</td>
</tr>
<tr>
<td></td>
<td>Erythropoietin</td>
<td>Cyclosporin</td>
<td>Gabapentin</td>
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<td></td>
<td>Cyclosporin</td>
<td>Tacrolimus</td>
<td>Octreotide</td>
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<tr>
<td></td>
<td>Pamidronate</td>
<td>Octreotide</td>
<td>Imatinib</td>
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<tr>
<td></td>
<td>Interferon alfa 2a</td>
<td>Interferon alfa 2a</td>
<td>Tacrolimus</td>
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<td></td>
<td>Tacrolimus</td>
<td>Dornase alfa</td>
<td>Linezolid</td>
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<td></td>
<td>Paclitaxel</td>
<td>Basiliximab</td>
<td>Enoxaparin</td>
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<td></td>
<td></td>
<td>Valaciclovir</td>
<td>Cyclosporin</td>
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<td></td>
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<td>Basiliximab</td>
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</tbody>
</table>
Table 3.2 Status of indications of medications approved between 1999-2001 expressed as percentages of the total number of IPU approvals

<table>
<thead>
<tr>
<th>Status</th>
<th>Year</th>
<th>1999 n=61 %</th>
<th>2000 n=77 %</th>
<th>2001 n=87 %</th>
<th>Total n=225 %</th>
</tr>
</thead>
<tbody>
<tr>
<td>Non TGA approved indication (&quot;off-label&quot;)</td>
<td></td>
<td>60.7</td>
<td>77.9</td>
<td>62.1</td>
<td>67.1</td>
</tr>
<tr>
<td>Section 100 PBS (patient not eligible)</td>
<td></td>
<td>16.4</td>
<td>10.4</td>
<td>11.5</td>
<td>12.4</td>
</tr>
<tr>
<td>TGA approved indication. Not listed on Section 100 PBS</td>
<td></td>
<td>16.4</td>
<td>5.2</td>
<td>14.9</td>
<td>12.0</td>
</tr>
<tr>
<td>SAS</td>
<td></td>
<td>6.6</td>
<td>3.9</td>
<td>9.2</td>
<td>6.7</td>
</tr>
<tr>
<td>Unknown</td>
<td></td>
<td>1.3</td>
<td>2.3</td>
<td>1.3</td>
<td>1.3</td>
</tr>
<tr>
<td>Other</td>
<td></td>
<td>1.3</td>
<td>0.4</td>
<td></td>
<td>0.4</td>
</tr>
</tbody>
</table>

A report of clinical outcomes was requested for 71 out of the 225 approvals (31.5%). Only 13 reports (18%) out of 71 requests were provided by the requesting clinician. Figure 3.3 shows the percentage of reports on outcomes requested and responses obtained between 1999-2001. The percentage of outcomes requested is the same for 1999 and 2001. During 2000 the percentage requested was 48% however the response rates were similar in 1999 and 2000.

Figure 3.4 shows not only that the Director of Pharmacy most frequently had the responsibility for the review of IPUs but also that the proportion of total submissions reviewed by the DoP had increased over the years. Next to the Director of Pharmacy, review was most often by the Chairman of the DTC. In a small proportion of approved IPUs, there was no available documentation, or another person (for example the DUE pharmacist) was involved (n=33).
Figure 3.3 Percentage of reports on outcomes requested and responses obtained between 1999-2001

Figure 3.4 Who reviewed the IPU submissions between 1999-2001
In 1999 the medical/surgical team with the greatest proportion of approvals was the Immunology team with 23% of the 61 approvals, followed by Haematology with 10% and Nephrology with 8%. In 2000 the trend was similar with 29% of 77 approvals being for the Immunology team, followed by Haematology with 12% and Lung Transplant with 9%. Figure 3.5 shows the percentage of approvals per medical/surgical team in 2001. As in previous years the Immunology team had the highest proportion with 23% followed by Lung Transplant with 13% and Haematology with 10%.

![Figure 3.5 Percentage of Approvals per medical/surgical team in 2001](image)

As the number of approvals increased so has the cost of the IPU scheme and between 1999-2001 this cost has doubled, as shown in Figure 3.6. As the yearly cost has increased so has the proportion of the medication expenditure involved in the IPU scheme. The proportion of medication expenditure through the IPU scheme
increased significantly ($p < 0.001$) from 1.6% in 1999 to 3.6% in 2001 as shown in Figure 3.7.

**Figure 3.6** Cost of IPU medications between 1999-2001

**Figure 3.7** Percentage of the pharmacy medication acquisition budget expended in IPU medications 1999-2001

*Chi square for linear trend $48343.6$ $p < 0.001$
3.4 Discussion

Through the years that were reviewed in this study, there was an apparent trend for the number of submissions and approvals for IPU access to medications to increase. The majority of these approvals (67.1%) were for “off label indications”. Similar percentages have been found in other hospitals in NSW. (251) This percentage is also similar in public hospitals in New Zealand. (252)

Only 18% of the reports on clinical outcomes requested from clinicians were reported. In this study there was no attempt to separately review outcomes through medical records. This would not be feasible in routine practice, and would not reflect the purpose and operation of the scheme. At the time the study was conducted the hospital did not have available resources to collect this data or the capacity to pursue this task.

It was noted that medications submitted for IPU approval have changed through the years (see Table 3.1). Not only newly available agents were requested but also older immunosuppressant agents such as cyclosporine, mycophenolate and tacrolimus were being used for a number of “off label” indications. (69) These changes reflect the trends in clinical practice.

Documentation allowed for the financial impact of the IPU scheme to be analysed. The financial impact of the IPU was substantial and accounted for 3.6% of the 2001 medication expenditure. Similar results have been found elsewhere. In Germany, a survey of hospital DTCs found that requests for non-formulary medications in half of the hospitals accounted for less than 2% of the total medication expenditure.
However in 19% of hospitals this share accounted for up to 6% of the annual medication cost. (253)

It might be suggested that the overall impact of this IPU scheme when compared with the entire medication expenditure is minimal. However when dealing with a capped budget and scarce resources, the question has to be asked, “Is it a fair distribution of resources to spend 3.6% of the medication expenditure on only 79 patients?” The decisions are not simply based on financial cost alone, and ethical aspects play a significant role.

As an incidental finding in this study, it was seen that the DoP reviewed the majority of the submissions and there was a trend for this workload to increase over the years. The cost of the scheme not only involves the cost of the medications but also the cost of the personnel administering this program. This may be significant. In a US study, Sweet reported that there was an incremental pharmacy labour cost associated with processing non formulary medication requests. (233) Pearce in New Zealand also acknowledged the direct operational costs associated with maintaining a formulary and medication utilisation review process on medication therapy costs. (241)

Analysis of the current IPU scheme showed the need for recording, monitoring and evaluating IPU systems and other programs that are influenced by medication costs. Cano in the USA described that tracking and documentation of non-formulary medications allowed predicting prescribing trends and financial impact. (237) For Hailemeskel, monitoring requests for non-formulary medications allowed the
identification of physician prescribing patterns. This also provided their DTC ‘equivalent’ with a guide for making formulary decisions. (238) It has also been described that an important factor in the success of a formulary is continuing monitoring. (9) Furthermore one of the DTC process indicators considers if DTCs have standard mechanisms to request non-formulary medications for individual patient. Those requests should also be overseen, reviewed and ratified by the DTC. (254)

The results of this study indicated that the number of approvals and submission for IPU tended to increase. The financial impact of the IPU scheme was significant and increased over the years. Himmel described that non-formulary medications can be an economic burden for the hospital pharmacy. (239) Revision and improvement of the current system should be considered in order to ensure efficient management. Individual applications for IPU approval are outside the clinical trial setting, and are, by definition, ‘n-of-one’ assessments of the medication for that patient.

Guidelines could be developed, implemented and evaluated for this IPU scheme, which may then have application in other Australian hospitals. At this hospital, as a consequence of this review, a new policy and procedure for managing requests for High Cost medications (HCM) for IPU were established. A DTC subcommittee, the High Cost Drug Subcommittee (HCD-SC) was created to improve the decision-making process to allocate resources to HCMs. Also as a result of this review the definition of HCM for the purpose of the IPU scheme was adopted as more than $5000 per patient in any given year.
Little is known on how decision making to access new medications is being conducted at the institutional level in Australia. As mentioned in the literature review, most studies at the institutional level have been undertaken in other countries. (138, 140, 142, 143, 236) Studies conducted in Australia have investigated DTCs in terms of their structure, roles and functions. The methodology used was both quantitative and qualitative in approach. (144, 157, 160, 163, 255)

Jenkings and Barber noted that looking at formal records (such as the IPU forms) does not capture some of the “local rationality” and the activity of the DTC decision making. They suggested that observing DTCs might provide valuable information. (256) Thus the newly formed HCD subcommittee provided a valuable opportunity to explore how decisions are being made regarding access to HCMs in a public hospital. According to Sabin, decisions about adoption of expensive new treatments provide a window for doing research about how resource allocation policy is actually made. (257) Investigating the decision making process at the HCD subcommittee level would allow observation of decision making in action, “real life decision-making”.
4. Allocating resources to High Cost Medications (HCMs) in Public Hospitals. The Role of a High Cost Drug Subcommittee (HCD-SC)

“The essence of ultimate decision remains impenetrable to the observer - often, indeed, to the decider himself …..There will always be the dark and tangled stretches in the decision-making process - mysterious even to those who may be most intimately involved”. John F Kennedy

This chapter provides an application of case study approach which offers insight into how decisions are made regarding access to High Cost Medications (HCMs) in a public hospital.

4.1 Introduction

While describing the purchaser perspective of managing new medications, Walley wrote “we believe that rationing high cost drug treatments in some form is inevitable”. A further statement emphasised the importance of this process being explicit and planned. (73) For Sabin and Daniels, decisions concerning the adoption of expensive new treatments provide one of the best windows for doing research about how resource allocation policy is actually made. (257)

As described in Chapter Three, a subcommittee of the Drug and Therapeutics Committee (DTC) at Vincent’s Hospital in Sydney, the High Cost Drug Subcommittee (HCD-SC) was established. The HCD-SC defined High Cost
Medications (HCMs) as those costing more than A$5000 per patient in a given year. (258) The DTC referred all HCM issues to this committee. These referrals included applications for Individual Patient Use (IPU) approval and other requests for HCM use. The aim of the HCD-SC was to overview the allocation requests for individual patient HCMs and to advise on management of and access to HCMs within the hospital. The recent formation of the committee provided an opportunity to describe the decision-making process for resource allocation, specifically with regards to HCMs in a public hospital setting.

4.2 Aim

The aim of this study was to describe the decision-making process associated with HCMs in an Australian public hospital.

The specific objectives were to:

- describe the operations of a recently formed HCD-SC in a public hospital, in terms of the role of the subcommittee, the role of the members, the criteria used and the process.
- explore the attitudes, perceptions, and concerns of the HCD-SC members regarding access to HCMs in public hospitals.

4.3 Methods

The method chosen for any research should be determined by the aim of the research. Qualitative or quantitative methods, or a mixture of both methodologies, may be utilised for different study aims. One methodological approach uses conceptualisation while the other uses statistics. (259) It is considered that the two
methodologies complement rather than oppose each other. (259-262) Some exploration of possible research approaches was undertaken before the optimal methods were chosen to address the objectives of this study.

4.3.1 Selection of methods

4.3.1.1 Quantitative research

Quantitative research deals with quantities and relationships between variables. Data are structured in the form of numbers. It is useful when testing hypotheses and allows examining associations between variables. (263) In this study quantitative methods were chosen for the descriptive analysis of demographic characteristics of patients who access a HCM via the HCD-SC.

4.3.1.2 Qualitative research

Qualitative research is characterised by allowing the researcher to interpret phenomena, generate hypotheses rather than test them and display the point of view of the people being studied. (264)

Qualitative research focuses more on the process rather than the outcome and analysis of data can be done intuitively. This research focuses on participants' perspectives and their meanings. (259, 260, 265) Through qualitative methods, researchers are allowed to understand, explain, discover and explore. (266, 267) These methods seem appropriate if we wish to learn from participants, the way they experience a process, their thoughts, perceptions and feelings. (266)
Keen et al identified the fact that decisions made by doctors and health care professionals are qualitative rather than quantitative in nature. (268) Qualitative research has been used extensively in health care services and policy research. (269-271) There is a wide spectrum of qualitative research methods that could be used in health research. (260, 272) These could be divided in three main categories according to Grbich: (272)

- Library based methods: documentation, historical method and discourse analysis.
- Field based methods: ethnography, phenomenology, grounded theory, oral biography, memory work, and case studies.
- Action based methods: evaluation, evaluation process and action research.

Due to the nature of the research conducted in this study it was important to uncover the “thoughts, perceptions and feelings of the participants”. (273) It was relevant to explore what influenced the decision makers and to gain access to their actions and reactions. The method sought needed to be one that provided an in-depth description of the activity of this particular group of individuals (HCD-SC). What follows is a detailed description of case study methodology, the qualitative technique chosen for this research.

4.3.1.3 Case study

Case study is a research method that focuses on concurrent events and is useful when studying individuals or organisations. (271, 274) The utility of case studies relies on the fact that it allows the researcher to understand and explore how real-
life decision-makers make those decisions and what are the real life consequences. (226, 271, 274) As stated by Pope, case studies are valuable in real world settings where policy change is occurring. (260)

The case study can help illustrate a particular social context. It allows for the observation of a particular phenomenon, and continuity and change can be documented. (272) It also has the ability to “discover” while in the process of doing research. (271) One of the strengths of case study research is that it provides rich data because the object of the case is studied in its normal setting, or context. It helps answer questions like why and how; (274, 275) why are decisions taken, how are they implemented and what was the result.

Case studies have been classified as exploratory, explanatory, or descriptive. (274) They can also involve one or more cases. (265) The study presented in this chapter was a descriptive single case study chosen due to its characteristics (first reported HCD-SC in Australia). This was described by Creswell as an intrinsic case. (265)

4.3.1.3.1 Triangulation in Case Studies

Data collection for case studies involves different sources of evidence and use of multiple methodologies. (275) If different techniques are used the evidence is stronger. The combination of methodologies allows strengthening of the study design. (271) To ensure validity of case studies triangulation is often used. Patton described that triangulation can be done in different ways: (276)
• Data triangulation: Different sources of data are used.
• Investigator triangulation: Different researchers or evaluators are used.
• Theory triangulation: A single set of data is interpreted from multiple perspectives.
• Methodological triangulation: Use of multiple methods.

Figure 4.1 Multiple sources of data and methodologies used in a single study.
Adapted from Yin (274)

4.3.1.3.2 Semi-structured Interviews in Case Studies

The interview is considered as one of the most important sources of case study information. (274) Interviews can be classified as: structured, semi-structured or in-depth. (277) Bowling described semi-structured interviews as a guided conversation. (278) The format should be flexible and, according to Patton, questions should be neutral, sensitive and clear to the interviewee. Interviews should allow researchers to gain insight into participants' thoughts, feelings, experiences and perspectives. Questions should be asked in a truly open ended...
way to allow the participants to respond using their own words. (262) Open ended interviews, the most common form used in case studies, were used. (274)

4.3.1.3.3 Direct Observations in Case Studies

Observational data allows the researcher to describe the location and activities that took place in that setting and the people who participated in those activities. (262) Keen described that “Asking participants about their experiences or observing them in meetings can provide rich data for descriptive accounts of organisational process, work practices and impact of change”. (279) Direct observations allow covering events in real time and provide insight into interpersonal behaviour. (271) Structured observations can provide the researcher with direct experience of the ways participants interact in their own setting. (270)

4.3.1.3.4 Document Review in Case Studies

Direct observations and interviewing can be complemented by analysing documents. As described by Marshall, documents such as minutes, letters and policy statements are useful in developing an understanding of the group being studied. (280)

In summary having considered the methodological approach for this study the following was conducted:

To meet the first objective, to describe the operations of the HCD-SC.
• Study meetings were observed and systematic detailed notation of events was performed.

• Documents produced by the HCD subcommittee such as terms of reference, meeting minutes, emails, letters, bulletins, the DTC website were analysed.

• In-depth open ended semi-structured interviews were conducted with members of the HCD-SC

To meet the second objective, to explore the attitudes, perceptions, and concerns of the HCD-SC members.

• In-depth open ended semi-structured interviews were conducted with members of the HCD-SC

Finally, the use of semi-structured interviews, observations and documents, allowed the researcher to draw contrast between HCD-SC members’ perceptions and actual practices about the decision-making process to allocate resources to HCMs.

4.3.2 Data Collection

The study was conducted over a 12-month period (December 2002 – December 2003). All decisions to allocate resources to HCMs, and the rationale for each decision, were identified (benefit, evidence, cost, availability of alternatives and budget constraints). For individual patient use (IPU) decisions, additional data were collected. These included: age, sex, area of residence of the patient and level of evidence for approved use (see Appendix 4.1).
Revision of documents produced by the committee was undertaken (terms of reference, meeting minutes, mails, letters). Observation of seven out of eight meetings also took place and two were audiotaped. The researcher observations were documented.

In-depth open ended semi-structured interviews were conducted with members of the HCD-SC. Members were asked to describe their role in the committee, describe the process, and comment on what they thought were the major problems and concerns. Interviews were digitally audio-taped with the permission of the interviewees.

4.3.2.1 Ethical considerations

Patients’ and subcommittee members’ data were de-identified. This study was approved by the University of Sydney and St Vincent’s Hospital Human Research Ethics Committees, and was endorsed by the Chief Executive Officer of the South Eastern Sydney Area Health Service.

4.3.3 Data analysis

4.3.3.1 Demographic data

Quantitative data such as demographic characteristics of patients who access a HCM via the HCD-SC (IPUs) were analysed using the Statistical Package for Social Sciences (SPSS®) for Windows Version 10 (SPSS Inc, Chicago, USA). Descriptive statistics were used to summarise the data.
4.3.3.2 Transcription and thematic analysis

Interviews and meetings were transcribed verbatim by transcriberonline®. Each transcript was validated by the interviewer against the original recording. Every tape was then listened to for a second time. Preliminary data analysis was conducted after each interview. Interviews, observations and documents were thematically content analysed and data analysis took place alongside data collection. (262, 269) Data were organised by coding into categories. (281) Coded units were then labelled as specific concepts and grouped together under a theme. These were then organised. QSR NVivo® Version 2.0 (QSR International, Australia) was used as a data management tool.

4.3.3.3 Validity

In qualitative research, validity rests in the extent to which the account accurately represents the phenomena under study. (272, 282) Validity is concerned both with the process of data collection and analysis, (282) relating to both researchers’ interpretation and informants’ response. (273) Validity of the findings were tested through different mechanisms in this study.

**Face validity** - even though the HCD-SC members were given the option to review the transcripts they declined based on time constraints. Therefore “respondent validation” was not performed.

**Negative case testing** – In this study the researcher searched through the data to find cases that run counter the findings.
Internal validity - The analysis of the HCD-SC was conducted using data and methodology triangulation. (274) Use of semi-structured interviews, observations and documents, allowed the researcher to draw contrast or confirm HCD-SC members’ perceptions and actual practices about the decision-making process to allocate resources to HCMs.

4.3.3.4 Reliability

Reliability can be evaluated by demonstrating that the area of research has been systematically explored. (272) In this study reliability was evaluated though different steps:

Intra-observer consistency – more than half of the interviews were coded on two separate occasions to ensure consistency with the coding.

Audit trail - the process from initial coding to incorporation of the concepts into an explanatory scheme was recorded. This included documenting major analytical and methodological decisions.

The ethics framework of accountability for reasonableness assisted interpretation of the results.
4.4 Results

During the study period, the HCD-SC met eight times and considered requests for ten medications for 14 indications with two medications being listed in the formulary. The rationale for the decisions is described in Appendix 4.1. As described in this appendix decisions were not based on a single criterion, but most often grouped criteria of effectiveness, benefit and cost.

Sixteen requests for High Cost Medications (HCMs) for individual patient use (IPU) were considered. Of these patients seventy five percent were males, the median age was 46 years (range 18-72 years), and 31% lived outside the Area Health Service. The evidence for the indication requested in 53% of cases was level II of evidence (Level I-IV described in Appendix 4.2). Figure 4.2 shows the HCMs approved for individual patient use (IPU).

![Figure 4.2 High Cost Medications approved for individual patient use. (n=16)](image-url)
4.4.1 Document Review and Observations

• **Role**
  During the meetings the role of the members was clear. Some provided the information, others the clinical expertise and others balanced both. The subcommittees’ terms of reference are listed in Appendix 4.3.

• **Criteria**
  Even though the criteria were not documented, the committee used the following when assessing a HCM: benefit (improved quality of life, prolonged survival, relief of symptoms), level of the evidence, alternative treatments, cost per patient per year and the number of patients likely to receive the treatment, to assess not only individual but formulary decisions. Cost effectiveness or any other type or pharmacoeconomic analysis was not used. The concept of “opportunity cost” was employed. The notion of the benefits foregone was expressed during the meetings observed. “What are we going to give up?” (member)

One of the arguments used by the requesting physicians during the meetings was “savings”. “If we use this drug we can save on xxxx”. (physician) However the members expressed that this was not as straightforward as expressed by the physicians. By spending money on a HCM this money was not going to be used somewhere else in the hospital and there was no such thing as “saving money”.

A member tried to exemplify this by telling the physician “If as you suggest, expenditure of the order of $60,000 per annum on (name of the medication) for patients who are not currently receiving treatment actually does produce savings
then the only way this hospital could currently afford such an investment would be by realizing those savings with the matching reduction in the (name) unit’s budget”.

(member)

Evidence of effectiveness was the most important criterion for HCD-SC members. Effectiveness was always balanced against the cost “we should not fund treatments to experiment” (member). However evidence of effectiveness was sometimes limited (case study, case series).

For physicians attending the meetings “need” was one of the main criteria. In some cases the HCM was presented as the patient’s last option. As previously mentioned improving the patient’s quality of life was an important criterion not only for the members but for physicians. “This (the medication) obviously has a huge impact on his quality of life and family”. “(name of the medication) has been an important therapy for (name of patient) which allowed rapid control of disease activity and greatly enhanced the quality of his life” (letter by physician to the HCD-SC).

• Process

Meetings were held on an ad hoc basis. If the cost of the medication presented to the DTC was higher than A$5,000 per patient per year, a meeting was convened by the chairman of the DTC. The requesting physician then had the opportunity to address the committee with the request. The submissions included information such as evidence of effectiveness, proposed dose and duration of treatment, cost of treatment, and expected numbers of patients per year.
If the medication was requested for Individual Patient Use (IPU), the physician provided relevant patient history details, previous treatments, evidence to demonstrate that any relevant formulary medication(s) had been tried and/or was (were) inappropriate and plans to follow up for outcomes of the therapy.

On two occasions the documents containing the information were forwarded to the members before the meetings. Otherwise the physician would describe the case during the meeting highlighting the benefits of the therapy for the patient(s).

Most meetings were attended by at least three members of the Committee (The Executive Director of the hospital, Director of Hospital Pharmacy Department, the chairman of the Drug and Therapeutics committee). When consensus was not reached during the meeting, the chairman of the DTC emailed or mailed the response to the physician detailing the reasons for the decision. However most of the time the physician took part in the decision-making process. If access to a HCM was denied the reasons were explained and discussed with the physician. If the criteria could be met later on the physician could present the case again (appeal mechanism).

Through meeting observations the researcher identified different mechanisms to control access to HCMs:

- Targeting certain patients with specific conditions
- Order by specific clinicians
• Order until a specific expenditure level had been reached (e.g. $50,000 per year for Infliximab for Crohn's disease)

• **Problems**

What was apparent from the observation of the meetings was that the decision-making process to allocate resources to HCMs with limited resources was not simple. As expressed by one of the members during a meeting. “*Decisions are not linked to resources and there won't be any extra money*” (member).

Documentation of the meetings (minutes) was available for seven out of the eight meetings held during the study period. In seven cases the results of the decisions taken by the HCD-SC were presented to the DTC members during the monthly meetings. HCD-SC meetings took an average of 50 minutes. However once the meeting was over it was hard to follow up the decisions. There were gaps in the documentation. Even though it was not stated in the terms of reference (see Appendix 4.3) the website where the decisions were documented was not updated in a timely manner and rationales were not available. At the time this thesis was written some of the decisions were still pending and had not been included. Information for a non HCD-SC member was hard to find.

Files remained in the Director of Pharmacy office, limiting the access to the information. Annotations into the STOCCA pharmacy dispensing system detailing the conditions of approval for IPUs were not always added. To exemplify this, during the study period the investigator did not attend the seventh meeting. The information about the decisions made at that particular meeting had to be obtained through the documentation. The minutes for that meeting were not available and
information was hard to find. To be able to document the decisions and complete
the information gaps the investigator had to seek clarification from two of the HCD-
SC members. Information therefore resided with the HCD-SC members. It should
be noted that the terms of reference of the committee did not state what information
was going to be available or who should have access to it (see Appendix 4.3).

One of the problems identified through the meeting observations and
documentation was the tension between medication availability and hospital
decision-making. As explained in Chapter One, once a medication is registered
(licensed) it can then be prescribed. However this does not mean subsidised
access. Sometimes there was a gap between registration and subsidisation. This
meant that some of the medications that were studied by the HCD-SC had been
rejected for listing in the PBS Section 100 by the PBAC on cost effectiveness
grounds. As a consequence the cost of this medication would be hospital
expenditure.

The following is an example of a medication not listed on Section 100 but sought to
be provided by the hospital. The physician stated “The rejection by the PBAC of
(name of the medication) for PBS reimbursement has left a few questions in the
air”. In this particular case, patients were exposed to the medication through what
the company called “Australia’s first national, open label trial in (indication) and an
approved Special Access Scheme for compassionate use”. Once the
pharmaceutical company was notified of the PBAC rejection, the company advised
hospitals that they would no longer provide medication free of charge and therefore
hospitals had to plan and consider options as well as alternative funding.
The HCD-SC had to decide if this medication was meant to be listed in the formulary. The HCD-SC needed to consider if resources were going to be allocated or if patients needed to wait until the medication was subsidised via PBS Section 100. During this decision-making process the following statements emerged: “devastating”, “life threatening disease” and “no other accessible and effective treatments”.

Even though further details of this decision are described in Appendix 4.1, it is important to highlight that one of the results of this discussion was the inclusion of a statement in the informed consent of the patients enrolled in the programs described above.

“For 9 January 2004, it is highly unlikely the manufacturer, (name of the company) will continue to supply the drug free of charge and reserves the right to review its policy in relation to funding. The cost may be as much as $40,000 per year (name of the hospital) will not be able to fund on-going supplies of the drug thereafter. In the event that (name of the medication) is no longer available, then individual options for alternative treatment will be discussed.”

In this particular case an approved medication (TGA) was denied on the grounds of “opportunity cost” to other patient groups. There were no resources within the fixed budget of the hospital to pay for this medication for these patients.

Problems were also identified when decisions were made regarding allocation of resources to an individual patient (IPU). The members had to consider identifiable individuals, for whom high cost medications were requested. This might have added
an explicit emotional factor to the decision-making process. Especially in those individual cases where the evidence of effectiveness was limited (eg. Appendix 4.1 decision 8).

4.4.2 Interviews

Interviews were performed with all members of the HCD-SC. On average the interviews lasted 30 minutes. Verbatim quotes from members of the HCD-SC illustrate the findings.

- **The Role**

Members were asked what their role in the committee was and the role of the HCD-SC. They all clearly identified theirs and the HCD-SC role. They perceived the committee was “decisive” and “effective”.

“to participate in the decision-making based on the role of the hospital, the mission of the organisation, the ethical decisions we have to make and the financial constraints that we operate under and also our legal obligations”.

“I've been involved in trying to rationalise the best way we will use those (HCMs)”.

“It's one of the more decisive and effective committees that I've seen operate and it generally makes its decisions by consensus with a lot of discussion”.
• **Criteria**

All members considered that safety, effectiveness and cost were the main criteria however they also distinguished that in some circumstance evidence of effectiveness was hard to obtain due to the characteristics of the medication (new and innovative) or the condition. Some members would like to have cost effectiveness information as included in the PBAC submissions (even though this is conducted with a societal perspective). Benefit and need were also considered very important especially when there were no alternative treatments.

“you’ve got some otherwise productive member of the society who is failing everything else that has been tried, you might want to try a high cost drug. It’s warranted I think, with someone like that”.

“Clinical need, including the fact that there are no other alternatives, and a total management plan around the care of the patient. Also some good predictions and management of how long that person may need to be on that drug. Then aligned with some evidence that that drug is going to work, that it’s going to useful for that patient. Now, some of these drugs are on the cutting edge and there is anecdotal evidence of, this is the heart, that it helps pulmonary hypertension very well and therefore people want to use it on the anecdotal evidence”.

“there's never been any real discussion around the main decider assuming that efficacy and safety are reasonable is total cost to the hospital what matters”.

“Efficacy, safety and cost effectiveness would have to be included as they are in the PBAC”
• The Process

All members perceived the process had improved the way the hospital allocated resources to HCMs. Members considered that there was different expertise in the group which added different values and perspectives to the decision-making process.

“I think it’s a good model of the way things can be done effectively and certainly it’s working in terms of controlling high cost drug costs and also in a way, I think is seen as reasonably credible.”

“I think it was a positive move. It’s much more decisive than (when) we used to agonise, all of us around the table and it seems to make decisions much better and I do think, I mean (person’s name) is very good at that and having (person’s name) there. Having those two, I think is very important.”

“I think it’s made a difference for (person’s name) and a couple of others in the pharmacy and for the full drug committee in terms of, we’ve been a bit stricter about going through the right process and the committee is there so it gets a bit more behind the pharmacy in saying ‘you can’t just order that drug, that’s a high cost drug, you’ve got to fill in that form and its got to be reviewed’.

“There’s a wider group of people considering the approval now. We deal with it in a much more regular and timed session than (what) we did in the past. The executive director is always involved in the decision and the process and we try to put people through the same sort of process”.

For the members one of the positive things of this new decision-making process was the physicians’ involvement. They were able to present the case and be involved in the decision-making process, perhaps making them more likely to
accept limits imposed by this process. Since some of the members were physicians they identified that their role was no longer the patients advocate but they were acting as the gatekeeper of the hospitals budget.

“I think also we’re fairly inclusive now because we do quite often ask the person (physician) to come along and make a presentation.”

“I am functioning on behalf of the hospital and I am trying to strike the best compromise that maximises both what the patient needs and what the hospitals needs are, don’t expect the treating physician to make that call”.

The members considered that the decision-making process was transparent. However it was acknowledged that physicians and other hospital staff needed to be involved in the process to understand it. Members were uncertain as to the amount of information that people in the hospital were able to obtain (re reasons for a decision).

“I think it’s fairly well known now across the hospital. I think we’ve developed it a little as we’ve gone along but I think we’re pretty much down to where we need to be. ………new medical staff members who come into the hospital; we may need to think about how we let them know about it”.

“Probably if you stopped any doctor in the corridor they wouldn’t but I think people that have been involved, like the xxx prescriber’s, the yyy with his zzz and if you’ve actually had a high cost drug and had to go through the process, you’d have a better appreciation. I don’t know that everyone actually has to know about it but I think those that have experienced the system would understand it”.
“Well depends I guess. To be honest I’m not sure what access to information they have on the web. The details of the setting up of the committee and the membership was sent out in the form of an executive bulletin at the time of the set up so that everyone in the hospital who gets the executive bulletin and that is virtually all of the staff of the hospital would have seen it at that stage. There is some turn over since then but not much if it’s not explicit on the web then it should be made more explicit. But I’m also very aware of the fact that most of the people who ask things don’t look on the web anyway”.

The members of the HCD-SC acknowledged that it was important to include a member of the public in the subcommittee. This added legitimacy and transparency to the decision-making process. However they recognised the difficulties with the logistics and the fact that this was a subcommittee of the DTC and there already was a member of the public in that committee.

“I actually think that they [members of the public] would be a very useful person to have because anyone within an organisation or within a system, does tend to slip into thinking within a particular framework and it’s always good to have someone from outside that framework to question what you do”.

“I don’t have any principal objection to having a member of the public on it. It’s just the practicality of getting these meetings organised will be magnified and multiplied even further if we had to get someone from the public as well and I’m not sure how much good we will get. I think probably not. The high cost drugs committee after all is a subcommittee of the drug committee which does have consumer rep on it. But the practicality is that these people aren’t available during working hours which is when the high cost drug committee meets.”
“I think we would benefit by the input of the consumer or lay person I think it would be helpful but how one will manage that within the constraints of a limited and capped budget I think is a very difficult issue. It will be quite challenging”.

- **Problems**

It was identified that knowledge was held by “some” like “corporate knowledge”. Therefore if these people left the hospital the knowledge would be lost.

“we do have a central repository of knowledge, but those sorts of things often depend on key people, so if you lose key. So that’s a problem. I think, because a lot of it is knowledge and corporate memory”.

All the members agreed that the main problem was the limited budget and therefore the opportunity cost. Evidence of effectiveness especially for the newer agents was limited.

“the decision-making is often being done in a bit of a vacuum from a policy perspective.” “The other problem for this hospital is the particular nature of the work we do and the fact that it does lend itself to people who are more likely to need the new drug treatments that we see and also the vast number of clinical trials we do which means that we quite often end up with these. So there is obviously compassion from clinicians to use them”.

“The problem with high cost drugs is obviously their cost, putting them into a budget, so given that we have to have a boundary on a drug budget and I think that’s not just a practical thing, that’s just the fact that we do and we do have to have a boundary on it”.
“One of the other difficulties is that often with these newer agents there is no high level of evidence for the use in particular indications maybe case reports and the drug will often be quite new”.

A member expressed that there was a conflict between the utilitarian principle “greatest good for the greatest number” versus a natural compassion for an individual in need. Decisions were affecting identifiable patients, who they are, the possible benefit from the HCMs, and this influenced their decisions. Described below are the thoughts expressed by the members of the committee.

“you can’t have any criteria that says some people are more worthy than others. I just don’t believe you can do that because that always becomes conflicting - is a single mother more worthy than – so all those sorts of things become a problem”.

“The answer will not be the more people you can treat with the money is always right. That won’t be right any more than treating one person… It’s a hard decision”.

By approving a HCM to be used in a group of patients the members perceived they were denying access to another, this was considered an “inequity” in terms of access.

“there’s also issues of equity of access with high cost drugs-should we make a particular high cost drug available to our patients and not make another high cost drug available. Why is one patient group more important than another patient group and how do we decide which drugs should be available to each patient?”
“there are a lot of deserving patients out there and you really can’t afford to give these things to them all and it’s working out who should get it and who shouldn’t”.

Another concern expressed by members was that decisions being taken by the committee such as report of outcomes, development of guidelines, restricted use etc were not actually being implemented.

“If we make a decision about a particular high cost drug and its use, we need to be able to monitor whether in fact it did work and follow it up later on and then use that information as a wording for other decisions. And I think that’s a problem. We’ve just got to keep that top in mind and keeping on track with it. You’ve got to learn from the things that go wrong or keep following them up so that you can use it again”.

“I think the audit procedure is 1) so that they follow the agreed protocol, 2) when they try and get some estimate of how many per year and if they say ‘5 to 10 per year’ and we find it is 50 a year, that’s going to have a very significant impact and 3) what is the outcome? If we’re paying all this money, even though our numbers might be small, we want to see some benefit, don’t we?”

4.5 Discussion

Role

In this study, the decision-making process to allocate resources to HCMs at a public hospital was described. The HCD-SC was a committee of “fair-minded” (as described by Daniels) people brought together to make decisions about the allocation of resources to HCMs. (3) The group included a broad spectrum of individuals from within the hospital (administrator, doctors, pharmacist, ethicist).
One of the incidental findings in Chapter Three was that the Director of Pharmacy was the person who most frequently had the responsibility for the review of Individual Patient Use requests for HCMs. From the observations and interviews reported in this chapter, it was perceived that the subcommittee took away some of the burden from the Director of Pharmacy.

The HCD-SC members believed they were accountable for the decisions being made and decisions were taken in a broader forum, with different values, perspectives and expertises added to the decision-making process. For Von Berger, in group decision-making there is a belief that “all of us know more than any of us know” and the responsibility is spread among individuals. (283)

**Criteria**

Data collected from observations, documents (terms of reference, meeting minutes, emails, letters, bulletins, the DTC website) and interviews were triangulated to find the rationale/s used in the decision-making process. As expected, several criteria were considered (see Appendix 4.1). Even though most individual patients (IPUs) were males (75%), gender was not considered as a criterion in the decisions. Neither did social class, occupation, family role, income or education level. Access to HCMs was not denied on the basis of age (range 18-72 years). However, consideration was given to younger patients (see Appendix 4.1 decision 3 case 1). The debate in the literature about age as a criterion to allocate health care resources is extensive. (219, 284-290) However consideration of age as a criterion for allocation of health resources is beyond the scope of this study.
Not surprisingly, effectiveness, cost and opportunity cost played a major role in decisions regarding the allocation of resources to HCMs. However, pharmacoeconomic evaluations were not used by the HCD-SC. This was consistent with findings from previous studies that have reported that health care organisations and hospitals make limited use of pharmacoeconomic data in the formulary decision-making processes. (140, 142-144, 151, 236, 252)

One of the members suggested that having access to pharmacoeconomic studies, such as those presented to the PBAC would be useful. This had been previously suggested by Plumridge who considered that PBAC pharmacoeconomic evaluations could be used by hospital drug and therapeutics committees in their decision-making process as an indication of economic value. (291) However hospitals still face “off-label use” (1, 291) and pharmacoeconomic studies presented to the PBAC are conducted from a societal perspective (i.e. Commonwealth Government) rather than the perspective of the institution (i.e State Government). (34)

HCD-SC decisions were taken with varying levels of evidence about effectiveness being available (see Appendix 4.1). It seemed that in some circumstances the HCD-SC applied the “rule of rescue” to high cost medications. This meant the HCD-SC members had the “imperative to rescue identifiable individuals facing avoidable death”. (292) Patients with “rare catastrophic illness” with no treatment alternatives were given HCMs. (191, 292, 293)
The tension between availability and hospital decision-making was identified. This could also be identified as tension between decisions made at the macro level (TGA/PBAC) and the meso level (hospital HCD-SC). The question asked by the members was: “should a medication that has been proven to be effective (by the TGA) be denied due to its cost?” On the other hand, should scarce resources be spent on medications where information on effectiveness is limited? For Leeder, to divert money to support medications with no proof of benefit seemed “inequitable and foolish”. The inefficient use of resources in a patient might deprive other services and goes against allocative efficiency. (294)

The Process
The ethical framework of accountability for reasonableness (A4R) described in Chapter Two was used to evaluate the decision-making process for legitimacy and fairness in this setting. (3) This framework requires all four conditions of, publicity, relevance, appeals and enforcement to be satisfied. This has been previously described in other settings. (140, 222, 224, 295-297) A procedural requirement of fairness involves dealing with similar cases similarly and different treatment justified by relevant reasons. (152, 298)

In this procedural justice approach (A4R) to decision-making, decisions are meant to be taken in an open, transparent way and based on sound reasoning that is publicly accountable. (223)

- Publicity (Transparency)
For the transparency condition, which Daniels and Sabin called publicity, to be met the public should have access to the decisions being made by the HCD-SC and the
reasons for decisions should also be transparent. (3, 152, 225) External transparency would be considered to be present if the process, deliberations, decisions and reasoning of the HCD-SC decision-making were available to stakeholders external to the membership. (141) Decisions might need to be accessible both by people inside and outside of the hospital. Wailoo stated that for a process to be legitimate it is essential that the reasons why decisions are made are transparent. This increases the level of confidence that people have in the process. (299) Daniels expressed that transparency about the grounds for decisions improves the quality of the decision-making and openness improves public understanding of the necessity of priority decisions and promotes a culture of education and learning between stakeholders. (3) According to Sabin, “unless the reasons why a HCD was not approved are articulated there is no opportunity for debate, focused criticism and societal learning”. (257)

In this study the rationales and decisions made by the HCD-SC were not accessible beyond the clinician, hospital executive and DTC. However it is important to consider that there are restrictions on the availability of information. For some information there may be “commercial in confidence” considerations for the pharmaceutical companies. Consideration is also given to patients’ confidentiality. It seems like transparency in this setting is not a reasonable requirement given the facts previously provided. It might not be workable for all decisions/processes in a hospital to be transparent to everyone. So to what extent is transparency reasonable? Do all decisions and decision-making processes have to be transparent and to whom? How much information do members of the general public want about decisions regarding access to HCMs in public hospitals? Ham has previously indicated that there is a gap between “aspiration” and current reality"
in priority setting in health care. This author considered we should be realistic and seek improvement in an imperfect world. (300)

Considering these difficulties, the proposal suggested to overcome these difficulties could be for the HCD-SC to state when information is "commercial-in-confidence" or when disclosure of information would jeopardise patients’ confidentiality. The hospital could consider ways for making this information available while still balancing the right of individuals versus the right of society.

Recognizing the limitations the hospital could post on their website or publicise the reasons for the decisions in a bulletin. This could allow all stakeholders (hospital staff, patients, family, etc) to engage in what Martin called a "kind of policy learning about appropriate limit setting decisions regarding medications". (140) However the challenge is not only for the HCD-SC. Transparency is needed not only for this particular process in the hospital but for other decisions such as access to intensive care beds, dialysis, transplants or waiting lists. The debate goes beyond setting priorities to HCMs and the complexities are vast.

Finally it is also important to distinguish between the transparency of decision-making about policy or individual cases to the parties most affected from "outside" the process, and the need for every last detail of the bureaucratic process on the "inside" to be open to external scrutiny. Beyond the decisions it is important to publicise the existence of a "process" to set priorities to HCMs.
• **Relevance**

Decisions should be “reasonable” in order to meet the relevance condition. (3) This means they should appeal to values and principles that “fair-minded” people can agree are relevant. People are considered “fair minded” if they are committed to participate and consider the common good. (141) As previously described, in this study different decision-makers were involved incorporating multiple perspectives. Also HCD-SC decisions were based on a range of different criteria (rationales) which have also been previously described. Transparency directly influences the degree to which the relevance condition is met. It is difficult from the outside to assess the extent to which the relevance of the rationales used in decision-making is, if there is limited transparency. (300)

• **Appeals**

According to this condition, there must be a mechanism for challenges, ongoing review and revision of the decisions as new information is available. (3) The HCD-SC process included an appeals mechanism. The trigger for an appeal was the availability of new information, usually about the effectiveness of a treatment. The process allowed the opportunity to challenge, and revise earlier decisions made by the subcommittee. To this extent the HCD-SC process met a requirement for an appeal. However this is limited to a change in information about the effectiveness of a treatment. There was no basis for appeal on other grounds, such as a challenge to the basis on which a decision was made (eg. patient disputing the fairness of a decision).
**Enforcement (Review and Revision)**

Daniels claims that there must be voluntary or public regulation of the process to ensure that the first three conditions are met. (152) To meet the conditions decisions need to be transparent, relevant and open to appeals. A mechanism could be in placed to hold the HCD-SC members accountable. Perhaps there could be oversight by someone, or some other committee to review whether the HDC-SC had acted according to its own terms of reference (eg independent experts be invited to review a sample of cases chosen by them). This could be analogous to an accreditation or peer review process.

In summary, the decision-making process was not “accountable for reasonableness” (eg meets Daniel and Sabin’s four conditions).

**Public participation**

Even though public participation in the decision-making process to allocate resources to HCMs was considered important by all HCD-SC members, the logistics were considered difficult. Similar results were found in a study conducted by Litvia, regarding public involvement at different levels of health care decision-making. Participants (members of the public and informants from health and non-health related organisations) acknowledged the logistical difficulties of involving the public in decisions concerning choices between patients. The role of the public was also seen as limited in these sorts of decisions. (301)

There are two opposing views regarding public participation in the allocation of resources to medications. One view expressed is that lay knowledge increases the
accountability and openness of the decision. (301) The second view states that even though it is important to involve the public there are some situations in which it is not appropriate to include members of the public because they do not have knowledge and skills to evaluate matters of medical and scientific nature. (302)

In 1994, Bochner presented a method for rationing medications in a teaching hospital in Australia. (161) In response to this method, Petrie commented that discussions about rationing of medications should be explicit and debated with the public. (303) Petrie went on to ask Bochner “Are patients admitted to this hospital aware that they are subject to drug rationing, and of the criteria? Can they choose to be admitted to another hospital?” (303)

It is important to note two issues. The HCD-SC is a subgroup of the DTC where there is a consumer representative. The second issue relates to patient involvement. As described in Appendix 4.1, in some cases shared decision-making between doctors and patients was encouraged in order to give patients a degree of autonomy in accepting or refusing treatment with a HCM (see decisions 4 and 6). In these situations patients had been asked to sign an informed consent form to record that these discussions had occurred. This might have allowed the patient’s opinion regarding access to a specific HCM to be considered. This practice could provide a means for allowing a patient’s opinion, regarding access to a specific HCM, to be considered by the HCD-SC. Patients were expected to be informed of costs and benefits by the treating doctor, with patients assuming what Poirier called a cost-sharing for high-cost treatment. (82)
In order to enhance and broaden the scope of factors considered relevant by the HCD-SC lay participation of both patients and community members could be included. However as previously explained the HCD-SC is a subgroup of the DTC.

**Physician involvement**

The physician not only presented the case but was actively involved in the decision-making process. This was seen as a positive move by the members of the HCD-SC. For Shenfield, rational equitable cost containment only works if the prescribing clinicians are involved in all stages of decision-making. (159) Pickette considered that if physicians feel engaged with the formulary decision-making process they are more likely to accept limits imposed by this process. (231) In this study the physician not only presented the case but was actively involved in the decision-making process. This was seen as a positive attribute by the members of the HDC-SC.

**Problems**

The decision-making process to allocate resources to a high cost medication to an individual patient (IPU) was described as complex by the HCD-SC members. From the quotes previously presented, HCD-SC members expressed the predicament they faced when balancing individual needs and providing maximum benefit for the greatest number of patients. (304)

For Fijn one thing that characterises hospitals is that sometimes medication allocation issues deal with identifiable individual patients. Hope in the UK has previously described that when dealing with an (identified) individual who may
benefit from treatment compared with a group of people (unidentified); there is a considerably different emotional response. (293)

Concerns about prioritizing between patient groups and individual cases have also been expressed by Poirier in the US and L de Lemos in Canada. (79, 82) For these authors several questions arise when deciding if a patient should have access to a high cost medication; should a severely ill patient receive a high cost medication when it is known that there are limited resources; does this patient have more rights than others? Should limited resources be used to benefit only a few? Or should we sacrifice one for the benefit of others? (79, 82) However L de Lemos considered that employing these considerations in practice is complex, especially with patients facing potentially lethal conditions (eg.cancer). (79) The study conducted by Singer et al looking at priority setting for new technologies (including HCMs) in medicine, also found that the possibility of “saving” patients tended to influence the allocation of resources. (142)

HCD-SC members identified that auditing the implementation of decisions being made, such as tracking outcomes and developing guidelines, was a problem. For some members it was important to have a review process to ensure that medications were not continued after the designated trial period unless positive outcomes of the therapy were demonstrated.

Benatar in South Africa, considered that little is known on how resources are allocated at a national, regional or institutional level (305) Even at the public hospital level, where resources are constrained it appeared there is no established
culture to make such decisions in an accountable and rational way. (305) This study provided data on the operations of the HCD-SC, the decision-making process and criteria used by it to allocate resources to HCMs within a capped budget.

Martin proposed a three step process to improve priority setting in health care institutions: describe, evaluate and improve. (113) This study involved steps one (describe) and two (evaluate). The decision-making process to allocate resources to HCMs was described through a case study. As previously described case studies provide “rich data from which generalisation to theory becomes possible”. (264) This study described what decision-makers actually did with “real life” decisions and consequences. The ethical framework of accountability for reasonableness was used to evaluate the process for legitimacy and fairness. Gaps were identified and considered as opportunities for improvement. Ways of improving the decision-making process will be discussed in Chapter Seven.

This study raised a number of questions: Are the problems described unique to this particular hospital? Are other decision-makers faced with the same problems and concerns regarding access to HCMs in public hospitals? How much information do members of the general public want to know about access to HCMs in public hospitals? These questions are addressed in Chapters Five and Six.
5. Decision-makers’ views about access to High Cost Medications (HCMs) in public hospitals.

“The greatest minds are capable of the greatest vices as well as of the greatest virtues”. Rene Descartes

This chapter describes the perceptions, concerns and attitudes of health care decision-makers regarding access to HCMs in public hospitals.

5.1 Introduction

Public hospitals in Australia are faced with budget cuts and decisions on allocating scarce resources. (210, 306) The question asked by Alexander is “how well founded are these decisions?” It is important to develop an understanding of how doctors and administrators deal with resource allocation and health care decision-making. (210)

Shenfield considered that hospital drug budgets are an attractive target for “an accountant’s red pencil”. This budget competes with wages and salaries, catering, cleaning services and other activities within the hospital. (158) High cost medications (HCMs), as described in Chapter Three, may have an impact on medication expenditure. Orme expressed that strategies to control hospital medication expenditure are needed in order to deal with “expensive” new medications. (307) To date, limited research has been conducted into access and
funding of HCMs in public hospitals in Australia. It would be valuable to evaluate how individual perceptions of decision-makers may influence and assist in the decision-making process. Understanding the role of these perceptions in the decision-making process may assist in future management of HCMs.

5.2 Aims and objectives

The aim of this study was to investigate the perceptions, concerns and attitudes of health care decision-makers (DMs) regarding access to HCMs in public hospitals. The specific objectives were to:

- investigate the understanding of decision-makers about HCMs
- examine how decision-makers make their decisions regarding access to HCMs in public hospitals (criteria and process).
- investigate the current problems and possible solutions when allocating resources to HCMs.

5.3 Methods

5.3.1 Selection of methods

As noted in Chapter Four, qualitative research aims to describe human phenomena. (260) It allows development of new concepts, seeks understanding and can generate hypotheses. (260, 270) Qualitative research allows “people to speak in their own voice”. (270) It helps uncover and describe participants’ perspectives on a certain event. (280)
The field based method of grounded theory was chosen in this part of the research to describe the perceptions, concerns and attitudes of health care decision-makers regarding access to HCMs in public hospitals.

5.3.1.1 Grounded Theory

Grounded theory is most useful in areas where research has not been extensive. It is an approach that helps to answer questions relating to interaction, process or understanding. (266) Grounded theories are expected to offer insight, enhance understanding and provide meaningful guide to action. Strauss states that theory emerges from the data and is more likely to resemble the “reality”. (308) Grbich describes it as an analytic inductive process where hypotheses develop from the research field. (272, 282)

This study was conducted using a modified grounded theory approach where theories of decision-making and the ethical framework of accountability for reasonableness previously described in Chapter Two and Four assisted interpretation of the results.

5.3.1.2 In-depth interviews

The primary source of data in grounded theory comes from in-depth interviews. (309) Within in-depth semi-structured interviews, Marshall and Grbich have described a subset called the “elite interview”. (272, 280) In this subset the interviewee represents a special case. Elite individuals are selected based on their expertise in the area of research and are characterised by being influential and well informed within the organisation. (280) It can provide valuable information but
accessing the elite individuals may be challenging. They are often busy people with time constraints. Elite groups studied in the past have included surgeons, anaesthetists and other health professionals. (272)

In this study health care decision-makers such as executive directors of hospitals, area health service managers, directors of hospital pharmacy departments and professors of medicine were the main group of interest. The sampling method will be discussed below however it is important to note that participants were chosen not only because of their interest, but also due to their “knowledge” in the area of research.

5.3.2 Setting

In the late 1980s, New South Wales (NSW) Area Health Services were established. The Areas included a variety of health facilities and management processes. (310)

The study took place in an Area Health Service in Sydney, NSW. At the time of this study, the South East Sydney Area Health Service (SESAHS) was one of ten state Area Health Services. There were nine public hospitals in the SESAHS which ranged in size, the largest of which had 560 beds. Some of Sydney’s major tertiary referral and teaching complexes were included in SESAHS. Approximately 780,000 people (12% of the NSW population) lived in south eastern Sydney. (311) On the first of January 2005, South East Health and Illawarra Health merged to form the South Eastern Sydney and Illawarra Area Health Service (SESIAHS).
5.3.3 Sampling

One of the main differences between qualitative and quantitative research is the sampling methods used. Smith describes the difference as follows: “Whereas statistical representativeness influences the sampling strategy in quantitative research, it is rarely a determining factor for recruiting subjects in qualitative research”. (312)

For this study a purposive sample of key decision-makers within the SESAHS were identified. These individuals included executive directors of hospitals, area health service managers, directors of hospital pharmacy departments and professors of medicine. In purposive sampling there is a purpose in mind. Participants have special characteristics. (262) In this study invited participants had already taken part on the "Access and Equity for High Cost Medications in South East Health Area Service" forum on the 11th of March 2003.

5.3.4 Recruitment

Letters of invitation to participate were sent to thirty seven key decision-makers in the SESAHS. (Appendix 5.1) The invitation letter outlined the objectives of the study and a reminder letter (Appendix 5.2) was also sent if no response had been received within four weeks. Those who answered positively were contacted and an interview was arranged to take place in a location that suited them. According to Gordon interviewees need to feel “comfortable and in control, making honest disclosure more likely”. (313)
5.3.5 Data collection

5.3.5.1 Interview Process

In-depth semi-structured interviews were conducted between August 2003 and April 2004. All interviews were conducted by the author. Before the interview, each respondent was given details of how the information was to be used. Due to the confidential nature of the information revealed by the decision-makers, emphasis was placed on reassuring each respondent that anonymity was guaranteed. The interviewer supplied a subject information statement which provided background information and a consent form (Appendix 5.3 and 5.4).

An interview schedule with a list of topics to be discussed during the interviews was used (Appendix 5.5). The schedule was a guide or prompt sheet to ensure the same topics were covered during the interviews. However questions were not asked in a standard way and respondents were able to generate their own concerns. The term high cost medication (HCM) was deliberately not defined by the interviewer. Decision-makers were encouraged to use their own definition. The interview guide allowed for relevant issues to be discussed and evolved as the study progressed to allow new emerging concepts to be included. Interviewees were reminded it was their thoughts and opinions as decision-makers that were sought.

Interviews were digitally audio-taped with the permission of the interviewees. Once the interview was finalised, a transcription file was completed which included information such as subject identification number, aim of the study, date, place, time, relevant information and special circumstances. (272) An example of a
transcription file has been provided in Appendix 5.6. Interviewees were asked if they wanted a copy of the transcript. Notes were also recorded on the interviewer’s diary after each interview. Interviews were conducted until thematic saturation took place. (309)

5.3.5.2 Demographic characteristics

Participants’ characteristics such as age, gender, organisation, position within the organisation and educational background were recorded.

5.3.6 Data analysis

5.3.6.1 Qualitative data

Interviews were transcribed verbatim by transcriberonline®. Each transcript was validated by the interviewer against the original recording. Every tape was then listened to for the second time. Preliminary data analysis was conducted after each interview. This allowed identification of issues that required further exploration in the interviews that followed. (282) Continuous analysis of collected data was performed. More interviewees were then chosen according to theoretical sampling.

Through theoretical sampling, interviewees are chosen to meet the objectives of the research; this allows the research question or objective to be thoroughly investigated. (264) The initial group of interviewees included pharmacists, since they were the ones who first responded. Without including experiences of the managers (executive directors) and doctors, the interpretation of the data would
reflect only the perceptions of the pharmacists. Therefore an attempt was made to include senior medical doctors and people in managerial positions.

After preliminary analysis was performed, segments (paragraph, sentences) were coded and labelled. Coded segments were then compared for differences and similarities of events and ideas. This process was repeated until all comments were assigned to categories (constant comparison). (262, 309) As per the grounded theory approach, analyses of the data were done through a set of relevant and specific questions: What is being described here? Who are the actors involved? How do they characterise the situation? What is its meaning to them? (309)

Systematic analysis was conducted following the steps typical of grounded theory: (309)

- Open coding produced the full range of categories that fitted the data.
- Axial coding allowed links between categories to be made.
- Selective coding facilitated the development of a core category.

The coding scheme was organised along tree axes:

- Action/Interaction
- Consequences
- Conditions

QSR NVivo® Version 2.0 (QSR International, Australia) was used as a data management tool.
5.3.6.2 Validity and reliability

Similar steps as those described in the previous chapter were followed to ensure reliability and validity of the data.

*Face validity* – as in Chapter Four, interviewees were invited to check the edited transcript. Decision-makers declined based on the same reason given by the HCD-SC members, they were “too busy”. Therefore “respondent validation” was not performed.

*Negative case testing* – in this study categories were reappraised by the researcher to judge whether any data had been misplaced.

*Intra-observer consistency* – more than half of the interviews were coded on two separate occasions to ensure consistency with the coding.

*Audit trail* - the process from initial coding to incorporation of the concepts into an explanatory scheme was recorded. This included documenting major analytical and methodological decisions.

*Internal validity* – Data relating decision-makers’ perceptions and concerns was triangulated to the HCD-SC study, as described in Chapter Four.
5.3.6.3 Quantitative data

Data relating to the demographic characteristics of the participants were analysed using the Statistical Package for Social Sciences (SPSS®) for Windows Version 10 (SPSS Inc, Chicago, USA).

5.3.7 Ethics

This study was approved by the University of Sydney and St Vincent’s Hospital Human Research Ethics Committees, and was endorsed by the CEO of the South Eastern Sydney Area Health Service.

5.3.8 Funding

This project was funded in part by the Society of Hospital Pharmacists of Australia (SHPA) Research Grants and Awards Scheme through the DBL Development Fund.

5.4 Results

5.4.1 Study participants

Thirty seven people were invited to participate. Seven did not respond, three said they were too busy, however one of these three later agreed to participate. Four considered they were not appropriate and recommended someone else. Twenty-four people expressed interest in being interviewed. Twenty five semi-structured interviews were conducted. One participant refused to be audiotaped and a second participant was interviewed twice to clarify previous answers. The demographic
characteristics of the participants are shown in Table 1. All interviews were conducted by the same investigator and had a mean duration of thirty five minutes.

5.4.2 Themes

The interviews drew out a broad range of perceptions, concerns and attitudes about access to HCMs in public hospitals. To allow the reader to judge the veracity of the interpretation, quotations were used to illustrate the themes presented. The quotes selected are meant to be illustrative of the themes. Names and places have been changed to protect anonymity.

Table 5.1 Participant demographic characteristics (n=24)

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<tbody>
<tr>
<td>Age</td>
<td>Mean 51 years</td>
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<tr>
<td></td>
<td>Range (30 – 72 years)</td>
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<tr>
<td>Gender</td>
<td>54% Male</td>
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<td></td>
<td>46% Female</td>
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<tr>
<td>Organisation</td>
<td>87.5% Public Hospital</td>
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<td></td>
<td>8.3% Area Health Service</td>
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<tr>
<td></td>
<td>4.2% Advisory body</td>
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<tr>
<td>Position</td>
<td>42% Administrative</td>
</tr>
<tr>
<td></td>
<td>25% Senior medical doctor</td>
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<tr>
<td></td>
<td>21% Director of hospital pharmacy department (DoP)</td>
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<tr>
<td></td>
<td>12% Other (deputy director of DoP, area advisor)</td>
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<tr>
<td>Education</td>
<td>46% Medicine</td>
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<tr>
<td></td>
<td>29% Management *</td>
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<td>25% Pharmacy</td>
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*(eg. academic background included degrees in Government and Public Administration, human resources management, and general management).*
5.4.2.1 High Cost Medications

- Definition of high cost medication

Most participants defined HCMs in two different ways: medications with a high acquisition cost used in low volume; medications with low acquisition cost but used in high volume. Only a minority attempted to give a dollar value to the definition. They perceived that ultimately they were concerned about those medications that had an impact on the capped and limited budget.

“So I think you have to talk about the two situations, one being that a drug that costs a thousand dollars is given for a short period of time, and other drugs which cost less than that are given over larger periods of time.” Interview#9

“the more common thing that I worry about is in an individual case where you have something that is say over $15,000 per year. That is the sort of figure that I start getting worried about.” Interview#18

“The high cost drugs that we tend to focus on are those in which the individual doses or courses are expensive and as a consequence a small variation in the number of eligible patients makes a large variation in the hospital’s budgetary position.” Interview#1

5.4.2.2 Cost

The majority of the respondents perceived that HCMs were “overpriced”. Some believed that the investment in research and development was not what drove the cost of these medications. Some participants perceived that at the end of the day pharmaceutical companies are like any other industry and they are in the business to “make profits”.

"overpriced, that they don't reflect the costs associated with their development, production or distribution. The pharmacy industry makes more profit than any other industry in the world by a long way". Interview #6

"It is very clear that the drug companies work on a share market basis. They are in it for the pocket and anybody who doesn’t understand that, they are being very naive. They are not charities, they are non-government organisations, they are share market driven profit driven companies and they are in it for the buck”. Interview #17

"look at all the money they spend on the marketing, and the training and the other issues as other people may feel as unnecessary marketing, but that is commercial and you can’t stop that. But all the costs associated with developing a drug may not necessarily be into research and development rather than the marketing aspect of it". Interview #22

Even though the discussion was regarding access to HCMs in public hospitals in NSW (where the Pharmaceutical Benefits Scheme (PBS) had not been introduced), the following comments emerged during discussions about costs. Most participants perceived that the cost of medications in Australia was lower compared to other countries. This, according to participants, was an influence of the PBS. They also expressed concerns about the effect the Free Trade Agreement between Australia and the United States would have on the PBS and the cost of medicines. This perhaps reflected the media commentary at the time of interviews.

“I think it is really clear in particular they (Pharmaceutical Benefits Advisory Committee-PBAC) do a fantastic job and I am really concerned about the free trade agreement that is being discussed which will undermine what is happening, what has been so good about the PBAC and I am very
concerned that the pharmaceutical industry have been talked into undermining the people out there”. Interview #6

“I think we’re lucky in Australia that we have the system that we do where the PBAC really fight for lowest cost and is often a lot lower then anywhere else in the world” Interview #9

“And that is part of the discussions going on with the trade negotiations now between us and America and they don’t like the PBS, not so much because they are losing money here in Australia but other countries are looking at that sort of regime to reign in the costs”. Interview #17

5.4.2.3 The decision-making process

5.4.2.3.1 Consistency and Transparency

The majority of participants perceived the decision-making process to allocate resources to HCMs in public hospitals lacked consistency. Hospitals within SESAHS utilised different approval mechanisms and procedures. Access to HCMs varied and it was not known how decisions and policies regarding allocations of resources to HCMs were implemented.

“There isn’t consistency across the area and there is not even consistency about whether decisions are made at a hospital level or an area level so there are differences with their approach”. Interview #6

“I don't know how consistent we are, let alone from one institution to another. I think that would be the biggest problem initially, we're not consistent with our decisions and follow-up decisions”. Interview #11
“I think we have to ensure it is a systematic approach and it needs to be worked out not just at a hospital level but much higher level than that …… we are all struggling though in terms of process and this is really one of the major ethical issues I think we all are facing in the next ten years”. Interview # 17

Participants also acknowledged a lack of transparency of the decision-making process. One member suggested that transparency was an issue not only at the public hospital level but also at the macro (PBAC) level.

“I believe it is transparent as is possible given the resources and the knowledge of people concerned. Even the PBAC that’s got far more scientific resources than the hospital drug committee, doesn’t always get it right”. Interview #4

“We don’t have access to the data. We don’t have access to what the PBS does and that is crazy because we are asked to make the same sort of decision and we don’t have any data”. Interview #10

“The decision-making process by which they (DTC say) yes or no answer would probably be quite difficult to find unless you happened to speak to a member of the drug committee and they were willing to take you through it. I’m sure that the fact that two drug committees come to the opposite decision on the same question would be anything but transparent”. Interview #21

In summary, according to these participants, access to HCMs varied across public hospitals in the area health service. There was a lack of consistency and transparency in the decision-making process to allocate resources to HCMs.
5.4.2.4 Criteria

Participants described criteria such as safety, effectiveness, efficacy and cost. Even though it seemed as though cost was the main criteria used by decision-makers it was perceived unethical to consider it as the sole driver for the decision.

“So it’s total cost that’s the most important driver as far as the hospital relates.” Interview #3

“cost isn’t an appropriate way of rationing the resources, you can’t just deal with the costs alone there has to be cost versus the clinical benefit.” Interview #17

“Quality, effectiveness, cost comes into it” Interview #20

Participants’ perceptions regarding pharmacoeconomic evaluation as a criterion to allocate resources to high cost medications varied. No attempt was made to verify their understanding of pharmacoeconomics. In general, most study participants described that pharmacoeconomic evaluation was not currently used in public hospitals. Participants identified three main barriers to its use:

- Lack of expertise and knowledge to analyse the data derived from pharmacoeconomic studies.
- The fact that most of the studies were sponsored by pharmaceutical companies.
- The perspective of the PBS submission (ie. Commonwealth Government) differs to that of public hospitals. Pharmacoeconomics studies conducted are from the societal perspective (PBS subsidy criteria).
“It would be nice to have access to cost effectiveness analysis done by professionals”. Interview #3

“doing their economic studies as to the reductions of length of stay and all of those sorts of things, but of course that is completely useless to a public hospital point of view”. Interview #16

“And we don’t have enough experts, we’d oversee so called pharmacoeconomic evaluations and you can’t directly relate them to the Australian scenario, it is very very hard, there are so many issues”. Interview #20

Despite participants’ perception that the role of pharmacoeconomic evaluation was limited, one of the issues that emerged was the notion of how much is a life worth and the relationship between cost and benefit. Some respondents considered that with a capped budget it was hard to allocate resources to some of the new HCMs which were targeted at improving quality of life. The question asked by some respondents was “how much do you spend on someone’s quality of life?”

“rapid emergence of some new drugs, some of them work extremely well and are very beneficial to patients, not necessarily for cure but for quality of life and so there are some real ethical issues around who gets access to drugs.” Interview #8

“you are giving them extra months of quality life. Nobody’s life is more valuable than anyone else’s, particularly where you have got a young person or a mother with children or something like that. You tend to have that emotional feeling where surely they deserve to have it”. Interview #20
“The difficulty with human life is that as the economics say, there is no price able to be put upon it. We consistently use the terminology “saving lives”. In fact the correct terminology is “delaying death”. Because regardless of how much we spend, how many drugs we give or whatever else we do, we will all die”. Interview #21

Furthermore some participants also considered they followed the utilitarian principle of “the greatest good for the greatest number”.

“We do apply roughly the same criteria, philosophical criteria, in the sense that we try to get the maximum benefit for the greatest number”. Interview #1

“If this one costs $2 million and can treat 3 people and this one over here costs $20,000 and can treat 53, how are you going to make those decisions. So it would have to be some sort of sense of weighing up the greater good as opposed to the individual”. Interview #8

In deciding who should have access to HCMs, participants also identified criteria such as “clinical need” and the lack of an alternative treatment.

“Clinical need, including the fact that there is no other alternative and a total management plan around the care of the patient”. Interview #7

“I mean, let’s say with Crohn’s disease, if they’ve tried everything else and you’ve got some otherwise productive member of the society who is failing everything else that has been tried, you might want to try a high cost drug. It’s warranted I think, with someone like that”. Interview #12
5.4.2.5 Problems and concerns

Respondents were asked about what they perceived to be the main problems and their concerns with regards to access to HCMs. Tensions were identified between:

- Funding models, Commonwealth versus State and Territories
- Private hospital versus public hospital
- Individual versus public good
- Providing access to HCMs and budget constraints
- Level of decision-making and resources

According to participants, the consequences of these tensions lead to “inequities in access to HCMs”. What follows is the description of each of these themes.

One of the main themes that arose was the tension between funding models. Participants perceived the current health care system funding model as an obstacle to “equity” of access to HCMs. Participants used terms such as equity and equality interchangeably. In this study the different meanings were not explored.

“We have a two pronged system and it is not very clear where the accountabilities are. It should be at both state and national levels. Cost shifting and all sorts of things going on and both recognise and don’t even fight it so much now. ………..perhaps a more centralised funding approach might be more appropriate for some of these high cost drugs”. Interview#17

“The anomaly we see and the system which appears to be lacking at a Federal/State interface level is that the decisions of the Federal
Government are not binding on the State Government and hence the decision that the taxpayer should not pay for this drug is overturned at hospital level and the self-same taxpayer pays for the drug.” Interview #21

Participants were also concerned that there were “inequities” in decisions for individual patients according to public or private sector status. This meant differences in access to medications between private and public hospitals.

“there are issues relating to equity and access between private hospitals and public hospitals. There is a number of drugs which are available on the PBS in some cases not available in public hospitals because they have been deemed too expensive or their use may be restricted”. Interview #2

“Where there are problems in equity of access are where you have private hospitals and there you have drugs like Active Protein C which is available on the PBS and can be given to any patient at all in the private hospital provided they get their PBS authority……. and as we know the PBS is funded by the Commonwealth. In public hospitals the drug is funded by the State and the hospitals have to look at how they can afford this very expensive drug and so there is an inequity between public and private”. Interview #20

The tension between individual and public good was also identified. Some participants expressed difficulty in balancing the needs of individual patients versus the whole population. By allocating resources to a HCM for a particular patient, the hospital was denying access to unidentifiable individuals.

“There is a trade off then between the hospital’s duty of care for the patient and the fact that you’ve got an external framework that is supposed to be
“providing care for the whole population which is clearly failing individual patients”. Interview #8

“………how should (we) balance against the hundreds or thousands of patients who could have received treatment otherwise but who are currently on a waiting list or in a queue (that question) is simply not asked. So the difficulty for the health care system and people like drug committees and clinicians is how do they satisfy infinite demand with finite resources and any decision-making system as to the ethics or economic consequences of one person receiving $2 million of medication X a year is simply not in the equation”. Interview #17

For some doctors the conflict they faced was between the duty of care to their patients and looking after the hospital budget.

“…but it then cost the hospital, so it creates, it actually makes decision-making more difficult sometimes because I guess you are trying to protect the hospital but at the end of the day you are trying to do the best for your patient. I have this dilemma that I have this duty of care to the patients and I have a duty to use the health budget as wisely as I can and sometimes it is a hard job to come up with the answers”. Interview # Dr. Y

“Well it’s all about access and I come also as a physician who would like to prescribe high cost drugs sometimes. So it’s really that there are advances in the field that we would like our patients to have. …… I think that the question is how can you understand what they all mean financially and how can you make sure that you’re roughly on target, how can you make it equitable, finally how can you afford it, because that is the big challenge”. Interview #Dr. Z
Participants thought that as hospitals deal with scarce resources and capped budgets allocating resources to HCMs meant they had to give up something else. The notion of benefits foregone, known as “opportunity cost” was noted by some respondents.

“if you don’t (calculate) what you want to put aside for your high cost drugs, then you might be unnecessarily squeezing other costs, whether it is replacing a light bulb or cleaning a toilet, it still has to be done. I think by and large, we just get fixated on drugs because (they) are such an obvious high variable cost when in fact (they) are only one part of a whole range of costs. What drugs are of course is a variable cost unlike, say staffing, which is a relatively fixed cost. You can’t rock up in the morning and try to close Ward 5”. Interview #1

“I suppose it is mainly about making sure you have the decision-making process right, that you have got an equitable process in terms of resource allocation of high cost drugs versus joint replacements versus cardiac stents versus other forms of intervention and I don’t think we (have). They tend to come out of different buckets (which we) look at quite separately and we probably need to be a little more sophisticated in the way we allocate our resources, again it is a factor of an ethical issue”. Interview #17

Participants also identified that sometimes the decision-making process is not linked to resources. Some expressed concerns about decisions being made at different levels, with no consideration with regards to the resources available.

“And because the advice needs to go to where the decisions are --- allocation decisions are really made and if they’re to be binding on a facility then it has to be (understood by) those people have to be part of that decision”. Interview #4
“Now if that decision has been taken, it can’t be taken absent of resources. Because it is a policy decision then the implication in my mind is that they are basically requesting that we reprioritise our funding and its coming from an area point of view”. Interview #16

Some of the concerns identified related to:

- Access to paediatric HCMs
- Access to HCMs for specific groups of patients
- The pharmaceutical industry using public hospitals to introduce HCMs before registration or subsidy

One of the concerns raised by those participants working in paediatric hospitals was the limited access to HCMs. According to these participants, limited research is conducted in children and therefore registration of some medications for paediatric indications is not achieved. Some participants perceived that children are denied equitable access to medications since data are not generated in clinical trials to support the indication in children.

“they (the drug companies) do not do the work and they refuse to do the work and without having an approved indication you cannot have subsidisation so I think there is a huge inequity with adults and children”. Interview # 20

This perception was not limited to paediatric medications. Some participants considered that evidence of effectiveness was limited for some of the new HCMs. Participants considered that pharmaceutical companies were not willing to invest in research for “small non profitable patient groups” and therefore the effectiveness
data were not available to support the allocation of resources HCMs. Some participants considered that the lack of evidence on effectiveness was not sufficient ground to deny access to an HCM.

“...lots of these high cost drugs are for a very small group of patients and it would never be economical for the drug companies to run a clinical trial for such a small patient group cause they won't get their money back”. Interview #5

“I'm the pharmaceutical company that's made it and I know that there is a good market there in cardiology, I may not want to put the resources into researching whether it's going to help the odd person with pulmonary hypertension so some of the clinicians will never be able to get the access through research, to get the evidence, to get the new indication and so they're sort of caught in the bind there”. Interview #7

“Sometimes the evidence to support those solutions are very thin on the ground, maybe one case report of some marginal benefit but it may be a very compassionate sort of scenario and therefore you are prepared to lower the threshold for evidence given the serious potential outcome of doing nothing”. Interview #18

Pharmacists were concerned about the origin of the HCMs. Biotechnology derived HCMs seemed to raise more concerns as being likely to be very expensive. Pharmacists comments included: “biologics are extremely in another league altogether” and “new sort of biologics which are going to become more and more expensive”.
As an incidental finding, some doctors acknowledged obtaining subsidised access in unapproved ways. Some mentioned it was the only way some of the patients would have access to a subsidised medication (eg PBS S-100). They would sometimes falsely claim the patient had a disease or complied with the selection criteria in order to obtain the subsidy for the medication.

“there are drugs where you use for breast cancer, but not approved for it and there are certainly some examples where you have got to say they have got some other disease so they can get the drug”. Interview #Dr. X

Some decision-makers considered that pharmaceutical companies use hospitals to expose doctors to HCMs before these medications are listed on the PBS. Some mentioned mechanisms such as familiarisation programs or provision of free stock. Some participants also considered that public hospitals (via clinicians) were the “back door” for medications that had been rejected by the PBS.

“……..patient familiarisation. Personally, I think that's a sneaky way of the pharmaceutical companies getting their drug and, you know, familiarising (a lot of people), you know, the patients, the medical staff etc before it had PBS listing, and ethically once a patient is commenced on a drug, it's impossible to withdraw it when it hasn't received whatever sort of listing that they're applying for”. Interview #11

“I really think it is disgraceful what they (pharmaceutical companies) do and we won't take part in any of those familiarisation programs because the problem is that the patients get established on the drug, the doctors like them and then suddenly the familiarisation program stops and there is this huge gap and the hospital ends up having to pay for it”. Interview #20
“The problem that we and other hospitals in other States have is that again, by a combination of the drug companies and the clinicians, a drug that is not PBS listed, clinicians attempt to use our hospitals frequently via their own out-patient clinics to have those drugs prescribed at public hospital expense”. Interview #21

On the other hand, some participants thought that public hospitals had to be at the “cutting edge” and therefore were required to fund new, expensive medications. This meant using HCMs either before TGA approval or for “off-label” indications, and before (or without) subsidy though PBS S-100 listing.

“so by and large, teaching hospitals are where new and expensive therapies are used first to the utmost degree of good.” Interview #1

“……….cutting edge hospital that has got leaders in those areas they want to be looking at evidence and everything else involved in the research, well part of the job is actually to be innovative you know cutting edge, but that has got to be done with great care and debate about allocation of resources and all the rest of it. So people often say well there is too much off label drug use in a place like this and my own view is that there is probably not enough off label drug use because if you are really treating people optimally then you have to use off label drug”. Interview #10

“teaching hospitals are always at the cutting edge of the treatment developments, so you are always going to have the innovation of the developments taking place of the pressure, taking place in these environments and that is good. There is research, there is teaching, there is evidence based practice, there’s a few that will need to move out their treatments, protocols this way or that way and so drugs are a critical part of it”. Interview #16
The next section discusses themes related to setting priorities for HCMs and the role of the general public in these sorts of decisions.

For study participants in hospital and Area Health Service managerial positions, choices about how to allocate scarce resources are made everyday. “Rationing” may occur without this being done in an explicit way.

“You pretend that you don’t have to make those decisions in terms of rationing services but we do, we have to make those decisions every day and we do make them every day but it would just be a lot better if we actually made them in a more consistent way”. Interview #14

“At the end of the day, the resources that the community had given us for health are capped and therefore decisions about rationing are real and happen all the time but nobody talks about it and said there is very poor community understanding of this major dilemma and very little debate about it. People seem to understand that rationing (occurs) but when you start then bringing into the equation, like should we be rationing who gets a renal dialysis or who gets a high cost medication, that debate hasn’t been had yet”. Interview #18

The majority of participants perceived that the “rationing debate” needs to happen in Australia and the community at large needs to understand that health care resources are limited and choices need to be made. However public involvement, according to participants, should be at a macro level.

“At the societal level we have to decide how far we are going to go and at the moment all of these decisions are being made in closets, you know,
within camera .......... you have to know what is going on, it is not a matter of the drug committee it is a matter of community for everybody and it is not openly debated and it really needs to be openly debated”. Interview #6

“I think where the public needs to be involved is if we work towards having a system whereby we say this is how much we are spending and this is the priorities. If we are going to introduce this we have got to knock something off and what we should do over the next few years is start to have this discussion publicly over and over and have people say what they think”. Interview #14

“…..until we have a community debate about what are we prepared to spend on health? How much tax are we prepared to pay? Are we prepared to continue with the situation where 50 percent of health care expense is in the last six months of life? Are we prepared to spend the sorts of money we are on the $2 million-a-year-person?” Interview #21

Different views were identified regarding the role of the “community”, consumer, and the general public (these terms were used interchangeably). Participants commented about public involvement in different ways. Most considered that the general public should be aware that if they want access to HCMs they may need to pay more taxes.

“It’s a challenge to get people the general population to realise that drugs, that equity, that access to drugs is going to cost a lot more money in the future and that if people expect to have access to those drugs probably more money needs to be diverted to help for drugs funding. I think the population in general doesn’t realise that and the challenge will be to get more of the health dollar available for drugs and to get the money spent effectively.” Interview #5
Some participants considered it was difficult to identify who represented the public. The general public, according to some participants, could focus on individual good and be biased towards their own need instead of adopting a societal perspective.

“but who represents the public? Often consumer activists are as typical as a kick boxer I don’t know” Interview #1

“but the public looks through their own eyes at what is my problem, and I am not getting access to a drug that is going to save my life, and I don’t have the money to pay for it, therefore I am disadvantaged (compared) to somebody who does have the money to pay for it and can get help.” Interview #16

Participants also considered that Australia’s health care system is complex and the general public might have difficulty understanding it. Some also suggested the general public would need to be educated regarding priority setting.

“I think the health system is very complex......and the reality is different to the policies. And every hospital situation is different”. Interview #13

“the State or the Commonwealth Government needs to start looking at an education program for the general populace that you can't necessarily have everything and there just isn’t infinite money and all that sort of thing”. Interview #20

“I think what consumers need to do is understand the extent of the problem and understand the decisions that have to be made and be prepared to accept the decisions that are made for them or on their behalf”. Interview #6
Participants were concerned about the role of the media and the sort of information the general public gets about access to HCMs. Some participants considered that the message sent by the media is not always right and tends to focus on cases where access to HCMs is denied.

“I think the media, the messages the media give the public is different from the reality and the reality is different to the policies as well. And every hospital situation is different”. Interview #13

“Because all they get is the opposite view, what the public gets is poor Mrs so and so who has got three children and she is dying of whatever, and the PBS not approve the drug that she needs to keep her alive on the PBS. Everyone goes, how terrible, that is awful, she should have the drug and our heart knows that she should have the drug but you have to think well you know the reason they haven’t put it on is that the evidence isn’t strong enough and you have got to have a basis for subsidizing something”. Interview #20

“…suddenly it becomes a big media story and then of course you get all that pressure and politicians and the thing is do we want this to go away and the way you do that is to address that issue”. Interview #24

5.4.2.6 Solutions

The majority of respondents identified problems; however they had difficulty in identifying solutions. Some perceived that having a single national body to fund pharmaceuticals would overcome some of the current problems.

“universal of funding of pharmaceuticals by the commonwealth” Interview #20
“it would be easier to make it transparent and equitable decisions by having a single funding body”. Interview #19

Some respondents considered that public hospitals should adopt the decisions taken by the PBAC. This meant that if a medication was denied PBS listing it should not be available in public hospitals.

“It would simplify matters enormously if the States and the Commonwealth agreed that a PBAC listing that a particular drug should not be publicly funded meant exactly what it said and that there was no debate at State level or area level or hospital level”. Interview #21

Others considered that the role of the PBS in public hospitals was limited. Some respondents considered that conditions that are typically dealt within hospitals are different to those seen in the community setting and medications are required for different indications. Some respondents also considered that having PBS for outpatients in public hospitals was not a feasible option for NSW due to the lack of (administrative) resources.

“we have to be more flexible because essentially the Australian system of PBS is bureaucratic and very rigid about what they can and can't do”. Interview #1

“I think there could be some other modifications of the PBS that might allow for indications that are typically dealt within hospitals as opposed to the community”. Interview #6
"In public hospitals in NSW we couldn't take on the PBS system the same way that it has occurred in other states". Interview #20

“But then I don’t know if you went to check the PBS within hospitals, you’re still going to be left with all the non-PBS indicated items. Who’s going to fund those?..........PBS that’s the funding source, I mean that’s been really designed for community and so how applicable is it to hospitals? I think you’ll still have issues with high-cost, you know, the funding of them". Interview #23

They also speculated about having a state-based approach to dealing with HCMs and the potential involvement of advisory bodies.

“Well if we’re still having separate funding for hospitals, I think some of these issues should be a state approach because it’s not --- it’s inequitable”. Interview #23

“I think the TAG group (NSW TAG) although they are not a government body, .....they have the skills to see whether these drugs, high cost drugs are they well worthwhile…”. Interview #24

5.5 Discussion

5.5.1 High Cost Medications

Despite many allusions to cost of health care there is no single generally accepted definition of HCMs. This may be important in the interpretation of data and development of policy. (75-78) In this study, participants unanimously defined HCMs as those which could have an impact on capped and limited budget holdings. Similar definitions have previously been described. (60, 85) The participants were
principally concerned with those medications with a high acquisition cost and low volume of use.

Consistent with participants’ perceptions, Moran in the UK considered that pharmaceutical companies spent more money on marketing, advertising and administration than they do on research. (314) This perception is not unique to Morgan. Other authors in the USA have expressed the same concerns. (11, 65-67)

An incidental finding of this study was participants’ perception that despite HCMs being ‘overpriced’, the cost of pharmaceuticals in Australia is lower compared to other developed countries. Participants attributed this fact to the PBS and concerns raised regarding the impact the Free Trade Agreement between the USA and Australia would have on the PBS and therefore on medication costs. Several authors have commented on the potential impact of the Free Trade Agreement negotiations on the PBS. (315-320) However it is important to note that the role of the PBS in controlling costs and the impact of the Free Trade Agreement were beyond the scope of this study.

The Productivity Commission, one of the Australian’s Government’s principal advisory bodies on all aspects of microeconomic reform, conducted a study in 2001. (321) This study aimed to investigate the differences between the prices of pharmaceutical benefit items in Australia and the prices of the same items in comparable overseas countries. (322) The study showed that manufacturer prices in Australia for pharmaceuticals (listed on the PBS) were lower than in the United States, Canada, the United Kingdom and Sweden. (322) The report also considered that it was difficult to identify robust specific explanations. For Taylor et
al, the monopolistic power of the Australian government is a factor that might influence price and allows the government to negotiate lower prices. (120) Both Taylor et al and the productivity commission report also considered that the use of reference pricing might also help keep the prices down. (322, 323) Nevertheless Australian prices for new innovative pharmaceuticals were found to be close to prices in other developed countries. (322)

It is important to note, as described in Chapter One, the role of the PBS in public hospitals is limited to outpatient prescriptions in some states. Inpatient medicine costs still remain the responsibility of public hospitals and state governments.

5.5.2 The decision-making process

5.5.2.1 Consistency and Transparency

The majority of participants considered that the decision-making process to allocate resources to HCMs in public hospitals in the SESAHS lacked consistency and transparency. According to the Society of Hospital Pharmacists of Australia discussion paper regarding funding of medicines in Australian hospitals, although all public hospitals across Australia seemed to have the same issues, it appears that the decision-making processes used are not consistent across hospitals or areas or states. (1) Benatar in South Africa, considered that few people know how resources are allocated at the level of major hospitals. Decisions seemed to be made secretly and "within a vacuum". (305)
These perceptions were also consistent with those results found in Chapter Four. Elsewhere, studies regarding priority setting for new technologies, including HCMs, have also found similar perceptions. (141-143, 226, 295)

The lack of transparency of the decision-making process to set priorities has also been identified at different levels of decision-making and in different settings in Australia. In a discussion paper by Mooney regarding Western Australia’s health service, the author considered that the lack of transparency lead to inequities and lack of allocative efficiency. (324) Mitton’s study with decision-makers in the South West Area Health Service in Western Australia also showed that decision-makers wanted a transparent approach for setting priorities. (325) Finally the Cookson case study of the PBS also described that this decision-making process lacked transparency. (122)

In the current study, according to participants, the lack of consistency and transparency meant that the availability of medicines varied across hospitals in the area. Similar concerns have been previously expressed by Low, who considered that the availability of medicines to patients varies within public hospitals in Australia. (97)

### 5.5.3 Criteria

Participants were able to identify criteria such as safety, effectiveness, efficacy and cost. A study conducted by Zwart-van Rijkom also showed that decision makers prefer to weight a broad range factors regarding allocation of resources to healthcare technologies including medications. (115) Pharmacoeconomic data were
reported as not currently routinely being used in the decision-making process. Lack of expertise and knowledge were identified as barriers to its use. Similar results have been previously obtained regarding the use of pharmacoeconomic data. (115, 146, 151, 236, 326)

In our study, the limited pharmacoeconomic data derived from studies using the perspective of the hospital was identified as a barrier to its use. For Drummond et al decision-makers at the institutional level need to balance a budget and therefore the societal perspective might not be the most relevant one. (327) On the other hand, Weekes considered that cost effectiveness can be difficult to manage in the hospital environment of capped budget. (144) Perhaps, as expressed by McDonald, the use of technical solutions such as economic evaluation to allocate resources at the institutional (meso) level could be limited and maybe the focus should be on having a fair and legitimate process for setting priorities. (328)

Participants were also concerned about balancing cost and benefits such as improving quality of life. They also wanted to achieve the greatest good for the greatest number of people when allocating resources to HCMs. This was also consistent with the results from Chapter Four.

5.5.4 Problems and concerns

“Inequity of access” was a recurrent theme in this study. Most participants considered there were “inequities of access” to HCMs at different levels and due to different issues which will be explored throughout this section. These include inequities:
• Between private and public hospitals due to different funding models.

• Between children and adults due to the lack of research and evidence of effectiveness.

As previously stated participants used terms such as equity and equality interchangeably. The scope of this study did not allow exploration of the different meanings. Some authors have expressed that there has been confusion between equity and equality. (329, 330) For some the definition of equity includes a sense of fairness in distribution of resources. (331-334) However it is unknown what participants in this study understood by the word equity.

Dealing with HCMs was considered difficult by participants for a number of reasons; including the complex health-care systems, Commonwealth and State Government as well as public and private systems. Decision-makers perceived the current health care system funding model was an obstacle to “equity of access” to HCMs. According to participants there were inequities because of the funding models in place capped in public hospitals and uncapped for the PBS. As a consequence there may not be enough funds to pay for HCMs in public hospitals.

Concerns about the consequences of the fragmentation and “lack of cohesion of the system” have been debated by several authors. (16, 49, 94, 306, 335) To illustrate this issue better in 1990 Peter Read, former Executive Director of the Australian Hospital Association expressed that: “…….hospitals are only a part of a much more complex and intractable problem --- the split responsibility for health financing. The current arrangements encourage duplication and overlap. They
promote larger rather than smaller bureaucracies. They make cost shifting, finger pointing and buck passing into an art form”. (336)

For Doecke, it is ironical that the source of funding for medicines is ultimately the same with the State funding coming indirectly from the Commonwealth. Doecke also considered that this leads to differences in access from State to State, hospital to hospital and patient to patient. (2) Reid considered that the different funding systems for pharmaceuticals sometimes results in public hospitals not providing adequate medications for patients on discharge. (337)

Tensions between individual and public good were identified as participants’ concern. Consistent with results from Chapter Four participants at the meso (institutional) level were sometimes faced with the identifiable individual. Finding the proper balance between interest of the collective and individual was considered difficult by participants. (338)

O’Donnell described this tension as rationalism (the individual) versus empiricism (the population). In O’Donnell’s discussion the author presents the case of Infliximab to treat rheumatoid arthritis compared with the costs of treating people with statins ie. Improving the quality of life of some identified individuals and balancing the long term benefits to an unidentifiable, but statistically significant, number of people. (339)

Klein considered that when allocating resources in healthcare managers are guided by the needs of the population. Doctors on the other hand are guided by the needs
of the patients. (340) However according to the findings in the current study decision-makers at the institutional level have to balance both population and individual needs. This study also showed how decisions at the macro (PBS) level differ from the meso (institutional) level. Whereas public hospitals via their DTCs are able to make decisions at the individual patient level, the PBAC has to make decisions which are community-wide. (1)

Participants considered they had a capped budget within which there was the responsibility to distribute resources efficiently and fairly. For Campbell “fairness is a key ethical goal. Decision-makers need to be seen to have acted fairly, and so they determine what is fair by virtue of process”. (341) However as previously described in Chapters Two and Four according to “accountability for reasonableness”, for a process to make legitimate and fair decisions on priorities, four conditions must be met: publicity, relevance, appeals and enforcement. (221, 225) So even though participants wanted to distribute resources in a fair way the decision-making process lacked consistency and transparency. Ham previously identified that there is a gap between decision-makers desire and the current reality. (300)

Decisions about access to HCMs according to participants, needed to be made not only on grounds of cost but opportunity cost to other patient groups. (342) Participants also considered that it was hard to balance the opportunity cost of allocating resources to HCMs as compared to other areas within the hospital.
Some participants also expressed concerns about decisions being made at different levels (eg. area, institution) with no considerations of the resources available. Smee considered that it is important to move the decision-making close to those primarily affected by the decisions. (117)

Participants who dealt with paediatric HCMs considered that children had limited access to HCMs, mainly due to the lack of effectiveness data. Gazarian described children as “therapeutic orphans” and, like participants in this study, considered that children are denied access to medicines due to the lack of evidence of effectiveness. (343)

However the scenario according to participants was not exclusive to children. Evidence for some patient groups with uncommon diseases was also limited. As expressed by the participants, decisions were sometimes made with low levels of evidence. Participants considered that the patient might not have other alternatives given the kind of disease being treated (eg. devastating, life threatening, uncommon). As previously expressed in Chapter Four participants in this study also acknowledged they were sometimes guided by the “rule of rescue”.

There seemed to be two opposing views to allocation of resources to HCMs for an uncommon disease with lack of evidence. The first view is expressed by Brinsmead, who considered that the fact that a person had a rare, as opposed to a common, condition was not a “good moral basis for accepting higher opportunity costs (meaning additional health sacrifices imposed on others)”. (344) Weale also considered that when resources are allocated to expensive treatments with lack of
effectiveness data, there is a conflict between medical ethics and the administrative imperative to secure value for money in health care. (345)

The second view is exemplified by the case of riluzole® for amyotrophic lateral sclerosis in the UK. (342) Despite the medication not being cost effective it was funded through the UK National Health Scheme. Maybe this was done given the nature of the disease (no cure available and degenerative) and the fact that the treatment relieved symptoms and improved the quality of life. (342)

These examples and the study findings may allow hypothesising that decisions regarding access to HCMs at the macro and meso level are complex and there are considerations that go beyond effectiveness, cost and opportunity cost. For Walley, decisions about the allocation of resources to medications within limited budgets (eg. public hospitals) could also be based on compassion and social factors. (59) Komesaroff, considered that when allocating resources in hospitals, ethical values like justice and respect for human autonomy and qualitative aspects of caring and community support should also be considered. (306)

Finally, Martin described that key values in decision-making include equity, public versus individual health and the rule of rescue. (346) Resource allocation could also be described as an ethical decision based on values. (341) Fuschs, expressed that “our values colour our judgement about policy”. (347) The question to be asked is whose values should be considered? In an environment characterised by a more informed and demanding public, it is important to recognise the role members of the general public values could play in the decision-making process. (300)
Participants expressed concerns about the pharmaceutical industry using hospitals to introduce HCMs before they had been approved by the TGA or listed on the PBS (eg. via outpatients). According to SHPA, medications are used in hospital prior to PBS listing or TGA approval. (1)

Some participants expressed that they were first exposed to HCMs due to the fact that public teaching hospitals had to be at the “cutting edge” and “off-label use” was unavoidable. On the other hand there were participants who perceived that “off-label” use was a consequence of pharmaceutical companies using public hospitals as a back door to introduce and familiarise physicians with their products.

The “back door” term was also used to exemplify cases where a medication considered cost ineffective for public subsidy (according to PBAC) was provided via public hospital outpatient departments. This according to Doecke is “simply a waste of health resources” and the only way to support equity of access to pharmaceuticals in Australia is to have consistency in the availability across all publicly funded health providers. (2) These concerns are also consistent with results from Chapter Three and perceptions expressed in Chapter Four. Results in Chapter Three showed that more than half of the medications (67.1%) approved via the IPU scheme, were for non-TGA approved indications (“off-label”). Also 12% had TGA approval for the indication of interest but were not funded through Section 100 of the PBS.

One incidental finding was the fact that doctors thought they sometimes needed to lie to obtain subsidised access to a medication (eg. S-100). They also expressed
the conflict they faced between providing care to a patient with a HCM and budget constraints. Liaw et al examined doctor’s perceptions and attitudes to prescribing within the PBS/Authority Prescribing System (APS) in the community. These authors reported that the patient-centred practice seemed to be fundamental to why medications are sometimes prescribed outside of the approved uses of the PBS/APS. (348)

Participants perceived that even though rationing is happening, it is not openly discussed in Australia. This is consistent with findings from several authors, who have identified that the Commonwealth Government denies rationing occurs in Australia. (117, 338, 349)

Some participants considered it was important to educate members of the general public about the need to set priorities. They also considered it important to have an open debate. New, in the UK, described that since rationing is unavoidable, there should be an explicit debate about the principles and issues concerned. (350) Ham considered that in a time when public awareness is increasing about medical advances the challenge is to find ways of informing and involving the public in the debate about the distribution of health care resources. (135)

The role of the public according to participants was considered diverse (tax payers, patients, consumers) and due to the complexities of the health care system their involvement in decisions regarding access to HCMs in public hospitals was considered limited. For participants the role of the public was more likely to be at the macro level, deciding how much they were prepared to spend on health care.
Participants also considered that members of the general public might not be willing to pay more taxes to fund access to HCMs.

Hogg described that members of the public tend to identify themselves with the area they live in. They are more likely to participate in decisions relevant to them and where participation is likely to have an impact. (351) It could be hypothesised that members of the public might be more likely to be involved in decisions regarding access to HCMs at the area health service level. However it is not known if they would be willing to participate in decisions regarding access to HCMs in public hospitals.

Wismenan’s study described in Chapter Two looked at patients’ willingness to be involved in decisions regarding priority setting in health care. In this study participants did not want to be involved in specific decisions eg. allocation of treatment between patients. (195) Participants in Wismenan’s study were patients and it is not known if their views differ from those of the members of the general public.

Participants also commented on the role the media has in shaping members of the general public views regarding access to HCMs. Concerns arose regarding the words the media uses to describe access to a HCM, especially when someone is “denied” access to a medication. Examples included access to trastuzimab and imatinib in Australia (88, 89) and interferon beta-1b in the UK (73). These cases as expressed by Daniels attract public sympathy and show the great difficulty members of the public have with accepting limits. (352) Marley considered that “on
emotional grounds, the public rejects the idea that any treatment, however expensive and unproven should be denied". (128) The media discourse and how it influences views of members of the general public regarding access to HCMs, were beyond the scope of this study.

5.5.5 Solutions

A single funding system was seen by some as a way to overcome the previous problems. (1, 100) However participants achieved no consensus and other approaches were described. Initiatives described by participants were considered at different levels, hospital, area and state. This mirrored current discussions across Australia.

In August 2002 the Western Australian Drug Evaluation Panel (WADEP) was established with the object of providing Western Australian Therapeutics Advisory Group (WATAG) with recommendations on the clinical and cost-effectiveness of new medications having a high cost impact on the WA health system. (353) A state approach was also being contemplated by Victoria. (54) An Area High Cost Drug committee was being considered by the SESAH and as described in Chapter Four the HCD-SC was created at St. Vincent’s Hospital in NSW. A position statement by SHPA in 2004 suggested that a single funding system for medications including HCMs in public hospitals could be one of the options “to move forward”. (1)

Similar to the Australian scenario, Martin in Canada reported that procedures to determine whether health technologies (including medications) were going to be funded were taken at different levels (federal, provincial governments, hospitals)
and using different procedures. Even though they considered similar criteria there was no single widely accepted procedural framework for setting priorities. (346)

The sine qua non that everybody should have access to medications regardless of their costs might no longer be supported. As participants in this study expressed, resources are limited and choices need to be made. Perhaps as expressed by Doyal explicit rationing might contribute “to the moral development of citizens by encouraging them not to believe that they are entitled to everything that is technically possible in health care”. (354) However as expressed by Ham, the decision-making process should be open with a stronger commitment to giving reasons for decisions, bringing forward the opportunity to enhance democratic deliberation, making decision-makers accountable to their stakeholders. (300)

The subsequent study, described in Chapter Six, which explored views and knowledge of members of the public regarding access to HCMs in public hospitals, was designed to address some of the questions raised by participants in Chapters Four and Five.

“Vox populi, vox Dei”. Latin maxim

This chapter describes the perceptions of members of the general public regarding access to HCMs in public hospitals.

6.1 Introduction

It is important to involve the community in the early stages of the debate about what should and should not be paid for in terms of health care. (200, 355) In contrast to Australian society, in Denmark community consultation is part of “Danish life”. Little suggested that community consultation in Australia is possible and might even produce surprising results. (356) Given limited health care resources, a community-wide consensus could be used to decide how best to employ these resources. (357) Decisions could include how to allocate resources to HCMs. (79) In a commentary regarding access to high cost genetically targeted medications in Australia, Hall proposed that the economic and policy challenges imposed by these medications merit public discussion. (358)

Lofgren considered that the introduction of new and more expensive medications on the Pharmaceutical Benefits Scheme (PBS) should ensure intense public
The importance of community consultation regarding access to HCMs can be highlighted by the fact that one of the drivers of health care cost is considered by some to be the patients’ demand for higher cost medications. (59, 352)

In 2002, Senator Coonan (the then Minister for Revenue and Assistant Treasurer) stated that “Australians now expect to access new (and more expensive) medications listed on the Pharmaceutical Benefits Scheme.” Coonan also stated that much of the growth in health spending has come from demand for new technology and treatments. (359)

However, Australians have a range of views regarding access to pharmaceuticals and the PBS. Issues regarding pharmaceuticals that were identified as important by the Consumers’ Health Forum (CHF) were: (214)

- Lack of access to information such as government policy, regulation and operation of the PBS.
- Lack of access to very expensive medications
- Privately versus publicly funded access to medications.
- The need for accountability, transparency and rationale behind the PBAC decisions.

Little is known about public attitudes towards access to HCMs. It is not known whether the general public is well informed or not. Views about access to HCMs in public hospitals have not been explored.
As described in the previous chapter of this thesis, health care decision-makers acknowledged the importance of public participation in decision-making regarding allocation of resources to HCMs in public hospitals. However the results of those studies also showed that those decisions were not generally made in consultation with the community. Decision-makers also perceived that the general public does not have good general knowledge about access to HCMs in public hospitals.

6.2 Aim
The aim of this study therefore was to gather information about the knowledge and views of members of the general public about access to HCMs in public hospitals.

6.3 Methods

6.3.1 Selection of Method
Some care was taken in the selection and development of an appropriate research method given the view that the general public has little knowledge of HCMs in public hospitals. Different methods have been described to explore views of the public about allocating resources for health care. (204) Quantitative and qualitative methods have been used. (179, 208, 209, 360, 361) The choice of methods included: individual interviews which as previously described, can provide detailed information especially about individuals’ experiences. Focus groups are useful to gain insight into people’s views and answer questions about “why” they think the way they do and “how” they are affected. Surveys are useful when exploring “what” members of the general public think about a topic. (204)
6.3.1.1 Self-administered surveys

A survey questionnaire is a quantitative research method best suited for collecting information, although open-ended questions can also be included to provide qualitative information. This involves sampling a population of interest. Bowling classified surveys as descriptive and analytic. Measures are calculated in descriptive surveys whereas in analytic surveys events are analysed at more than one point in time. (263)

Surveys are frequently self-administered and could be classified as: (362)

- Supervised: people answer the questionnaire in the presence of a surveyor.
- Unsupervised: questionnaires are sent by mail and people answer them without supervision.

One-to-one supervision typically provides a higher response rate compared to mail surveys, especially when there are no incentives available for people to answer the survey. Self-administered surveys can also have the advantage of a relatively short data collection period. (362)

Given that the aim was to gather information about the knowledge and views of members of the general public about HCMs in public hospitals, and the concern that this was a subject that the general public knew little about, it was decided that the best method was a supervised one-to one survey. Respondents were guided through the questionnaire and answered the questions in the presence of the researcher. (362) This method made it possible to include questions about their knowledge of HCMs in a situation where respondents could ask questions of the
researcher, and clarification of the question could be offered. It also provided the opportunity for some open-ended questions for qualitative comments and was a method with a predicted high rate of response.

6.3.2 Sample selection

The sampling frame for this study was people living in the Sydney metropolitan area. The population in Sydney comprises 21% of Australia's population. (363)

6.3.2.1 Sample size

Determining sample size depends on 1) the level of confidence, 2) margin of error tolerated and 3) variability in population studied. (364) The target population was people in Sydney, and according to the 2001 census, this included 3,997,321 people. (363) For this study the level of confidence was set at 95%. The sample size calculation assumed a population diversity of 50/50 on the study variables. A split of 50/50 is recommended since it produces the largest sample size requirements. (365) The margin of error tolerated for this study was set at ± 7% (sampling error). A sample of 196 was determined as providing a representative range of views and would allow the detection of a proportion of 50% (the least precise) with a 95% confidence interval (CI) of 7%.
• **Calculations**

1) **Calculation of standard error of a proportion**

   Sampling error = 95% Confidence Interval (CI) x SE (p)

   \[ 7\% = 1.96 \times SE(p) \]

   \[ SE(p) = \frac{7}{1.96} \]

   \[ SE(p) = 3.57 \]

2) **Calculation of simple size (n)**

   \[ SE(p) = \sqrt{\frac{p(100 - p)}{n}} \]

   Where:

   - n= sample size
   - p= proportion of individuals with a particular condition in a population

   \[ 3.57 = \sqrt{\frac{50(100 - 50)}{n}} \]

   \[ n = 196 \]
6.3.3 Eligibility criteria

The criteria established were that to be eligible to answer the survey respondents needed to be:

- Over 18 years of age
- An Australian citizen or permanent resident
- Able to complete the questionnaire in English

6.3.4 Questionnaire development

A survey instrument was developed using information from the literature and the National Resource Centre for Consumer Participation in Health (NRCCPH). (366) The initial questionnaire was distributed among supervisors and a representative from the NRCCPH for appraisal of appropriate wording, readability and content validity.

The questionnaire was divided into four main sections:

Section 1 - Health & health services

Four questions were included in this section (Q1-4). Respondents were asked to rate their health and experience with the public hospital system (frequency of visits—as a patient—by respondent or any family member in the last 12 months).
Section 2- Knowledge

There were six questions (Q5-10) about knowledge regarding public hospitals and access to HCMs. Respondents were given statements and asked to indicate if these were true or false.

Section 3- Factors & choices

This section included six questions (Q11-16). Respondents were asked to allocate resources to HCMs in public hospitals given a limited budget. The first question in this section (Q11) asked respondents which factors were the most important in deciding who should be given a HCM. Respondents were asked to rank factors from one to four, with one being the most important factor. This was then followed by two hypothetical scenarios with multiple options.

Scenario One: was the first hypothetical scenario. Respondents had a limited budget for medications in a public hospital. They were asked to provide a basis for choosing between two patients. In the second hypothetical scenario (Scenario Two) respondents had a limited pool of money and they had to spend it on two treatments: Medication A, to prevent heart attacks or Medication B to improve the quality of life of a person with cancer and lengthen that person’s life. An open ended question was included asking participants for their reasons for their response to the second scenario. In this same section respondents were asked who should make those decisions, if they would they like to be involved in the decision-making and if they were willing to pay more taxes to subsidise access to HCMs in public hospitals.
Section 4 - Demographics

This final section consisted of nine questions (Q17-24) about demographic information which included: age, sex, marital status, language spoken at home, economic activity, highest level of schooling, postcode, annual household income and whether respondents had private health insurance.

6.3.5 Pilot testing

The questionnaire was pilot tested with a convenient sample of 15 people. These were members of the community who met the eligibility criteria. Respondents were advised that the purpose of the pre-test was to improve the questionnaire and were asked to critically evaluate it. Minor changes were made to the format as a result of this pilot testing. The final questionnaire is attached as Appendix 6.1

6.4 Data Collection and Analysis

The survey was conducted between October 2004 and April 2005. Individuals were randomly approached by the interviewer in venues such as shopping centres and on public transport in the Sydney metropolitan area. To make it a representative sample, people were approached at different times during the day and different days of the week including the weekend. (362) Every fifth person approaching the interview setting (train station, shopping centre etc) was invited to participate. If they agreed the interviewer then supplied a subject information statement (Appendix 6.2) which provided background information and guided respondents through the questionnaire. Respondents were encouraged to express their views freely and told that it was their “views and perceptions” that were being sought. Any issues could
also be discussed openly with the interviewer. They were advised that data would be treated as strictly confidential and all the information would be de-identified.

6.4.1 Quantitative data

Responses were collated and analysed using the Statistical Package for the Social Sciences (SPSS) for Windows Version 10 (SPSS Inc., Chicago, USA). Descriptive statistics were used to summarise the data. Frequencies and proportions were calculated with 95% confidence intervals. The sample demographics were compared with the Australian population as a whole using the one sample t test. To test the associations between responses and demographic characteristics (eg. age, gender, income etc.), responses were dichotomised and a chi-square (χ²) test for linear distribution was conducted. Significance was set at the five per cent level.

6.4.2 Qualitative data

Responses to open ended questions were imported into QSR NVivo® Version 2.0 (QSR International, Australia), qualitative research software to facilitate data analysis. The analysis was descriptive and responses were categorised.

6.5 Ethics

This study was approved by the Human Research Ethics Committee of The University of Sydney.
6.6 Results

6.6.1 Demographics

Two hundred and ninety eight people were approached and two hundred people completed the questionnaire. The reasons people gave for not accepting the invitation to answer the survey included: “not interested”, “don't have time” or “couldn’t read English”. Fifty six per cent of the respondents were female and the median age was 36 years (range 18-82). The sociodemographic details of the respondents are shown in Table 6.1 along with the 2001 census data for the Australian population (367) and 2001 National Health Survey Data. (368)

The study sample differed from the Australian population in a number of respects. There was a higher percentage of females, a higher proportion of people aged between 25-44 years (p<0.05), a lower proportion of people aged 65 years or older (p<0.05), a higher proportion of people who were university educated (p<0.05) and respondents from the sample population were more likely to have private health insurance (p<0.05).

In addition, annual household income was higher than in the Australian population. However is important to note that Sydney has the highest average household income in Australia reported as A$63,000 in 2001. (369) Twenty seven percent of people in New South Wales had an income higher than A$80,000 according to the 2003 NSW Health Survey. (370)
Table 6.1 Sociodemographic characteristics.

<table>
<thead>
<tr>
<th>Socioeconomic characteristics</th>
<th>Sample population</th>
<th>Australian population</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Gender (n=200)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Female</td>
<td>56.0%</td>
<td>50.6%</td>
</tr>
<tr>
<td><strong>Age (n=199)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Median</td>
<td>36 years</td>
<td>35 years</td>
</tr>
<tr>
<td>18-24 years (n=24)</td>
<td>12.0%</td>
<td>9.4%</td>
</tr>
<tr>
<td>25-44 years (n=108)</td>
<td>54.0%* (CI 47-61)</td>
<td>29.8%</td>
</tr>
<tr>
<td>45-64 years (n=58)</td>
<td>29.0%</td>
<td>23.0%</td>
</tr>
<tr>
<td>65 years or more (n=10)</td>
<td>5.0%* (CI 2-8)</td>
<td>12.6%</td>
</tr>
<tr>
<td><strong>Marital status (n=198)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Married</td>
<td>46.5%</td>
<td>50.7%</td>
</tr>
<tr>
<td><strong>Language spoken at home (n=200)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>English</td>
<td>84.0%</td>
<td>80.0%</td>
</tr>
<tr>
<td><strong>Level of education (n=200)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>University degree</td>
<td>61.0%* (CI 54-68)</td>
<td>9.7%</td>
</tr>
<tr>
<td><strong>Economic activity (n=200)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Working (full time or part time)</td>
<td>88.0%</td>
<td>92.6%</td>
</tr>
<tr>
<td><strong>Annual household income (n=186)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>&gt;A$100, 000</td>
<td>54.0%</td>
<td>^</td>
</tr>
<tr>
<td><strong>Private Health insurance (n=196)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Yes</td>
<td>68.0%#* (CI 61-74)</td>
<td>43.0%#</td>
</tr>
</tbody>
</table>

† Answers to some questions were not provided by all participants
* One sample * t test p<0.05
^ Average household income in 2004 was $50 000 (371)
# People covered by Private Health Insurance (PHI) refers to anyone with hospital cover, including those with hospital cover only and those with hospital and ancillary cover. (368)
6.6.2 Health and health services

- **Self reported health status**

The majority of the respondents considered themselves to be well (41%), with 90% reporting their health status as good, very good or excellent (Table 6.2) illustrates these results. In 2001 the majority of Australians (82%) also reported their health status as good, very good or excellent. Respondents with higher income (>$50,000) were generally more likely to report their health to be excellent, very good or good. \( \chi^2 = 12.814; df=1; p=0.001 \)

### Table 6.2 Self reported health status

<table>
<thead>
<tr>
<th>Self-reported health status</th>
<th>Percentage % (n=198)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Excellent</td>
<td>26.3</td>
</tr>
<tr>
<td>Very good</td>
<td>40.9</td>
</tr>
<tr>
<td>Good</td>
<td>22.2</td>
</tr>
<tr>
<td>Fair</td>
<td>10.1</td>
</tr>
<tr>
<td>Poor</td>
<td>0.5</td>
</tr>
</tbody>
</table>

- **Experience with Public Hospitals**

Forty percent (81) of the respondents reported that they or a family member had been a patient in a public hospital in the past 12 months. Of those, forty nine percent had been a patient in a public hospital at least once. The median number of times was three and the range was one to 50. The person who reported 50 times had a family member with cancer and reported that the patient had been very unwell in the last year. Figure 6.1 illustrates the number of times respondents (or family members) had been a patient in a public hospital in the last 12 months.
Figure 6.1 Number of times respondents (or family members) had been in a public hospital as a patient in the last 12 months (n=81).

Respondents were asked to think about their most recent visit to a public hospital as a patient and they could offer more than one response. Some respondents had difficulty understanding the terms inpatient and outpatient. When needed, examples were provided to clarify. The most frequent visit to a public hospital as a patient was to an emergency department (36%) (Table 6.3)

Since questions two, three and four did not limit the question to the respondent (you or any of your family members), comparisons with available Australian data can not be made directly. However in the 2003 NSW Health Survey, 13.5% of the respondents reported that they had spent at least one night in the hospital and 13.5% had been in hospital emergency department (total 27%) in the last 12
months. (372) As expected percentages were higher in this study sample given that the questions included “you or any of your family members”.

Table 6.3 The most recent visit to a public hospital as a patient.

<table>
<thead>
<tr>
<th>Hospital Visit</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Emergency Department</td>
<td>36.0</td>
</tr>
<tr>
<td>Outpatient</td>
<td>30.0</td>
</tr>
<tr>
<td>Inpatient</td>
<td>29.0</td>
</tr>
<tr>
<td>All of the above</td>
<td>2.5</td>
</tr>
<tr>
<td>Inpatient and Emergency Department</td>
<td>1.3</td>
</tr>
<tr>
<td>Outpatient and Emergency Department</td>
<td>1.3</td>
</tr>
</tbody>
</table>

6.6.3 Knowledge about access to HCMs in public hospitals

The summary of responses regarding knowledge about access to HCMs in public hospitals is presented in Table 6.4. Eight percent of the respondents believed public hospitals had unlimited resources for HCMs. Forty per cent thought the Commonwealth Government funded public hospitals directly. Seventy nine percent were aware that permanent residents have the right to public hospital treatment at no charge to the patient. Twenty eight percent thought there was no difference between public and private hospitals with respect to access to HCMs. Thirty percent considered public hospitals may restrict HCMs by supplying them only to people from the hospital’s area and 29% believed public hospitals provided medications regardless of cost.
**Table 6.4 Summary of responses about knowledge**

<table>
<thead>
<tr>
<th>Question</th>
<th>True</th>
<th>False</th>
<th>Don’t know#</th>
<th>Missing*</th>
</tr>
</thead>
<tbody>
<tr>
<td>Public hospitals have unlimited resources for high cost medications.</td>
<td>8.00%</td>
<td>90.5%</td>
<td>0.5%</td>
<td>1.0%</td>
</tr>
<tr>
<td>The Commonwealth Government funds public hospitals directly.</td>
<td>40.0%</td>
<td>57.0%</td>
<td>1.0%</td>
<td>2.0%</td>
</tr>
<tr>
<td>All Australian permanent residents have the right to public hospital treatment at no charge.</td>
<td>79.0%</td>
<td>20.0%</td>
<td>0.5%</td>
<td>0.5%</td>
</tr>
<tr>
<td>There is no difference between public and private hospitals when it comes to access to high cost medications.</td>
<td>27.5%</td>
<td>68%</td>
<td>1.5%</td>
<td>3.0%</td>
</tr>
<tr>
<td>Public hospitals may restrict high cost medications by supplying them only to people from the hospital’s area.</td>
<td>29.5%</td>
<td>63.5%</td>
<td>3.5%</td>
<td>3.5%</td>
</tr>
<tr>
<td>Public hospitals provide medications regardless of their cost.</td>
<td>29.0%</td>
<td>71.0%</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

#Although this was not a given option, people wrote don’t know next to the answer.

*People who left the answer blank and stated they didn’t know the answer.

**Correct answer has been bolded**

Most people commented that they were unsure of the correctness of their answers.

Typical comments from the respondents were:

“I'm not a 100% sure of my statements”. Respondent #172

“These are my perceptions of the truth based on recent but limited experience as an end user of hospital services”. Respondent #71

“I’m not 100% confident of my knowledge here, last time as a public patient was 52 years ago”. Respondent #131
“My knowledge of such details is limited. I do know for sure that hospital funding is often in the news”. Respondent #133

“My perception is that public hospitals are funded both by state and federal government”. Respondent #161

To determine if there was a difference in knowledge regarding public hospitals and access to HCMs between respondents who had been in a public hospital (as a patient or with a family member) and those who had not attended a public hospital in the last 12 months, proportions were compared using the chi-squared ($\chi^2$) test.

Results showed that there was no statistically significant difference between these two groups except for question seven “All Australian permanent residents have the right to public hospital treatment at no charge”. Respondents who had been in a public hospital (as a patient or with a family member) in the past 12 months were more likely to give the correct answer (true) to this question (see Table 6.5).
Table 6.5 Association between, attending a public hospital in the past 12 months and obtaining the right answer.

<table>
<thead>
<tr>
<th>Question</th>
<th>Yes</th>
<th>No</th>
<th>$\chi^2$†</th>
<th>p Value</th>
</tr>
</thead>
<tbody>
<tr>
<td>You (or any family member) have been a patient in a public hospital in the past 12 months</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>n</td>
<td>%</td>
<td>n</td>
<td>%</td>
<td></td>
</tr>
<tr>
<td>Public hospitals have unlimited resources for high cost medications.</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>True</td>
<td>4</td>
<td>12</td>
<td>10.1</td>
<td>0.930</td>
</tr>
<tr>
<td>False</td>
<td>74</td>
<td>107</td>
<td>89.9</td>
<td></td>
</tr>
<tr>
<td>The Commonwealth Government funds public hospitals directly.</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>True</td>
<td>27</td>
<td>53</td>
<td>46.1</td>
<td>2.741</td>
</tr>
<tr>
<td>False</td>
<td>52</td>
<td>62</td>
<td>53.9</td>
<td></td>
</tr>
<tr>
<td>All Australian permanent residents have the right to public hospital treatment at no charge.</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>True</td>
<td>69</td>
<td>89</td>
<td>74.2</td>
<td>5.992</td>
</tr>
<tr>
<td>False</td>
<td>9</td>
<td>31</td>
<td>25.8</td>
<td></td>
</tr>
<tr>
<td>There is no difference between public and private hospitals when it comes to access to high cost medications.</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>True</td>
<td>27</td>
<td>28</td>
<td>24.3</td>
<td>2.789</td>
</tr>
<tr>
<td>False</td>
<td>49</td>
<td>87</td>
<td>75.7</td>
<td></td>
</tr>
<tr>
<td>Public hospitals may restrict high cost medications by supplying them only to people from the hospital’s area.</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>True</td>
<td>26</td>
<td>33</td>
<td>30.3</td>
<td>0.254</td>
</tr>
<tr>
<td>False</td>
<td>51</td>
<td>76</td>
<td>69.7</td>
<td></td>
</tr>
<tr>
<td>Public hospitals provide medications regardless of their cost.</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>True</td>
<td>21</td>
<td>37</td>
<td>30.6</td>
<td>0.371</td>
</tr>
<tr>
<td>False</td>
<td>58</td>
<td>64</td>
<td>69.4</td>
<td></td>
</tr>
</tbody>
</table>

* $p<0.05$
† $df=1$
6.6.4 Factors & choices

- Factors

Respondents were asked to rank factors from one to four, with one the most important factor. The most important factors that respondents thought should be considered when deciding who should receive a HCM have been listed in Table 6.6. The most frequent response was "treatment outcomes" (35%). This was followed by current health status (26%). Quality of life was third with 15%, and the fourth place was for life expectancy and age.

**Table 6.6 The most important factors considered when deciding who should receive a high cost medication.**

<table>
<thead>
<tr>
<th>What factors are most important in deciding who should get high cost medications?</th>
<th>Percentage</th>
<th>Rank Order</th>
</tr>
</thead>
<tbody>
<tr>
<td>Treatment outcomes</td>
<td>34.7</td>
<td>1</td>
</tr>
<tr>
<td>Current health status</td>
<td>25.5</td>
<td>2</td>
</tr>
<tr>
<td>Quality of life</td>
<td>14.8</td>
<td>3</td>
</tr>
<tr>
<td>Life expectancy</td>
<td>9.2</td>
<td>4</td>
</tr>
<tr>
<td>Age</td>
<td>9.2</td>
<td>4</td>
</tr>
<tr>
<td>Socioeconomic status</td>
<td>4.6</td>
<td>6</td>
</tr>
<tr>
<td>Family commitments</td>
<td>1.0</td>
<td>7</td>
</tr>
<tr>
<td>Lifestyle</td>
<td>0.5</td>
<td>8</td>
</tr>
<tr>
<td>Other</td>
<td>0.5</td>
<td>8</td>
</tr>
</tbody>
</table>

“Other” represents one respondent who wrote: “those individuals who have dedicated most to elevate humanity for example like Ghandi”.

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• **Scenario One**

In the first hypothetical scenario respondents had a limited budget for medications in a public hospital. They were asked to provide a basis for choosing between two patients with exactly the same characteristics. Eighty percent of respondents favoured a choice based on ability to benefit quality and length of life. This result is consistent with responses to the previous question where respondents rated quality of life and length of life as two of the factors that should be considered when allocating resources to HCMs. Table 6.7 show how respondents decided who should receive the treatment.

**Table 6.7 Who should receive treatment – Scenario One**

<table>
<thead>
<tr>
<th></th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>There is a limited budget for medications in a public hospital.</strong> Two patients need to be treated with a high cost medication for cancer but there is only enough money to treat one of them. How do you think the hospital should choose which one receives the treatment?</td>
<td></td>
</tr>
<tr>
<td>One of them randomly</td>
<td>5.5</td>
</tr>
<tr>
<td>The one who benefits the most in terms of quality and length of life</td>
<td>79.5</td>
</tr>
<tr>
<td>The youngest</td>
<td>1.5</td>
</tr>
<tr>
<td>The one whose work contributes more to society</td>
<td>1.5</td>
</tr>
<tr>
<td>The one who has more family members to support</td>
<td>4.5</td>
</tr>
<tr>
<td>Don’t know</td>
<td>5.0</td>
</tr>
<tr>
<td>Other</td>
<td>2.5</td>
</tr>
</tbody>
</table>

A small proportion of respondents 2.5% (6) provided different answers, these included:

“Every one should be entitled to medications and treatment no matter what the circumstances”. Respondent #69
“Couldn’t choose – they both should get the treatment”. Respondent 116

“Combination of all of the above except for one of them randomly”. Respondent #152

“Why do we have a limited budget?” Respondent #174

“Other: A mix of factors weighted – there is no one answer”. Respondent #179

- **Scenario Two**

In the second hypothetical scenario respondents had a limited pool of money and they had to spend it on two treatments: Medication A, to prevent heart attacks or Medication B to improve the quality of life of a person with cancer and lengthen that person’s life. The most frequent response (66%) as shown in Table 6.8 was “Spend some money on Medication A for 500 patients and some of the money on Medication B for 5 patients”. Eleven percent allocated all the resources to Medication A and five percent to Medication B. Fifteen percent decided to allocate more resources to Medication A compared to B. Table 6.8 shows how people decided to spend the limited pool of money.
Table 6.8 Distribution of resources for medications A and B – Scenario Two

<table>
<thead>
<tr>
<th></th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>There is a limited pool of money to be</td>
<td></td>
</tr>
<tr>
<td>spent on medications. Medication A costs</td>
<td></td>
</tr>
<tr>
<td>$40 dollars per patient per month and</td>
<td></td>
</tr>
<tr>
<td>could prevent heart attacks. Medication</td>
<td></td>
</tr>
<tr>
<td>B costs $4,000 per patient per month and</td>
<td></td>
</tr>
<tr>
<td>could improve the quality of life of a</td>
<td></td>
</tr>
<tr>
<td>patient with cancer and lengthen that</td>
<td></td>
</tr>
<tr>
<td>person’s life. What would you do?</td>
<td></td>
</tr>
</tbody>
</table>

| Spend all the money on Medication A       | 11.0       |
| Spend all the money on Medication B       | 5.0        |
| Spend some money on Medication A for 500  | 66.0       |
| patients and some of the money on Medication B for 5 patients | |
| Spend some money on Medication A for 800  | 15.0       |
| patients and some of the money on Medication B for 2 patients | |
| Other                                    | 3.0        |

The “other” answer (3%) was given by six respondents. Two respondents wanted more information or thought the information provided was insufficient to make the decision.

“Don’t know – not enough information”. Respondent#186

“Cannot answer as too little information”. Respondent#96

Another respondent stated that:

“Every one should be entitled to medication treatment no matter what the circumstances”. Respondent #69

The remaining three respondents thought money should be spent in the following ways:
“50% each”. Respondent #47

“Spend all of the money on patients as needed until it is gone then stop treatment with both drugs”. Respondent #71

“Would depend on demand (no. of patients for each condition)”. Respondent #115

Of the 200 respondents who completed the questionnaire, 117 (58.5%) wrote a response to the open ended question to Scenario 2 – What are your reasons? The majority of the respondents perceived this was the most “equitable” distribution of resources. The terms “fairness” and “solidarity” were also expressed in the respondent’s comments. The following were typical examples:

“One needs to bear in mind “the common good ie balance benefits = ethical decisions”. Respondent #11

“All people have the right to life. Equal opportunities to live”. Respondent #12

“Society has a responsibility for all its members. After all, it is empathy and compassion that really separates the civilised from the uncivilised. However a cost/benefit analysis must also be made”. Respondent #30

Those who decided to spend more money on Medication A expressed that with limited resources this would benefit more people. Some also expressed that they would rather spend money on Medication A to “save lives” rather than B that only “prolongs life”. 
“The more patients that can benefit from the limited resource the better”. Respondent #14

“Ultimately you must save as many lives as possible but some exception should be made for the more expensive meds/Improve quality of life”. Respondent #29

“If a medication costs $4000 they need to find an alternative way or a different medication. Is the drug really worth someone’s hard earned money? I might change my mind 20 years from now”. Respondent #32

“Public health resources need to be allocated in a way that attempts to meet the health problems of a large group”. Respondent #38

“Can help more people, possibly save lives. Whereas cancer drugs are merely prolonging life”. Respondent #60

“Cardiovascular disease is a bigger problem than cancer more prevalence, effects more people. Scenario suggests that person/people with cancer are not going to be cured”. Respondent#132

Respondents who decided to allocate more resources to treatment B expressed a view that heart attacks could be prevented with life style changes instead of using medications.

“To my understanding people have no choice when they are struck down with cancer. Heart attacks can be mostly attributed to poor life choices, and mostly at a later stage in life”. Respondent #48
"Life style, diet, exercise etc could alter/improve group A chances of not having heart attack whereas group B already have a life threatening condition". Respondent #93

"Prevention is more than medication. Lifestyle factors are more important than drugs in this scenario". Respondent #171

Some respondents also stated that everybody should have access to these medications and choices should not be made.

"Everyone should have some access to all medications". Respondent#82

“All patients with different treatment needs have the right to receive the right treatment". Respondent #113

Some objected with comments such as:

“Couldn’t choose-they both should get the treatment”. Respondent #116

“(I) don’t imply agreement with a system that would force such decisions. Politicians need to be accountable for their management of health policy and compromises that flow on from their decisions on resource allocation”. Respondent#145

“I believe it would be unethical to deny all public patients access to high cost medications. Distribution should be to those who benefit the most in terms of efficacy of the medication”. Respondent#162

“It doesn’t matter which answer is correct, the hospital should have enough money to supply both drugs”. Respondent #176
• **Who should decide?**

Respondents were asked who should decide who has access to HCMs in public hospitals. Forty six percent of respondents answered that decisions regarding access to HCMs in public hospitals should be made by hospital doctors. Results are presented in Table 6.9.

**Table 6.9 Who should decide who has access to HCMs in public hospitals?**

<table>
<thead>
<tr>
<th>Who should decide</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hospital doctors</td>
<td>45.5%</td>
</tr>
<tr>
<td>Other (specify)</td>
<td>19.5%</td>
</tr>
<tr>
<td>Managers of health services</td>
<td>17.0%</td>
</tr>
<tr>
<td>Don't know</td>
<td>7.0%</td>
</tr>
<tr>
<td>Pharmacists</td>
<td>3.5%</td>
</tr>
<tr>
<td>General Public</td>
<td>3.0%</td>
</tr>
<tr>
<td>Patient's</td>
<td>2.5%</td>
</tr>
<tr>
<td>Politicians</td>
<td>1.0%</td>
</tr>
<tr>
<td>Patients family</td>
<td>0.5%</td>
</tr>
<tr>
<td>No response</td>
<td>0.5%</td>
</tr>
</tbody>
</table>

“Other” was chosen by 20% (39) of the respondents. Of those, 10% (19) stated that the decision should be made by a committee composed by all of the above. Three percent (5) said all of the above except for politicians. Three percent (5) also mentioned an ethics committee, a bioethicist and/or ethicist plus other health professionals. Two percent (4) mentioned a committee made up only by health professionals. The remaining (6) included responses like: “God”, “the Geniocrats”, “all except perhaps family members”, “a small group of specialised people within the hospital” and “everyone should be entitled to medication”.

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To determine if there was an association between the respondents’ characteristics and their support for hospital doctors, proportions were compared using the chi-squared ($\chi^2$) test. There was no association between respondents’ characteristics and selecting hospital doctors as the decision-maker (see Table 6.10).

### Table 6.10 Association between respondents’ characteristics and their selection of hospital doctors as the decision-maker.

<table>
<thead>
<tr>
<th>Characteristic</th>
<th>Hospital Doctors</th>
<th>Other</th>
<th>$\chi^2$ *</th>
<th>p Value</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>n</td>
<td>%</td>
<td>n</td>
<td>%</td>
</tr>
<tr>
<td><strong>Gender</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Female</td>
<td>48</td>
<td>52.7</td>
<td>55</td>
<td>58.5</td>
</tr>
<tr>
<td>Male</td>
<td>43</td>
<td>47.3</td>
<td>39</td>
<td>41.5</td>
</tr>
<tr>
<td><strong>Income</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>&lt;30,000 - 49,999</td>
<td>26</td>
<td>30.2</td>
<td>26</td>
<td>30.2</td>
</tr>
<tr>
<td>50,000 &gt;</td>
<td>60</td>
<td>69.8</td>
<td>60</td>
<td>69.8</td>
</tr>
<tr>
<td><strong>Economic Activity</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Employed</td>
<td>82</td>
<td>90.1</td>
<td>80</td>
<td>85.1</td>
</tr>
<tr>
<td>Unemployed</td>
<td>9</td>
<td>9.90</td>
<td>14</td>
<td>14.9</td>
</tr>
<tr>
<td><strong>Level of Education</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>High school</td>
<td>18</td>
<td>19.8</td>
<td>10</td>
<td>10.6</td>
</tr>
<tr>
<td>Certificate &gt;</td>
<td>73</td>
<td>80.2</td>
<td>84</td>
<td>89.4</td>
</tr>
<tr>
<td><strong>Health Status</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Good health</td>
<td>81</td>
<td>89.0</td>
<td>85</td>
<td>92.4</td>
</tr>
<tr>
<td>Poor health</td>
<td>10</td>
<td>11.0</td>
<td>7</td>
<td>7.60</td>
</tr>
<tr>
<td><strong>Been in the hospital</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Yes</td>
<td>38</td>
<td>41.8</td>
<td>36</td>
<td>38.3</td>
</tr>
<tr>
<td>No</td>
<td>53</td>
<td>58.2</td>
<td>58</td>
<td>61.7</td>
</tr>
</tbody>
</table>

*df = 1
• **Involvement in decision making**

Fifty percent of the respondents said they would not like to be involved in decisions regarding access to HCMs in public hospitals (Figure 6.3).

![Figure 6.2 Willingness to be involved in decisions regarding access to HCMs in public hospitals.](chart)

Gender, income, economic activity, level of education and health status were not associated with respondents’ involvement in decision making (see Table 6.11).

• **Paying Tax**

Forty four percent of the respondents said they were willing to pay more taxes to subsidise access to HCMs in public hospitals. Some of the respondents expressed that they would do so if taxes were distributed in a better way. Comments included:

> "More taxes only if money was well spent". Respondent# 41
“I would like our current taxes to be better used”. Respondent #57

“The heart of the problem is how the total tax money is spent. A great deal is not used for the common good. More should be spent on health”. Respondent #138

“Not pay more taxes but instead 1) That money could be used to increase hospital funding 2) more tax money spent in general for public hospitals instead of defence”. Respondent #161

Table 6.11 Association between respondents' characteristics and willingness to be involved in decision-making regarding access to HCMs in public hospitals.

<table>
<thead>
<tr>
<th>Characteristic</th>
<th>Willingness to be involved</th>
<th></th>
<th></th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Yes</td>
<td>No</td>
<td>$\chi^2$</td>
<td>p Value</td>
<td></td>
</tr>
<tr>
<td></td>
<td>n</td>
<td>%</td>
<td>n</td>
<td>%</td>
<td></td>
</tr>
<tr>
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There was no association between respondents’ characteristics and their willingness to pay more taxes to subsidise access to HCMs in public hospitals (see Table 6.12).

Table 6.12 Association between willingness to pay more tax and demographic characteristics.

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*df=1*
6.7 Discussion

6.7.1 Knowledge

In this study, almost one third (29%) of the respondents believed public hospitals provide medications regardless of their cost. The survey has also shown that a similar proportion of people (28%) believe there are no differences between private and public hospitals when it comes to access to HCMs. Both these answers are contrary to current practice and indicate a lack of knowledge of the health system. Almost half of the respondents thought the Commonwealth Government funds public hospitals directly, another incorrect assumption. Most respondents noted they were unsure of their answers.

Having been (or family member) a patient in a public hospital in the last 12 months did not influence knowledge regarding public hospitals and access to HCMs. However those respondents who had been a patient in a public hospital (or had family members) in the past 12 months were more likely to know that Australian residents have access to public hospital treatment at no charge. It is acknowledged that the format of the questions might have felt like a test. Perhaps making the response a five point Likert scale from “strongly disagree to strongly agree with an “I don’t know response” would have make it less threatening.

There are no published data specifically about knowledge of the community about access to HCMs in public hospitals. Given the cohort of the community sampled and the location of this research in Sydney, it is acknowledged that the people in rural and remote areas, Indigenous, low income groups with low levels of education, unable to communicate in English are not represented in this sample. It
is unknown if their knowledge about access to HCM in public hospitals would differ. It might be speculated that other members of the community may have less access to information about health system funding and implications for the patients and the community. However it is important to note that, despite the fact that the sample was composed by highly educated people (defined by university education), they were relatively poorly informed about the details of funding of hospitals and HCMs in private and public hospitals.

In a health care system that several authors have described as confusing, these results are not surprising. (94, 373) For example Rodger, a physician who worked in Australia for several years, described it as a “complex, blurry-edged, two level system”. (373)

6.7.2 Factors and choices

- Factors
Respondents considered treatment outcomes, current health status, quality of life, age and life expectancy as important factors in deciding who should have access to HCMs. Similar results have been found elsewhere. These results refer to individual circumstances where “benefit to patient” and quality of life are considered factors to take into account in the allocation of resources. (196) In the United Kingdom (UK) a survey regarding health care rationing showed that most respondents agreed or strongly agreed that patients’ quality of life should be considered. (199)
Even though it did not rate highly (4th), age was chosen as a factor to determine access to HCMs by 9% of the respondents. Age as a factor to allocate resources has been debated extensively. (219, 285-287, 289)

In this study some respondents asked for an explanation of “socioeconomic status” indicating that the term “socioeconomic” was not well understood by all. Only five percent of the respondents considered socioeconomic status as a factor leading to a ranking of fifth. This meant that for this 5%, people with a lower socioeconomic status should be given priority to access HCMs.

Even though factors were identified there was no attempt to make a quantitative trade-off between them. Conjoint analysis could be a useful way to evaluate tradeoffs between factors. The potential value of this methodology will be addressed in Chapter Seven.

- **Scenario one**
  In scenario one, the majority of respondents (80%) considered that the person who benefits the most in terms of quality and length of life should have access to a HCM. The 1998 Eurobarometer survey, which was intended to monitor public opinion in the European Union, had five questions which related to rationing and priority setting issues. One of the questions gave participants a hypothetical scenario. “There is only one place in the hospital to treat two patients with chronic health disease. Both patients are the same age, support similar families, have similar jobs and the same heart condition”. Participants were asked how they thought the hospital should choose which one to treat. Consistent with the results
of our survey, the European response was “whoever can benefit most in terms of quality and length of life”. (196) This result is also consistent with the factors chosen in the previous question. Length of life and quality of life were ranked as important factors to consider.

One of the limitations of scenario one was that length of life and quality of life were not given as separate factors. Therefore it was not possible to determine the importance respondents may have given to prolonged life versus improving quality of life or the trade-off between both of them. However there was a need in constructing the questionnaire to strike a balance between simplicity and completeness. Respondents already had five options to choose from.

- **Scenario two**
  In scenario two, most respondents allocated resources according to what they considered would benefit the greatest number of people. Respondents stated that they tried to divide the limited amount of money equally across disease states. The concept of fairness applied by respondents meant that both groups of patients would benefit from a treatment. For those who gave priority to Medication A over B, prevention was regarded as important. Medication A was favoured on the grounds that Medication B was not going to provide a cure. Those who decided to choose Medication B over A stated that medication is not the only way people can prevent heart attacks. Respondents mentioned that a healthy life style may also prevent heart attacks. These results were similar to those of Alexander’s study, previously described in Chapter Two. In that study, participants chose services that would benefit the greatest number of people. Respondents also considered that money
should be invested in services which diagnose illness and provide early treatment. Services which aimed to prolong individuals' life had a lower ranking. (210)

For Williams, those people who benefit most from the resources available should be the ones that receive priority. The values of the community as a whole must override the values of a particular interest group within it. (287)

- **Who should decide?**

A high proportion of respondents (46%) in this study viewed hospital doctors as the most appropriate group to make decisions regarding access to HCMs in public hospitals. A Swedish study involving patients with rheumatoid arthritis showed that respondents considered that physicians should have the responsibility to determine who should have access to HCMs. (77) Surveys in the UK regarding public opinion about health care rationing have also shown that members the general public see doctors, especially hospital doctors, as the most appropriate group to make decisions regarding the allocation of resources. (196, 374, 375)

In this study members of the general public also considered that other people should be involved in the decision, most often described as a committee with hospital doctors, managers, pharmacist, members of the public, patient and patient’s family. This perhaps indicates that some members of the general public favour a multidisciplinary approach with decision-makers with a range of expertise.

Half of the respondents (50%) did not want to be engaged in decisions regarding access to HCMs in public hospitals. This result is consistent with previous studies.
In a study conducted by Litvia, the role of the general public was seen as limited regarding decisions concerning choices between patients. Participants were asked if they wanted to be involved in the decisions about which patient gets a medication within a limited budget. The majority of the participants were unwilling to have any role in these types of decisions. As previously described in Chapter Two, patients in Wiseman’s study were also less willing to be involved in decisions regarding “specific medical procedures”. According to Litva, the willingness of the public to participate is based on where the priorities are being set (e.g., macro, meso, micro). It might be concluded that the general public is less willing to participate in the allocation of resources at the institutional or patient level. However, they could be involved as expressed by Litva, in assisting to set criteria for the decision-makers.

• Paying Tax

Almost half of the respondents (44%) were willing to pay more taxes to subsidise access to HCMs in public hospitals. Consistent with this result, in 2003 a public opinion poll showed that 46% of the respondents indicated strong agreement to increased taxes, or redirection of spending from other areas to enable access to the latest medicines, medical devices and clinical treatments. This same poll also reported the general public regarded hospitals and health care services as the most important areas for government funding. Hays had previously found that the general public supported increased expenditure on health despite the awareness this might result in increased tax payment.

A frequent comment by respondents was that the questions about factors and scenarios were difficult to answer. The scenarios were also criticised by
respondents who believed this should never occur. Some people did not want to make a choice and perceived that choices should not be made. This suggests that some respondents were unaware of the extent to which rationing decisions are currently made. This is consistent with the perceptions of decision-makers, discussed in Chapters Four and Five, who believe that the general public is not aware that rationing is happening and has insufficient knowledge about this. Some authors have previously noted that health care rationing is not explicit in Australia. (117, 338, 349)

King, in the UK, contended that a debate is necessary for greater awareness among all citizens of issues involved in rationing and priority setting. (375) The Australian public have been less exposed to issues of rationing and priority setting and therefore might have less familiarity with the issues.

6.7.3 Demographics

Surveys of members of the Australian community about priority setting in health care have been conducted previously. (195, 208) The sociodemographic characteristics of the respondents in those studies appear similar to the respondents in this study. Both Wiseman and Nord also reported that, compared to census data, respondents were more likely to have private health care insurance. (195, 208). The differences between the respondents in our study and the census descriptions are noted. Furthermore it is acknowledged that people who were illiterate or had difficulty reading or were visually impaired and those who spoke languages different than English were excluded from this study. Even though the sample may not be representative of the Australian population at-large, the results
do provide an insight into the views and knowledge of the community about access to HCMs in public hospitals.

Baume states that the general public has insufficient knowledge to play a useful role in making decisions about hospital or medical resource allocation. (378) However, it is the community that bears the cost of health care allocation decisions as taxes pay for these resources. (378) Several authors have suggested that the values of the community may be different from those of politicians, health care providers and health care professionals. (132, 182, 200, 378) From this perspective it is necessary to inform the public about how the health care system is funded in Australia, including the mechanisms for access to medicines, and to seek the considered views of at least a representative sample of community members. Smith et al considered that an informed consumer sector has the capacity to influence government and add transparency to national committees. (312)

Those who support the involvement of the general public in decision-making priority setting consider that this would increase local accountability and decision-makers should be accountable to the actual and potential consumers. Decisions should be made more explicit and open. (301) For Lofgren it is no longer acceptable for decisions regarding access to pharmaceuticals in Australia at the macro level to be taken “behind closed doors”. (379) Lindblad, in Sweden, considered that involving patient perceptions in the priority setting process for HCMs at the micro level might facilitate the implementation in practice. (77)
Comparing data from surveys can be challenging. Differences in study design, sampling, questionnaire wording and analyses sometimes make it difficult to compare results across studies regarding allocation of resources in health care. (196, 375, 380) It is difficult to interpret results from questions which are not identical. Caution should therefore be taken when comparing results. The majority of the published surveys have explored views of the general public regarding the allocation of resources in health care. This survey looked at the views of the general public regarding access to HCMs in public hospitals. In a review of studies involving citizens in rationing, Mossialos expressed that “there is considerable ambiguity” regarding “very expensive” high tech treatments. Also data derived from surveys sometimes differs from that derived from focus group discussions. (196)

One of the limitations of surveys, as previously stated, is that the wording of questions and the way they are presented influences responses. (106, 187, 380) Finally, surveys such as the one undertaken for this study, could be criticised for not allowing respondents to deliberate due to the limited amount of time and information. (381) Bowling wrote that it is important to provide the public with the necessary information to make informed judgements. (198) Mooney suggests respondents need time to reflect and deliberate. (200) Nevertheless despite their limitations, surveys allow a wider cross section of the general public to be approached. (196) They also form one body of evidence that can be supplemented by other methodologies such as focus groups and interviews. (374)

This study was conducted to gather information about knowledge and views of members of the general public about access to HCMs in public hospitals. This information addresses a gap in the knowledge needed to make informed decisions
about the allocation of resources to HCMs in public hospitals. Health care decision-makers make choices about patients and/or treatments in resource constrained environments. However, there is a lack of adequate guidance for those decisions based on community views. Mooney wrote that “health care spending decisions made within budgetary constraints are not generally made in close consultation with the community”. (200)

From these findings it is clear that almost half the respondents did not want direct involvement in decision-making, however 38% do. This could mean that decision-making processes could directly include interested members of the community. Alternatively these findings offer support for the development of a process to involve community members in discussion on policy on the provision of treatment and services within health care institutions and specifically, seeking the views of members of the public on the provision of HCMs and expensive services within public hospitals. Members of the public could be involved in developing broader principles or criteria which decision-makers could follow rather than in direct decision-making.
7. Summary and future directions

“Would you please tell me, please, which way I ought to go from here?” asked Alice. “That depends a good deal on where you want to get to”, said the cat.

Lewis Carroll (1865), Alice's Adventures in Wonderland.

This chapter discusses the relevance and significance of the research projects described in this thesis and the future direction of research in this area.

Despite studies conducted elsewhere describing the decision-making processes for allocation of health care resources to medications at the institutional level, (141-143, 226) literature shows that data from Australia are limited. (60, 137, 161) The research reported in this thesis includes a description of the decision-making process for access to HCMs in public hospitals and explores the perceptions, concerns and attitudes of health care decision-makers and members of the general public about access to HCMs in public hospitals.

The field was first explored in a public hospital where there was a perception that individual patient use (IPU) requests and approvals for HCMs had increased over the years and associated costs had escalated. Two research questions arose: 1) had the number of requests and approvals increased over time? and 2) was the cost of the approvals consuming greater percentage of the medication expenditure than in the past?
To answer these questions the study reported in Chapter Three documented and described the IPU Scheme for HCMs in a public hospital. This study led to an understanding of the IPU scheme for HCMs being used at the time. It also revealed that the number of approvals and submissions for IPUs for HCMs had a trend towards increasing. The IPU scheme also had a significant financial impact on hospital medication expenditure and on the staff time involved in the administration of this scheme. Subsequent to this review, a new policy and procedure for managing requests for HCMs for IPU was established. A high cost drug subcommittee (HCD-SC), operating under the auspices of the Drug and Therapeutics Committee (DTC) was created to act as a forum for priority setting and decision-making.

The newly formed HCD-SC provided a valuable opportunity to explore how decisions were being made regarding access to HCMs in a public hospital. The case study reported in Chapter Four aimed to describe the operations of the HCD-SC and explore the perceptions, concerns and attitudes of the HCD-SC members regarding access to HCMs in public hospitals. Some lessons emerged from the description of the operations of the HCD-SC. Decisions were not solely based on effectiveness and cost. Additional factors such as “clinical need” and the lack of an alternative treatment were involved in decisions about access to HCMs. Members of the HCD-SC also considered it was important to have consistency in the way decisions were being made.

The evaluation of the HCD-SC process allowed identification of good practices which included the involvement of physicians in the decision-making process and the existence of an appeals mechanism. Gaps were also identified and considered
as opportunities for improvement. Lack of transparency and potential inconsistency in the process were considered to be areas for improvement. However, as Martin points out, change is not brought about by describing and evaluating, but by implementing strategies to close the identified gaps. (113) Further research could include “action research” to improve the decision-making process.

Designed to bridge the gap between theory, research and practice, action research focuses on generating solutions to practical problems. (382) Action research involves local participants and the researcher in developing and implementing strategies for improving the decision-making processes. (113, 382) It is been suggested that by involving the decision-makers in the process, the strategies are more likely to be implemented. (325)

The findings from this study also provide an evidence base for developing strategies to improve this hospital's decision-making process regarding access to HCMs. The process shown here used case study methods to describe the decision-making process and evaluation was conducted through using "accountability for reasonableness". This is a generalisable process that could be used for improving the fairness of priority setting in hospitals more widely.

To explore a broader perspective, a cohort of health care decision-makers in the South East Sydney Area Health Service (SESAHS) was approached. This in-depth study reported in Chapter Five, explored the perceptions, concerns and attitudes of health care decision-makers in an Area Health Service. The concerns expressed by these decision-makers were consistent with those previously expressed by the
members of the HCD-SC. In both studies decision-makers acknowledged that the rationales for the decisions were not available to anyone other than the decision-makers. These studies established that decision-makers wanted an explicit, systematic process to allocate resources to HCMs. Inconsistency and lack of transparency in the process lead to what participants referred to as “inequity of access to HCMs”.

Consistent with findings reported in Chapter Four, the in-depth interview results also showed that decision-makers used different kinds of evidence and different factors influenced the decision-making process. This study provided some understanding of why these decision-makers did not utilise pharmacoeconomic evaluations at the public hospital level. Future studies could explore decision-makers knowledge and training in pharmacoeconomic evaluation and how this methodology could be incorporated more consistently as a tool to aid decision-making at the public hospital level.

These studies also identified tensions between funding systems and hospital decision-making. According to participants there were no mechanisms in place to systematically capture, analyse and share the lessons learned between the macro level (ie. Federal, PBS) and the meso level (ie. Institution, public hospital) regarding funding for HCMs. Furthermore, decision-makers considered there are strong incentives for cost-shifting. For decision-makers there were inequities in access to HCMs between public and private hospitals. Participants in both studies considered that public hospitals provided access to HCMs where no other mechanisms exist.
Decision-makers in this study considered that rationing does occur in Australia. However, rationing is not explicit. Participants considered there should be a public debate to raise awareness about the fact that health care resources are scarce and choices need to be made. How much are people prepared to spend on medicines as opposed to other areas of health care, or non-health care costs? This is a question expressed by several authors. (201, 289, 355, 357) The study reported in Chapter Five also raised several questions regarding the role of members of the public in the decision-making process about health care resources.

To explore these issues, members of the general public were surveyed regarding their knowledge and views about access to HCMs in public hospitals. The results of this survey, reported in Chapter Six, were both convergent and divergent with the findings from the interviews with decision-makers.

One of the points of convergence between the general public and decision-makers was that both groups considered factors such as treatment outcomes, quality of life, and current health status when determining who should have access to HCMs. Both groups also wanted resources to be allocated to provide the “greatest benefit to the greatest number of people”. For decision-makers, public involvement is necessary but only possible at the macro level of decision-making. Fifty percent of the respondents said they did not want to be involved in decisions regarding access to HCMs in public hospitals. As stated by Mooney, “citizens may accept their limitations in some areas of decision-making, while insisting on their right to decide in others”. (200)
Decision-makers expressed the view that members of the general public had insufficient knowledge regarding funding for and access to HCMs in public hospitals. Our study showed that the surveyed respondents were relatively poorly informed about the details of funding of hospitals and HCMs in private and public hospitals. Systems for funding and knowledge of the precise methods by which HCMs are funded, and who pays (ie Federal or State budgets) might be seen as a technical detail which the community would expect the sector to manage.

One point of divergence between the general public and decision-makers was willingness to pay more taxes. Decision-makers considered that the general public may not be willing to pay more taxes to access HCMs. Contrary to this expectation, the survey results showed that the majority of the respondents from the general public were willing to pay more taxes for this purpose. However they also expressed the view that they wanted taxes to be distributed in a “better way”.

The factors (listed above) described by the general public could be further explored by using conjoint analysis. This analysis could allow evaluation of the tradeoffs between factors. Originally developed as a market research tool, conjoint analysis is a survey technique that allows determination of the extent to which various characteristics of a service or product determine its overall demand. (187, 202)

Furthermore analysis of the members of the general public survey could be done through qualitative methods such as focus groups and/or semi-structured interviews. Previous research has shown that when participants are given time to deliberate, their decisions change. (360, 381) The studies conducted by Cookson
and Dolan suggest that an evaluation of uninformed views could be compared with views expressed after information is provided (informed). This would be consistent with the argument that deliberative methods should be used in research regarding health care priority setting and that people should be given the opportunity to reflect and deliberate. (200, 360, 381)

As already stated, decisions about spending on medicines in general, not only HCMs, occur at different levels in Australia. There has been little systematic study in these settings. Knowledge of actual practice is important to advance understanding of how decisions are being made, especially with the increasing demand for evidence-based policy making. (383, 384)

The case study presented in this thesis (Chapter Four), described practices within a specific context and these findings could contribute to knowledge about how decisions are made more generally as well as to policy development. In particular, the studies described in Chapters Four and Five described the evidence and criteria decision-makers actually used. A further step could be what Martin described as “cross institutional learning”, in which institutions share good practices and develop strategies from lessons learned. (346)

The work undertaken in this thesis provides a better understanding about how decisions are made in public hospitals regarding access to HCMs. It also provides an understanding of the perceptions, concerns and attitudes of health care decision-makers and members of the general public about the allocation of resources to HCMs in public hospitals. These data could allow development of
informed policies and procedures to improve the transparency and consistency in
the decision-making process in the future.
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Appendix 4.1


Decision 1. Temozolomide in Acute Myeloid Leukaemia as second line therapy. Individual request (IPU) for two patients.

A Phase I study was presented. Results showed that nine of the 20 patients treated with temazolomide for relapse/refractory acute leukaemia had a significant decrease in bone marrow blasts. The medication was thought to be less cardiotoxic than alternative available treatment. The medication was an oral treatment.

Due to concerns about the potential number of eligible patients that could be treated, the committee decided to give approval for use of temazolamide in five patients in a given year for up to two courses of therapy each. A report of outcomes was required to be submitted to the HCD-SC before further courses would be provided. The cost of temozolamide was approximately A$5,000 per patient per year compared to approximately A$3,500 for traditional therapy.

Decision 2. Linezolid for MRSA (Methicillin-Resistant Staphylococcus aureus). (IPU)

Treatment with linezolid was requested for a 72-year-old patient who developed MRSA infection of hip prosthesis. The request was for linezolid 600 mg twice a day for six weeks. Previous therapy included vancomycin for three weeks plus rifampicin and fusidic acid for three weeks. Rifampicin was thought to be associated
with a severe skin rash. After consultation with the head of Division of Microbiology, approval was given for the patient to receive linezolid (cost A$15,170). The Committee considered this was the only option for the patient. It was considered appropriate that the patient should have rifampicin desensitisation in the future.

**Decision 3. Infliximab for severe refractory Crohn’s disease.**

The DTC committee had previously added Infliximab to the SVH formulary for refractory severe Crohn’s ileocolitis and fistulizing Crohn’s disease with the following conditions:

- Only one authorised prescriber and an Individual Patient Use Request form was required.
- A multidisciplinary team was going to be responsible for each approval (Dr. XX, Prof YY, a surgeon and a pharmacist)
- Criteria needed to be developed.
- The number of doses and regimen required for maintenance had to be specified before the application would be considered.
- A maximum of A$50,000 was allocated to this program in one year.

Treatment was requested for two new patients (case 1 and 2) and one ongoing (case 3). The Unit had A$50,000 allocated for this program in a given year. The unit had already spent A$46,000.
Case 1. Eighteen year old patient with severe refractory Crohn’s disease, on steroids and azathioprine, with active and difficult to control disease. His family decided to pay for infliximab and he received his first dose in December 2002 with good initial response. Physician wrote “His family is likely to continue scraping money together for the maintenance doses but it is obviously a big financial impost. I appreciate that the high cost drug committee is in an invidious position but is there any possibilities of supporting this man and his family?”

Case 2. Thirty six year old HIV patient with refractory fistulising perianal Crohn’s disease. Physician wrote “Apart from infliximab therapy the only other option is diverting stoma. There is little data re infliximab in HIV patients. The goals of the treatment are to stop fistulae discharge and recurrent perianal sepsis, allow better qol (Quality of life), avert need for diverting stoma and return to work in unbroken [sic] fashion.“

Case 3. Patient started treatment a year ago and physician reported positive outcome with treatment. “Brilliant response, needing only two doses in 12 months.”

After two meetings considering these cases the committee decided to approve ongoing supply for case three. Treatment for cases one and two were approved if infliximab supply was within the approved A$50,000 per year budget assigned to the program for Crohn’s disease.
**Decision 4.** Infliximab for Rheumatoid Arthritis (RA). (IPU)

It was previously agreed that the hospital could no longer provide infliximab for ambulatory arthritis patients. The physician wrote in his application that this patient was in a desperate situation. “We have the means to break the downward spiral and with care and proper support we could salvage this patient to the benefit not only to himself but society” This patient had severe RA of the hands and feet and hepatitis C related to his past history of heroin addiction, which he had overcome. However his hepatic function had deteriorated due to alcohol abuse and he could not continue treatment with methotrexate. The physician wanted to break the pain cycle by using infliximab, allowing the liver to recover and the patient to cease drinking. “according to xxxx and with support, we could get him off alcohol and back on methotrexate” (physician). After discussion the committee reaffirmed the general policy and was strongly opposed to providing ongoing supplies of infliximab to RA patients. However due to the nature of the request “to break the pain cycle” three injections of infliximab were offered and it was determined that, regardless of the response, no further supplies would be made available. The patient and physician signed a consent form stating the terms of approval.

**Decision 5.** Darbepoetin alfa in severe anaemia secondary to HIV infection. (IPU)

Two applications for use of darbepoetin alfa for severe anaemia secondary to HIV infection were reviewed. Both patients had received blood transfusions and had failed their retroviral therapy. It was proposed (by the physicians) that treatment would provide better outcomes compared to blood transfusion.
The maximum estimated cost per year in the worst case scenario was A$18,000 per patient per year. The subcommittee considered the cost of blood transfusion compared to treatment with darbepoetin alfa. If more patients required this treatment it was going to be a huge burden for the hospital. It was decided that the HIV unit should incur the cost of treatment for these two patients. A three month report to the HCD-SC reporting outcomes of these two patients was required. Costs were to be monitored.

**Decision 6. Sildenafil in pulmonary hypertension. (IPU)**

The subcommittee decided to approve a four to six week trial to relief the symptoms and improve the patients’ quality of life until the patient undergoes pulmonary endarterectomy. However if symptoms do not improve treatment should be stopped. The patient needed to sign a consent stating the terms of approval. (No extra medication was going to be provided if symptoms did not improve)

**Decision 7. Sirolimus for prevention of renal transplant rejection.**

Evidence of efficacy came from two randomised, double blind, multicentre, controlled trials. The subcommittee approved its use and suggested a protocol should be developed and suggested that diltiazem could be used as a sirolimus sparing agent.

**Decision 8. Sirolimus for prevention of lung transplant rejection (IPU)**

Ongoing use was granted for patient xxx (IPU) on compassionate grounds. However since evidence of efficacy came from case series, the committee decided that sirolimus was not going to be approve for further patients. Due to the lack of
evidence of effectiveness (no head to head trials with sirolimus and other first line agents in lung transplant patients). The members thought that it was appropriate to require higher quality evidence before considering spending A$5110 per patient per year.

**Decision 9.** Sirolimus for prevention of heart transplant rejection in patients with failing renal function.

The committee felt sympathetic with the proposal to use sirolimus in heart transplant patients failing renal function, but felt that the issue of the promised savings (based on reduced number of patients coming to dialysis) needed to be explored further. The cost was A$60,000 per year.

**Decision 10.** Sirolimus for progression or development of transplant coronary artery disease (CAD)

The cost was A$153,300 per year for 20 patients. There was only anecdotal experience. In the absence of evidence the HCD-SC decided not to support funding for this particular indication. If the evidence can be produced, the issue would be revisited.

Further evidence was produced at a later stage. The committee considered the evidence and decided that the number of patients that could be treated at any one time with low dose sirolimus for prophylaxis against transplant related CAD could be increased to 20. Costs year 1 $51 k extra, year 2 $92 k, year 3 $130 k if all patients survive.
**Decision 11.** Bosentan in pulmonary hypertension.

The Therapeutics Goods Administration (TGA) approved Bosentan in pulmonary hypertension but the PBAC did not approve it for funding as an S-100 medication. The subcommittee recognised this was an effective medication. However it was a very expensive one, $40-50 000 per annum, and the hospital was not able to fund ongoing therapy. Therefore no new patients were to receive treatment with Bosentan. Since there were different groups of patients receiving it, through the programs stated in the results section. Consents were developed to cover all scenarios. “They should all clearly state ‘If I am not Medicare eligible and drug becomes unavailable on PBS I won’t be able to access and hospital may not be able to pay.’” It was important that patients understood the circumstances under which they were supplied this medication.

The first of March 2004 Bosentan was listed in the PBS under Section 100 for the treatment of pulmonary arterial hypertension (PAH).

**Decision 12.** Recombinant Factor VIIa in life threatening bleeding.

The evidence came from case reports and small series in non-haemophiliac patients with life threatening bleeding. Double-blind randomised control trial was conducted with patients with haemophilia. The subcommittee approved it with stringent controls, a protocol was developed and approval from haematology was needed for each patient before use.
Decision 13. Risperidone depot in schizophrenia

The data available was limited and it appeared to be as effective as oral Risperidone in controlling positive and negative symptoms. It had not been compared against conventional depot injection. Randomised control trials were needed to fully assess the effects of this new preparation. The subcommittee considered it could only be used for patients with no other alternatives; it was 90% more expensive than current phenothiazines. No extra budget was going to be made available for this medication, only two psychiatrists were able to authorise approval and a protocol needed to be developed.

Decision 14. Sildenafil and/or Iloprost in patients undergoing pulmonary endarterectomy.

Approximately 50% of patients undergoing the procedure might need some short/long term pulmonary vasodilatation treatment. Cost was estimated as A$10,000 per patient per year for sildenafil. The subcommittee decided that the physician such seek special state funding since this procedure was only performed in three centres around Australia.

Decision 15. Thalidomide in different indications

The Therapeutics Goods Administration (TGA) approved thalidomide as maintenance therapy for prevention and suppression of cutaneous manifestations of erythema nodosum leprosum (ENL) and recurrence and treatment of multiple melanoma after failure of standard therapies. Usage of thalidomide at SVH was reviewed and off label indications included graft versus host disease (GVDH) and Crohn’s disease. TGA approval required all patients to be registered with the
pharmaceutical company risk management program. Usage and cost were going to be monitored initially for three months and then again at six months. The HCD-SC expected that few patients would be prescribed this medication.
Appendix 4.2

Designation of levels of evidence

I  evidence obtained from a systematic review of all relevant randomised controlled trials.

II  evidence obtained from at least one properly designed randomised controlled trial.

III-1  evidence obtained from well-designed pseudo-randomised controlled trials (alternate allocation or some other method).

III-2  evidence obtained from comparative studies with concurrent controls and allocation not randomised (cohort studies), case control studies, or interrupted time series with a control group.

III-3  evidence obtained from comparative studies with historical control, two or more single-arm studies, or interrupted time series without a parallel control group.

IV  evidence obtained from case series, either post-test or pre-test and post-test.

Adapted from the NHMRC Guidelines (385)
Appendix 4.3

Terms of reference of the HCD Sub Committee of SVH Drug Committee

2002

Aim/Terms of Reference should include:

- To define criteria and a process for decision making re high cost drugs
- To set up a management process for handling these drugs
- To consider applications for high cost drugs and to advise the Drug Committee of recommendations and decisions.
- To set up a review process to ensure that drugs are not continued beyond the designated trial period unless positive outcomes of therapy are demonstrated.
- To consider the $s which should be allocated to High Cost drug therapies
- To consider strategies for controlling spending on high cost drugs
- To produce and distribute and Executive Bulletin re process for making applications to prescribe High Cost Drugs at SVH
- To consider Ethical questions re choosing one high cost drug over another
- To keep the Board informed re issues (Ethical and Financial) arising from marketing of new high cost drug therapies
- To work with NSW Therapeutic Assessment Group (TAG) to lobby appropriate funding programs for high cost drug therapies
Appendix 5.1

Invitation to participate in decision-makers study

Date

Person’s name
Position
Institution

Dear (salutation)

We are currently conducting a research project aimed to develop novel approaches for management of High Cost Medications in the public hospital setting.

As a first stage of the project we would like to explore views, perceptions, concerns, problems and solutions regarding access to high cost medications and the current approaches for management.

In order to do so we will be conducting interviews with individuals who have different roles within the South Eastern Sydney Area Health Service (SESAHS).

Therefore we would really appreciate it if we could have a 30-minute conversation that will be recorded with your consent. The data collected during the interview will be confidential. The Human Research Ethics committee of the University of Sydney has approved this project.

Your collaboration will be greatly appreciated and we thank you in anticipation for considering this request.

Yours sincerely

Gisselle Gallego
PhD Candidate
The University of Sydney.
Phone: 83822053
Email: giselle@pharm.usyd.edu.au

Enclosed

• Semi-structured interview.
Appendix 5.2

Reminder letter decision-makers’ study

Date

Person’s name
Position
Institution

Dear (salutation)

Re: Invitation to participate in the High Cost Medication (HCM) Research project.

This is a follow up to a letter sent to you in November, inviting your participation in a research project. The overall aim of the research is to develop novel approaches for management of High Cost Medications in the public hospital setting.

In this stage of the project we are exploring perceptions, concerns and attitudes regarding access to high cost medications among health care providers.

In order to do so we have been conducting interviews with individuals who have decision-making roles within the South Eastern Sydney Area Health Service (SESAHS). This project has the support of the SESAHS Area Chief Executive Officer, Ms Deborah Green.

Participation in the project involves a 30-minute conversation that will be recorded with your consent. The data collected during the interview will be confidential. The Human Research Ethics committee of the University of Sydney has approved this project.

We would be happy to answer any queries you have about any aspect of this research project. Gisselle will contact you in the next week to seek your involvement. Your assistance is greatly appreciated.

Yours sincerely

Gisselle Gallego
PhD Candidate
The University of Sydney
Phone: 83822053
Email: giselle@pharm.usyd.edu.au

Jo-anne Brien
Professor of Clinical Pharmacy
St. Vincent's Hospital
Appendix 5.3

Consent form

CONSENT FORM

Faculty of Pharmacy, University of Sydney
Tel: (02) 9351 5818, Fax: (02) 9351 4391

I, ……………………………………………………………………………………………………….. of ……………………………………………………………………………………………………….

hereby voluntarily consent to participate in the study entitled Re: “Access to High Cost Medications.”

This project is being conducted by the researcher Ms Gisselle Gallego under the supervision of Professor Jo-anne Brien from the Faculty of Pharmacy, University of Sydney.

I understand that any data collected for the purpose of this study will remain strictly confidential. The fact that I may be audiotaped during the interview has been explained to me. I have been informed that the information obtained from this research will be used in future research, and may also be published.

Details of this study have been clearly explained by the researcher. Any questions that I have had to date have been answered to my satisfaction. I am aware of the purpose of this project and what my involvement entails. I have read the Participant Information attached. I understand that my participation is entirely voluntary. I have been informed of my right to question any part of the procedure or withdraw from the project at any time.

Name: …………………………………………………………………………………………………..

Signature: ……………………………………………………………………………………………

Date: ………………………………………………………………………………………………..

Witness Name: ………………………………………………………………………………………

Signature: ……………………………………………………………………………………………

Date: ………………………………………………………………………………………………..
Appendix 5.4

Participant information sheet

Faculty of Pharmacy, University of Sydney
Tel: (02) 8382 2053, Fax: (02) 9351 4391

SUBJECT INFORMATION STATEMENT

Research Project

Title: “Access to High Cost Medications”

(1) What is the study about?

Currently the burden of high cost medications in public hospitals is borne by individual institutions. The future management of costs of pharmaceuticals may be influenced by current discussions in relation to reforms in the Australian political system and consideration of expansion of the role of the Pharmaceutical Benefit Scheme. In addition the roles of state-based and Area Health services in consideration of high cost pharmaceuticals may evolve. However, there is likely to remain a need for an efficient, evidence-based, transparent and accountable process to manage local decisions regarding high cost medications. This pilot project is planned to elucidate aspects of the process at the institutional/area/state/national level. We wish to explore your views, perceptions, concerns, problems and solutions regarding high cost medications and the current approaches for management. This may assist in the development of novel approaches for management of High Cost Medications.

(2) Who is carrying out the study?

The study is being carried out by the Faculty of Pharmacy, University of Sydney, under the supervision of Professor Jo-anne Brien.

(3) What does the study involve?

If you choose to be involved in this study, you will be invited to participate in a single interview with the researcher. During this interview your discussion will be taped recorded to help the researcher collate the results of this study, however data will be deidentified.
(4) **How much time will the study take?**

The interview will last up to 60 minutes

(5) **Can I withdraw from the study?**

Being in this study is completely voluntary - you are not under any obligation to consent.

(6) **Will anyone else know the results of my interview?**

The information obtained from this study may be published, and will be used to develop future research. All data collected will remain strictly confidential and all information will be de-identified.

(7) **Will the study benefit me?**

No direct benefits to you are likely to occur as a result of this study. However, information obtained through this research may improve access and equity to High Cost Medications.

(8) **Can I tell other people about the study?**

Yes

(9) **What if there’s a problem?**

If you require further information or have any other questions, please contact Professor Jo-anne Brien at the Therapeutics Centre St. Vincent’s Hospital on (02) 8382 2605

Any person with concerns or complaints about the conduct of a research study can contact the Manager for the Ethics Administration, University of Sydney on (02) 9351 4811
Appendix 5.5

In-depth Interview Study - Interview Guide

INTRODUCTION - background to study, purpose of research, what happens during/after interview

- Broad, open-ended questions

High Cost Medications

1. What do you think are the problems with HCMs? (Access)
2. Do you deal with High Cost Medications at your hospital? Could you give me some specific examples?
3. How were decisions made in each of those cases?
4. What do you think of this approach?

Current problems and limitations - at your hospital/in your experience

5. Does the Drug and Therapeutic Committee (DTC) have the total responsibility of dealing with these High Cost Medications? Would you give me more information on how these decisions are made and on who takes responsibility?
6. Do you think the decision-making process for approval of High Cost Medications in public hospitals in your experience is an explicit and transparent one? If no, What problems do you encounter? What are your concerns?
7. If you were personally in the role of a decision-maker regarding approvals for HCMs what would be the main difficulties/challenges/concerns for you?
8. What do you perceive are the main problems for the hospital/institution in approving a high cost medication for public hospital use?

Solutions

9. Do you have any suggestions or possible solutions to the problems that you have indicated?
10. Have you any mechanisms or processes that you could suggest for the prescriber/institution/area health service/state/nationally to address the problems you’ve been talking about and any other problems you are aware of?
11. Do you think there should be criteria? If so what are would you suggest as important points? How would you rank these? why?
12. Should outcomes be tracked and evaluated for HCMs differently to other drugs/expensive services?
13. What is the role for Economic Evaluation?
Costs

14. What are your perceptions about the costs if these HCMs?
15. If the same amount were being spent for a large number of patients would that amount still be a problem?
16. Do you have any comments on how these medications could be subsidised / funded?

Public

18. What do you think could be the role of the public when it comes to HCMs?

That is the last question, is there anything that you would care to add?
Do you have any questions for me?
Would you like to receive a summary of findings?
Thank you for your time today. I appreciate you giving up your time to talk to me....

NOTES:
Appendix 5.6

Example of Transcript File

Subject:

Topic: Perceptions, concerns, and attitudes of decision-makers

Date:

Place:

Time:

Relevant Information:

Position:

Professional Background/education:

Gender:

Age:

Special Circumstances:
Appendix 6.1

Questionnaire used in general public survey

Questionnaire for research study:
Access and Equity for High Cost Medications – Public Perspectives.

The purpose of this questionnaire is to explore your views about access to high cost medications in public hospitals. The survey is expected to take approximately 15 minutes to complete. Your privacy whilst participating in this study will be maintained at all times. The information you provide in this survey will be identifiable via numerical code only. If you have any questions regarding this survey, please contact Gisselle Gallego on 8382 2053 or Prof Jo-anne Brien on 8382 2605.

1. How would you rate your current state of health?
   Please tick one box
   - Excellent
   - Very good
   - Good
   - Fair
   - Poor

2. In the past 12 months have you or any of your family members been a patient in a public hospital?
   Please tick one box
   - Yes
   - No  [ ] Go to question 5

3. How many times have you or any members of your family been a patient in a public hospital in the last 12 months? __________

4. Thinking of your most recent hospital visit, were you or your family member:
   - an inpatient?
   - an outpatient?
   - an emergency room patient?
We would like to know how much you know about paying for high cost medications in public hospitals.

The following statements are true or false. Please tick one box

5. Public hospitals have unlimited resources for high cost medications.
   - True  - False

   - True  - False

7. All Australian permanent residents have the right to public hospital treatment at no charge.
   - True  - False

8. There is no difference between public and private hospitals when it comes to access to high cost medications.
   - True  - False

9. Public hospitals may restrict high cost medications by supplying them only to people from the hospital’s area.
   - True  - False

10. Public hospitals provide medications regardless of their cost.
    - True  - False

Comments: (if any)
_________________________________________________________________________
_________________________________________________________________________
_________________________________________________________________________
_________________________________________________________________________
In this section you will be given choices about allocating resources to high cost medications.

11. What factors are the most important in deciding who should get a high cost medication? 
   Draw a line between the numbers on the left (1 being the most important) and the four most important factors on the right.

<p>| | |</p>
<table>
<thead>
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<tbody>
<tr>
<td>1</td>
<td>Socioeconomic status</td>
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<td>2</td>
<td>Current health status</td>
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<tr>
<td>3</td>
<td>Life expectancy</td>
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<td>4</td>
<td>Quality of Life</td>
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<td>Family commitments</td>
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<td>6</td>
<td>Lifestyle</td>
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<td>7</td>
<td>Treatment outcomes</td>
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<td>8</td>
<td>Other (specify)</td>
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</table>

The following are some hypothetical examples of the types of decisions that need to be made

Scenario 1

12. There is a limited budget for medications in a public hospital. Two patients need to be treated with a high cost medication for cancer but there is only enough money to treat one of them. How do you think the hospital should choose which one receives the treatment?
   by choosing

   [Please tick one box only]:
   - one of them randomly
   - the one who would benefit most in terms of quality and length of life
   - the youngest
   - the one whose work contributes more to society
   - the one who has more family members to support
   - don’t know

Scenario 2

13. There is a limited pool of money to be spent on medications. Medication A costs $40 dollars per patient per month and could prevent heart attacks. Medication B costs $4,000 per patient per month and could improve the quality of life of a patient with cancer and lengthen that person’s life.

What would you do?

Please tick one box only

- Spend all the money on Medication A for 1000 patients
- Spend all the money on Medication B for 10 patients.
- Spend some of the money on Medication A for 500 patients and some of the money on Medication B for 5 patients.
- Spend some of the money on Medication A for 800 patients and some of the money on Medication B for 2 patients.
What are your reasons? :
_________________________________________________________________________
_________________________________________________________________________
_________________________________________________________________________

14. If decisions need to be made about who has access to high cost medications in public hospitals, who should make these decisions?

   Please tick one box only
   
   ❑ Hospital doctors  ❑ Nurses  ❑ Pharmacists  ❑ Managers of health services  ❑ Politicians  ❑ Patients  ❑ Patient’s family  ❑ General Public  ❑ Other (specify) ________________  ❑ Don’t know

15. Would you like to be involved in decisions regarding access to high cost medications in public hospitals?

   Please tick one box
   
   ❑ Yes  ❑ No  ❑ Don’t know

16. Would you be willing to pay more taxes to subsidize access to high cost medications in public hospitals?

   Please tick one box only
   
   ❑ Yes  ❑ No  ❑ Don’t know
We would like you to answer some questions about yourself.

17. What is your age? _________ years

18. What is your sex?
   Please tick one box
   
   ☐ Male
   ☐ Female

19. What is your marital status?
   Please tick one box

   ☐ Single
   ☐ Married
   ☐ Divorced
   ☐ Widowed

20. What is the main language you speak at home?
   Please tick one box only

   ☐ English
   ☐ Vietnamese
   ☐ Cantonese/Mandarin
   ☐ Arabic
   ☐ Italian
   ☐ Greek
   ☐ Other ______________

21. What is your economic activity?
   Please tick one box

   ☐ Working for money (full or part time)
   ☐ Not in paid employment

22. What is the highest level of schooling you have had?
   Please tick one box

   ☐ Never attended school
   ☐ Primary school
   ☐ Intermediate or school certificate
   ☐ Leaving or higher school certificate
   ☐ Certificate or diploma
   ☐ Currently at university
   ☐ University degree
   ☐ Other (please specify): ______________

23. What is your postcode?

   ___ ___ ___ ___
24. What is your combined annual household income?

Please tick one box

- < $30,000
- 30,000 – 50,000
- 50,000 – 75,000
- 75,000 – 100,000
- >100,000

25. Do you have private health insurance?

Please tick one box

- Yes
- No
PARTICIPANT INFORMATION STATEMENT

Title: “Access to High Cost Medications – Public perspectives”

(1) What is the study about?

The future management of costs of medications may be influenced by current discussions in relation to reforms in the Australian healthcare system. Health care practitioners are increasingly aware of pressures (and limitations) on funding for health care services, and specifically for high cost medications. A high cost medication has been defined as a medication that has a financial impact on the public hospital medication expenditure. Our research to date has focussed on the perceptions, attitudes and concerns of health care practitioners regarding equity of access to high cost medications. The views of the general public are less well understood. It is appropriate that the perspective of the broader community is sought to inform future programs regarding equity of access to high cost medications. Therefore we wish to explore your views regarding funding for high cost medications in public hospitals and the criteria used in decision making regarding access to these medications.

(2) Who is carrying out the study?

The study is being conducted by Gisselle Gallego and will form the basis for the degree of Doctor of Philosophy at the University of Sydney under the supervision of Professor Jo-anne Brien, Professor of Clinical Pharmacy.

(3) What does the study involve?

Faculty of Pharmacy, University of Sydney
Tel: (02) 8382 2053, Fax: (02) 9351 4391
If you choose to be involved in this study, you will be invited to fill out a survey. All data collected will be de-identified.

5. How much time will the study take?

Filling out the survey could take up to 30 minutes.

5. Can I withdraw from the study?

Being in this study is completely voluntary. If you agree to participate you can withdraw at any time.

6. Will anyone else know the results of my survey?

The information obtained from this study may be published, and will be used to develop future research. All data collected will remain strictly confidential and all information will be de-identified.

7. Will the study benefit me?

No direct benefits to you are likely to occur as a result of this study. However, information obtained through this research may improve access and equity to High Cost Medications.

8. Can I tell other people about the study?

Yes

9. What if there’s a problem?

If you require further information or have any other questions, please contact Professor Jo-anne Brien at the Therapeutics Centre St. Vincent’s Hospital on (02) 8382 2605.

Any person with concerns or complaints about the conduct of a research study can contact the Manager for the Ethics Administration, University of Sydney on (02) 9351 4811.