

Paying for (expensive) drugs in the statutory system: An overview of experiences in 13 countries

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Summary

Introduction

The advent of new and expensive drugs creates challenges for health systems about how to make these new treatments available under the statutory system given inevitably limited resources. While these treatments have the potential to be effective for some individuals, through, for example, extending a patient's life by months or even years, their overall cost-effectiveness may be questionable and health systems may decide not to pay for these under the statutory system. Yet, patients may still wish to access these drugs and be willing to pay out of pocket to have them.

We here provide a rapid review of how countries have addressed this issue. We present an overview of policies in 13 countries on the funding of licensed pharmaceuticals under the statutory system describing the process of decision-making used by the main actors (regulators/health authorities) involved in the system for reimbursement of pharmaceuticals. The countries reviewed in this report include 10 European countries (Denmark, Finland, France, Germany, Italy, the Netherlands, Norway, Spain, Sweden, and Switzerland), plus Australia, Canada and New Zealand. These countries are characterised by different types of health systems: (i) national health service systems, and (ii) health insurance systems (social or private).

The report has been informed by several key sources: (a) an iterative search of the published literature using bibliographic databases (PubMed and Web of Knowledge), of the world wide web using common search engines (Google, Yahoo), and of governmental and non-governmental agencies/organisations of the literature on general pharmaceutical policies in the countries in question; and (b) information provided by country informants in response to a detailed questionnaire (included in the Annex to this report).

The report is broadly in two parts. We begin with an overview of the key observations on pharmaceutical policies in different countries with a particular focus on policies on funding new and/or expensive pharmaceuticals under the statutory system. This is followed by a table summarising the main characteristics of pharmaceutical policies in 13 countries, including general principles of decision-making on new drugs under the statutory system; the use of positive and/or negative lists; policies on co-payments for pharmaceuticals; time between licensing and reimbursement-decisions; the role of cost-effectiveness criteria in decision-making; examples for drugs that have been rejected for funding under the statutory system along with some general information about the systems included in this review. Part 2 of the report provides detailed assessments of each of the 13 countries reviewed here.

Key findings

1. All 13 countries have established national bodies separate from the Ministry of Health which either have an advisory role (Australia, Canada, France and the Netherlands) or have a regulatory function and make decisions on behalf of the Ministry of Health (Denmark, Finland, Germany, Italy, New Zealand, Norway, Sweden, Spain and Switzerland) about the reimbursement of new drugs under the publicly funded/statutory health system. It is worthy of note that the Ministry of Health remains the final decision-maker in some countries.
2. Federal states, such as Canada or Germany, vary in how decisions on reimbursement are taken. Thus, in Germany, decisions are taken by a federal level committee with representatives of the main health system stakeholders (Federal Joint Committee) whose decisions are binding on all statutory health insurance funds once approved by the Federal Ministry of Health. In Canada, where each Province has ultimate responsibility for health care, the majority of the jurisdictions follow the recommendations of the Common Drug Review undertaken by the national expert committee CEDAC. However, Provinces are not required to follow these recommendations and Quebec makes its own decisions without reference to recommendations from CEDAC.
3. The typical process for deciding whether a new drug should be paid for as part of the statutory system of a country includes the Ministry of Health (or an arm's length body of the Ministry of Health responsible for drugs) approving the list or formulary, after they have received advice from a specialised scientific committee or separate body (usually this is part of a national medicines agency and/or an independent organisation).
4. Cost-effectiveness is an overt criterion in decision-making on the reimbursement under the statutory system of new drugs in Australia, Finland, the Netherlands, New Zealand, Norway, Sweden and Switzerland. However, decision-making does not always depend exclusively on the cost-effectiveness evidence. Other criteria, such as the therapeutic value, effectiveness and efficacy of the drug may play a more important role. In most other countries the evaluation of cost-effectiveness of a new drug is not yet a formal requirement, but it is increasingly used in decision-making (i.e. Denmark, France, Germany).
5. Time from licensing to regulatory approval for reimbursement under the statutory system varies. In Germany, for example, drugs are automatically eligible for reimbursement by the statutory health insurance funds as soon as they are licensed, while in France the time between market authorisation and reimbursement approval may take an average of 16 months.
6. New drugs have to be included in *positive lists* in Australia, Canada, Denmark, France, Finland, Italy, the Netherlands, New Zealand, Norway, Spain, Sweden and Switzerland. In contrast, in Germany, reimbursement is automatically granted once market approval has been obtained. However, Germany has introduced an explicit *negative list* for pharmaceuticals which are not eligible for reimbursement under the statutory system, such as inefficient drugs.
7. Several countries have made special arrangements for the reimbursement of expensive drugs under their positive list (e.g. Australia, Canada, the Netherlands, and France). Access to these drugs is granted on the basis of specific criteria as to who is eligible to receive treatment, how the treatment is to be funded, and

who is going to deliver and administer the treatment (for example Australia's Highly Specialised Drugs Program and the Special Authority Program).

8. The availability of new and in most cases expensive drugs (especially cancer drugs) under the statutory system has lately received considerable public and media attention in several countries. In addition, there have been cases where media attention has been sought by those lobbying for inclusion of a new drug in order to increase the pressure on the decision-making bodies to allow for the funding of new but expensive drugs (e.g. in the Netherlands). Competing private insurers in the Netherlands have on several occasions taken advantage of the media debate by including expensive drugs in their reimbursement schemes for marketing reasons.
9. In terms of specific drugs not being reimbursed by the statutory system, information is hard to obtain since few countries have transparent procedures. This point has previously been made in the Transparency Directive by the European Council (European Council 89/105/EEC). In brief, the European Council was concerned about the transparency of the methods used by the EU member states when determining the price and reimbursement level of pharmaceutical products under the statutory system, and indicated that both processes should not exceed 180 days. It further noted that when member states decide not to reimburse a specific pharmaceutical product under the statutory system, the process of coming to this decision should also be made transparent and the relevant authorities should be in a position to provide detailed information on the process to relevant actors and the public. The lack of access to this kind of information was noted by several key informants. To our knowledge, only Australia, Canada, New Zealand and the Netherlands provide accessible and transparent information about the decision-making process and the specific pharmaceuticals rejected from their positive lists as well as reasons for inclusions.

In conclusion, pharmaceutical policies in the 13 countries reviewed for this report vary considerably, largely reflecting countries' institutional, political, social and historical contexts, which determine the weight given to the views of the local pharmaceutical industry and more importantly how susceptible governments and other health system actors are to external pressures (media and general public opinion) in terms of their reimbursement decision-making processes. Tensions between authorities, whether governmental or non-governmental, responsible for reimbursement decisions and the pharmaceutical industry regarding reimbursement issues are seen in most countries.

Table 1 Decision-making related to the funding of new drugs under the statutory system in Australia, Canada, Denmark, Finland, France, Germany and Italy¹

	Australia	Canada	Denmark	Finland	France	Germany	Italy
Primary body responsible for assessing new (outpatient) drugs for funding/subsidy under the statutory system	Pharmaceutical Benefits Advisory Committee (PBAC)	Canadian Expert Drug Advisory Committee (CEDAC)	Danish Medicines Agency (DKMA)	Pharmaceutical Pricing Board (PPB)	Transparency Commission (CT) and Economic Committee for Health Care Products (CEPS) (both part of the High Authority for Health, HAS)	Federal Joint Committee (G-BA)	Technical Scientific Committee (CTS), which is part of AIFA (Italian Medicines Agency)
Summary of process	PBAC requires a value for money case for each new drug, which is then subject to assessment by HTA organisations contracted by PBAC. Decisions on drugs and devices for use in public hospitals are made by state governments with some having established advisory committees and working groups to assess requests to use new medicines in hospital settings.	CEDAC considers reviews received through the Common Drug Review (CDR) at the Canadian Agency for Drugs and Technology in Health (CADTH); it makes recommendations for listing of new drugs to participating federal/provincial/territorial drug plans (except Québec). Hospitals determine their formularies through their Pharmaceutical and Therapeutics Committee.	DKMA decides on information received by the Reimbursement Committee (within DKMA). The Danish Centre for Health Technology Assessment (DACEHTA) is responsible for assessments of new drugs (especially new cancer drugs). Hospitals have one or more Drug and Therapeutic Committees which determine the hospital's formulary lists.	The PPB is responsible for decisions on the reimbursement status of pharmaceuticals and for confirming wholesale prices. Hospitals decide independently on their drug formularies on the basis of therapeutic and economic effectiveness.	CT is responsible for reimbursement, CEPS for pricing; both produce technical advice to the Ministry of Health on new drugs. CT advice is on the level of actual clinical benefit and of improvement of clinical benefit; HAS may also make recommendations after assessing specific pharmaceuticals. Hospital pharmaceuticals are approved by the Ministry of Health.	Licensed prescription drugs are automatically covered (except drugs for trivial diseases, inefficient drugs & lifestyle drugs). G-BA receives advice from IQWiG (Institute for Quality and Efficiency in Health Care) which assesses the effectiveness of drugs and issues prescribing recommendations. Individual hospitals' commissions decide on the hospital formulary.	The Interministerial Committee for Economic Planning (CIPE) provides the CTS with eligibility criteria for the reimbursement of a new drug; the same criteria apply for in-patient drugs

¹ Information displayed in this table is derived from the country reports presented in this report and the following sources: Pharmaceutical pricing and reimbursement information, Country Profiles; Sorenson et al. (2008); Office of Fair Trading (2007).

	Australia	Canada	Denmark	Finland	France	Germany	Italy
Principal role of the assessing body: advisory or regulatory²	<u>Advisory</u> : PBAC makes recommendations to the Government	<u>Advisory</u> : CEDAC makes formulary listing recommendations for Canada's publicly funded plans	<u>Regulatory</u>	<u>Regulatory</u>	<u>Advisory</u> : Transparency Committee and CEPS report to the Ministry of Health	<u>Regulatory</u>	<u>Regulatory</u>
Positive and/or negative list	Positive list: Pharmaceutical Benefits Scheme (PBS)	Positive lists (formularies) for each Canadian jurisdiction	Positive list	Positive list	Positive list (separate lists of innovative drugs allowing for special funding arrangements)	Every licensed drug is covered under SHI except for lifestyle drugs and others ('negative' list)	Positive list
Arrangements for co-payment for pharmaceuticals: ambulatory/out-patient sector	General beneficiaries: AUD 31.30 Concessional beneficiaries: AUD 5.00	A variety of income-related deductibles and co-payments	i) General reimbursement for prescription-only medicines and over-the-counter drugs ii) According to 4 reimbursement rates: 50%, 75%, 85% and 100% of the retail price	Basic refund/ lower special refund: 58% & 28% co-payment. Higher special refund/ additional refund: EUR 3 and 1.5	Severe chronic disease/ serious disease/ moderately serious diseases: 0%/35%/65% Pending delisting: 85% co-payment Where reference pricing applies, a patient has to make additional payment for the difference between the retail price of the drug and its reference price	i) children <18 years: 0% ii) adults: 10% of drug price EUR 5–10 with upper limit dependent on income	i) Class A: essential drugs: 0% co-payment ii) Class C drugs for less important diseases: 100% co-payment
Arrangements for co-payment for pharmaceuticals: in-patient sector	No co-payment required	No co-payment required	No co-payment required	No co-payment required	No co-payment required	Co-payment/flat fee of EUR 10 /day of hospital stay for the first 28 days	No co-payment required

² Advisory bodies are defined as bodies that make reimbursement recommendations to a national/regional government, ministerial or self-governing body. Regulatory bodies are accountable to health ministries and responsible for listing drugs for reimbursement/subsidy under the statutory system (Sorenson et al. (2008)).

	Australia	Canada	Denmark	Finland	France	Germany	Italy
Time from licensing to regulatory approval for reimbursement under the statutory system	Around 6 months	Between 3 and 4 months (94 to 124 days)	Less than 3 months (longer if the Reimbursement Committee is consulted more than once)	6 months (180 days)	Around 16 months	Automatically reimbursed once licensed	Around 13 months
Is cost-effectiveness, an overt criterion for the decision whether to list a drug for reimbursement/subsidy under the public system?	<u>Yes</u> PBAC requires a 'value for money' case for each new drug. In addition, PBAC takes into account: the importance of the clinical area; the availability of alternative treatments; the likely effect of listing on the health system and other therapeutic activities; and the investment of the sponsor in primary research	<u>Yes</u> However, processes and rules for formulary listing differ among provinces and territories. Except for Québec, all Canadian jurisdictions consider CEDAC's recommendations for their decisions. Economic considerations range from simple budget impact analysis to more elaborate cost-effectiveness studies provided by the manufacturer	<u>No</u> A health economic analysis may be enclosed by the pharmaceutical companies to demonstrate to the Reimbursement Committee the cost-effectiveness of a new drug, but this is not mandatory. A health economic analysis as part of the reimbursement decision-making is only relevant for drugs that contain a new active agent.	<u>Yes</u> PPB's decision-making is based on the therapeutic benefit, patient benefit, cost-effectiveness and budget impact	<u>No</u> Decision-making is based on clinical, epidemiological data. In addition, financial and public health impact of the listing of a new drug is considered. Plans for using full economic evaluations as part of the decision-making have been announced in the 2008 and 2009 social security finance acts.	<u>No</u> Federal Joint Committee's decision is based on medical need and efficiency. The 2007 health care reform has mandated IQWiG to extend its assessments to also evaluate the cost-effectiveness of drugs (from 2008).	<u>No</u>
Examples of drugs that have been excluded from reimbursement/subsidy under the statutory system	Sunitinib (<i>Sutent</i>) for renal cancer Erlotinib (<i>Tarceva</i>) for NSCLC Cetuximab (<i>Erbitux</i>) for colorectal cancer Bevacizumab (<i>Avastin</i>) for colorectal cancer	Sunitinib (<i>Sutent</i>) for metastatic renal cell carcinoma	Not known	Citalopram (<i>Cipramil</i>) because of cheaper generic alternatives	Lifestyle drugs	Lifestyle drugs, e.g. Viagra	Ranibizumab (<i>Lucentis</i>)

	Australia	Canada	Denmark	Finland	France	Germany	Italy
Nature of funding of the health system (% of public funding)³	Federal taxes (68%)	Provincial, territorial and federal taxation (70%)	Central and local (municipal) taxes (84.1%)	Central and local (municipal) taxes plus national health insurance contributions (78%)	Compulsory contributions levied on earnings and income (74.9%)	Compulsory social health insurance contributions levied on earnings (67.4%)	Central and regional taxes (76.4%)
Total expenditure on health in % of GDP³	9.5	9.8	9.2	7.4	11	10.6	8.7
Total expenditure on pharmaceuticals (in % of total health expenditure)³	13.3	17.3	9.0	16.3	16.6	14	21.2
Public expenditure on pharmaceuticals (as % of total expenditure on pharmaceuticals)³	57.6	38.4	56.3	56	68.8	70.4	50.2

³ Data relating to year 2004; Source: OECD (2007). OECD Health Data 2007. Paris, OECD.

Table 2 Decision-making processes related to the funding of new drugs under the statutory system in the Netherlands, New Zealand, Norway, Spain, Sweden and Switzerland⁴

	The Netherlands	New Zealand	Norway	Spain	Sweden	Switzerland
Primary body responsible for assessing new (outpatient) drugs for funding/subsidy under the statutory system	Pharmaceutical Aid Committee (CFH) at the Health Care Insurance Board (CZV)	Pharmac Board	Norwegian Medicines Agency (NoMA)	Dirección General de Farmacia y Productos Sanitarios (DGF) at the Ministry of Health	Pharmaceutical Benefits Board (LFN)	Federal Office of Public Health (OFSP)
Summary of process	CFH assesses new drugs on the basis of their effectiveness, overall costs for health care and efficiency presented by manufacturers' application for reimbursement. Decisions on the reimbursement of expensive in-patient drugs within hospital budgets are made by the Dutch Health Care Authority (NZA).	Pharmac's decisions are informed by the Pharmacology and Therapeutics Committee (PTAC). Hospital Pharmaceuticals Assessment Committee informs Pharmac on in-patient drugs.	NoMA evaluates the pharmacoeconomy of new drugs; in cases of considerable budgetary impact of a new drug, the Ministry of Health/Parliament has the final decision. For hospitals the decision rests on specialists	The Director General of the DGF signs off the decision to fund or reject the public funding of a new drug. The same procedure applies for in-patient pharmaceuticals	LFN receives economic evaluations submitted by the pharmaceutical companies and decides on the in-/exclusion of drugs under the statutory system (decision applies to both outpatient and in-patient drugs)	A drug may be included in the positive lists if (1) it is licensed by Swissmedic, (2) it is effective & appropriate, and (3) it provides value-for-money. A drug is considered value-for-money when it produces a given therapeutic effect at the lowest cost.
Principal role of the assessing body: advisory or regulatory⁵	Advisory to the Ministry of Health	Regulatory	Regulatory	Regulatory	Regulatory	Regulatory
Positive and/or negative list	Positive list: Drug Reimbursement System (DRS)	Positive list (Pharmaceutical Schedule)	Positive list	Positive and negative lists	Positive list (Pharmaceutical Benefits Scheme)	Positive list

⁴ Information displayed in this table is derived from the country reports presented in this report and the following sources: Pharmaceutical pricing and reimbursement information, Country Profiles; Sorenson et al. (2008); Office of Fair Trading (2007).

⁵ Advisory bodies are defined as bodies that make reimbursement recommendations to a national/regional government, ministerial or self-governing body. Regulatory bodies are accountable to health ministries and responsible for listing drugs for reimbursement/subsidy under the statutory system (Sorenson et al. (2008)).

	The Netherlands	New Zealand	Norway	Spain	Sweden	Switzerland
Arrangements for co-payment for pharmaceuticals: ambulatory/out-patient sector	Prescription drugs are, for the most part, provided at no cost to patients	If prescribed by a Primary Health Organisation practitioner the maximum co-payment is NZD 3. The remainder have a max. of NZD 15 for 3-month's supply/ Lower levels apply, depending on age & income of patients	Schedule 4 – Drugs for serious contagious diseases: 0% co-payment Schedule 9, Schedule 2 & Schedule 10a – Drugs for specific conditions, rare diseases and others only for long-term treatment. For children under 12 years of age, low income pensioners and for patients who have reached the co-payment ceiling (EUR 205)	60% of the cost of a drug is reimbursed (Class N drugs). Some patients pay 10% for drugs used in the treatment of chronic disease (Class R drugs). Pensioners and those with special permission are exempted from co-payment. Non-reimbursable drugs: full costs.	i) Patient pays full price up to EUR 96.96 ii) Max. amount payable by the patient for 12 months is EUR 193.91	Insured persons pay a fixed annual amount (franchise) plus 10% of further costs (share). The sum of the franchise and the share is limited by a fixed maximum per year
Arrangements for co-payment for pharmaceuticals: in-patient sector	No co-payment required	No co-payment required	No co-payment required	No co-payment required (Class H drugs)	Co-payment/flat fee of EUR 8.62 for every day of hospital stay	A small contribution must be paid towards hospital costs in some cases
Time from licensing to regulatory approval for reimbursement under the statutory system	Around 6 months	Not known	Within 6 months (180 days)	Maximum 6 months as stipulated by Law (Ley 30/1992) (average of 115 days in 2007)	Decisions have to be made within 6 months (average of 91 days in 2006)	Less than 5 months
Is cost-effectiveness, an overt criterion for the decision whether to list a drug for reimbursement/subsidy under the public system?	<u>Yes</u> Criteria considered in decision-making include efficacy, safety, effectiveness, cost-effectiveness, financial impact, quality of life and social/ethical/legal considerations	<u>Yes</u> An additional 8 criteria are taken into consideration by Pharmac when deciding on a new drug	<u>Yes</u>	<u>No</u>	<u>Yes</u> Criteria considered in decision-making include the therapeutic benefit, the patient benefit, cost-effectiveness, the availability of therapeutic alternatives, and equity	<u>Yes</u> (as defined by the principle of 'value-for-money'). A new drug has to be effective appropriate and value for money in order to be included in the positive list. Effectiveness is the most important criterion

	The Netherlands	New Zealand	Norway	Spain	Sweden	Switzerland
Examples of drugs that have been excluded from reimbursement/subsidy under the statutory system	Cetuximab (<i>Erbtux</i>) for metastatic colorectal cancer Rotigotine (<i>Neupro</i>) for Parkinson's disease Erlotinib (<i>Tarceva</i>) for non-small cell lung cancer	Sunitinib (<i>Sutent</i>) for renal cancer Bevacizumab (<i>Avastin</i>) Erlotinib (<i>Tarceva</i>) for NSCLC Ranibizumab (<i>Lucentis</i>)	Not known	Lifestyle drugs	Not known	Not known
Nature of funding of the health system (% of public funding)⁶	General taxation, Statutory Insurance (Exceptional Medical Expenses Act), Statutory Insurance (Sickness Funds Act) (80%)	General budget for health care: Central taxes (78.1%) Accident Compensation Scheme (ACC): Contributions plus central taxes	General taxation (85.5%)	Central and regional taxes (66.5%)	Regional and local taxes (72%)	Mandatory health insurance, individuals' out of pocket payments, and State (federal, cantonal, regional) financing (16.2%)
Total expenditure on health in % of GDP⁶	9.2	8.5	9.7	8.1	9.1	11.5
Total expenditure on pharmaceuticals (in % of total health expenditure)⁶	11.5	12	9.4	23.2	12.5	10.4
Public expenditure on pharmaceuticals (as % of total expenditure on pharmaceuticals)⁶	57.2	64	59.2	72.8	69.8	67.2

⁶ Data relating to year 2004 (Netherlands: 2002); Source: OECD (2007). OECD Health Data 2007. Paris, OECD.

Country reports

Australia

Background

Responsibility for health care in Australia's federal system of government is divided between the national (Australian or Commonwealth) government and the eight states and territories. The Australian government subsidises patient medical services consultations and tests (ambulatory care by general practitioners and specialists) through the Medicare national health insurance scheme, public hospital services through Australian Health Care Agreements (five-year funding agreements) between the Australian government and the states (and including in-hospital medical care and pharmaceuticals), and pharmaceuticals dispensed by private community-based pharmacies through the Pharmaceutical Benefits Scheme (PBS). About 44% of the population take out private health insurance (additional to universal Medicare) to cover treatment as a private patient in hospitals and for extras such as dental care and physiotherapy (Private Health Insurance Administration Council, 2008).

The Pharmaceuticals Benefits Scheme (PBS)

The Pharmaceutical Benefits Scheme (PBS) is a major government programme with expenditure of around Australian dollar 5.7 billion in 2003-04, accounting for 11.6% of total recurrent health expenditure (Productivity Commission Steering Committee for the Review of Government Services Provision, 2006). PBS subsidies cover most of the costs of most prescribed drug purchases and all 'essential' drugs. The scheme subsidises the purchase of pharmaceuticals on its approved list for two groups: general beneficiaries, and concessional beneficiaries (holders of pensioner and other entitlement cards). General beneficiaries make a co-payment of AUD 31.30 on each prescription, and for concessional cardholders the co-payment is AUD 5 per prescription (at January 2008). The PBS sets the cost of pharmaceuticals for consumers (indexed on 1 January each year to movements in the Consumer Price Index). The scheme also includes a patient/family safety net to limit annual expenses on pharmaceuticals covered under the PBS (Department of Health and Ageing, 2008a).

Policies on funding of licensed pharmaceuticals under the statutory system

Before a medicine can be sold in Australia, it must be assessed for its safety, quality and efficacy by the Therapeutic Goods Administration (TGA) within the Australian Government Department of Health & Ageing. Once the TGA has approved the medicine for marketing, a submission can be made, by a drug company, to the Pharmaceutical Benefits Advisory Committee (PBAC) for listing on the PBS. A drug company can choose to market an approved drug with or without the PBS public subsidy (a practice endorsed in the Australia-United States Free Trade Agreement). The PBAC is a statutory independent expert committee established under the *National Health Act 1953* to advise the Minister for Health and Ageing on which medicines should be included on the Pharmaceutical Benefits Scheme and any conditions that should apply. Section 101(3) of the National Health Act (1953) requires that, when considering a proposal for listing on the PBS, the PBAC takes into account information on the comparative clinical effectiveness, safety and cost-effectiveness of the new product. A product that is more costly is generally only recommended for subsidy if it provides a significant improvement in effectiveness or reduction in toxicity. The Act does not prevent the PBAC from taking into account other factors in addition to cost-effectiveness, such as clinical need for the drug; level of uncertainty relating to costs and health outcomes; the total annual costs to the

PBS; and the likelihood of the drug being prescribed beyond any restriction for subsidy and the available methods of limiting this.

PBAC requires applicants to prepare a ‘value for money’ case. Detailed submissions providing evidence of effectiveness and cost-effectiveness are then subject to rigorous assessment by HTA organisations contracted to PBAC and provided as confidential reports. If a manufacturer seeks a higher price on the PBS schedule for a new drug, it has to prove that the new drug has a significant therapeutic advantage over currently listed drugs, that is, that the new drug is more cost-effective. Many drugs are placed on the PBS based on PBAC recommendations that restrict prescribing/subsidy to clinical criteria (indications, response to therapy within a specified period), or limited to specialty doctors. In effect, access to some subsidised drugs is limited to ‘approved’ patients judged most likely to benefit.

A recommendation by the PBAC for listing of a product, or extension of the terms of an existing listing, is then referred to the Pharmaceutical Benefits Pricing Authority (PBPA), a non-statutory body which advises the Minister for Health and Ageing on the pricing of pharmaceutical benefits supplied under the Pharmaceutical Benefits Scheme (PBS). The PBPA recommends the prices for new drugs and reviews the prices of drugs listed in the PBS schedule at least annually (Box 1).

Box 1 Pricing of pharmaceutical benefit supplied under the PBS

The main pricing method used by the Pharmaceutical Benefits Pricing Authority (PBPA) is reference pricing, whereby the price of medicine is determined by its relationship to either the price and/or the therapeutic benefit of another medicine. ‘Cost minimisation’ is the simplest form of reference pricing and is usually applied when the PBAC believes, on the basis of evidence put before it, that a drug provides a similar health outcome (therapeutic benefit) as another drug listed on the PBS (the comparator). The new drug will be linked by a ‘therapeutic relativity’ to the comparator, either joining an existing reference group or forming a new one. Cost minimisation ensures that the price per quantity of the new drug is no more than the price of a therapeutically equivalent quantity of the comparator. The price the Government pays for each drug in a reference group is set by the lowest price (‘the benchmark’) which has been secured for any drug in the group. The therapeutic relativity is based entirely on therapeutic benefit, thus the price of a drug which remains under patent may be linked to that of a drug for which the patent has expired and generic versions are available.

Where a proposed listing is expected to add AUD 10 million or more per annum to the cost of the PBS (a ‘high-cost medicine’ because of high unit cost and low utilization or low unit cost and high utilization), its subsidy will require approval by the Commonwealth Department of Finance and Administration, or by the Cabinet (Lu et al., 2008).

PBAC and PBPA recommendations are referred to the Minister for Health and Ageing for a decision. A positive recommendation by PBAC does not ensure listing, but a recommendation *not* to list a product requires legislative (not just Ministerial) intervention to be overturned. When proposing to de-list a product from the PBS, the Minister must seek advice from the PBAC, and that advice must be tabled in both Houses of Parliament, but the Minister is not obliged to accept that advice. The Health Minister and Parliament may reject an affirmative PBAC recommendation to list a new drug or to amend its coverage, but they may not add a new drug to the PBS that has not been endorsed by PBAC.

State governments also make decisions on the drugs and devices used in their public hospitals, and they are developing technology assessment capabilities and processes to regulate the introduction of new technologies. These often build on longer-standing ‘hospital formulary’ processes, and they usually rely on submissions prepared by public hospital physicians. They may or may not include consideration of cost-effectiveness. Some State governments have established advisory committees and working groups to assess requests to use new medicines or other medical technologies in hospital settings (Productivity Commission, 2005), where in the past, such introduction was decided entirely at the hospital level.

Paying for high-cost medicines

PBAC may recommend that very high-cost medicines (in terms of a high unit cost) be added to the schedule under Section 100 of the *National Health Act 1953* which includes, amongst others, the Highly Specialised Drugs Program and the Special Authority Program. These are funded by the Commonwealth government and only available through public and private hospitals when administered on an outpatient basis. Many cancer drugs fall into this category. PBS-subsidised access to high-cost medicines is targeted to patients with the highest capacity to benefit in line with PBS recommendations (Lu et al., 2008). The PBS does not formally define ‘high-cost medicines’, however, as noted above, if the cost is expected to exceed more than AUD 10 million a year its subsidy requires ‘whole-of-government’ approval. In the State of Victoria public hospitals have described high-cost medicines as those whose acquisition cost is greater than AUD 10,000 per patient per treatment course.

Biological medicines, also known as ‘targeted therapies’ can offer better quality or prolong life for some conditions and some patients. Doctors may prescribe these drugs once they are approved for marketing by the Therapeutic Goods Administration pending PBS approval (Box 2). The PBAC approval process may however take six months or longer and in the meantime some doctors and patients may use these drugs on the basis of results from clinical trials and information from drug companies. Cancer patients, for example, may be determined to try a promising and usually very expensive drug. Financial assistance for access to non-PBS listed drugs thus depends upon a hospital or clinic willing to cover the cost (and generally they wait for PBS listing), some private health insurance funds making time-limited ex-gratia payments, or else a patient may be enrolled in a clinical trial. Other patients meet the cost from their own pockets, while a few mount a public campaign for donations. For example two sons raised money to pay for the cancer drug bevacizumab (Avastin), which is not listed on the PBS, for the treatment of their mother for inflammatory breast cancer at a cost of AUD 4,000 (GBP 1,800; EUR 2,300) for each three-weekly treatment.⁷

Box 2 Accessing high Section 100 items

To prescribe drugs that are listed according to Section 100 (‘s100 drugs’) as PBS items, certain conditions have to be met: medical practitioners have to be affiliated with specialist hospital units and the patient must attend a participating hospital and be a day admitted patient, a non-admitted patient or a patient on discharge; be under appropriate specialist medical care; meet the specific medical criteria and be an Australian resident in Australia (or other eligible person). The patient is required to make a co-payment for each supply of a highly specialised drug at a similar rate to the Pharmaceutical Benefits Scheme (Department of Health and Ageing, 2008b).

⁷ <http://www.australiandoctor.com.au/news/d1/0c0540d1.asp>

In addition, access to some listed high-cost medicines under the PBS requires the presence of a severe active disease, of ‘molecular markers’ that predict a good treatment outcome, failure to respond to cheaper existing therapies, and the patient must sign a form to acknowledge that treatment will continue only if the pre-determined response criteria are achieved at follow-up assessment (for example 12 weeks for biological antirheumatic agents) (Lu et al., 2008).

An example of a high-cost medicine listed under Section 100/Special Authority Program is Trastuzumab (Herceptin) which the PBS in 2006 approved for women diagnosed with HER-2 positive early breast cancer who are receiving adjuvant chemotherapy, which previously could cost a patient AUD 50,000 per year. An example of a drug listed under the Highly Specialised Program is entecavir (Baraclude) for the treatment of chronic hepatitis B. imatinib mesylate (Glyvec) has been approved by PBAC to be PBS-listed for the treatment of some rare cancers, but some patients with other conditions pay over AUD 3000 per month for the drug. From 2007, women with breast cancer can access treatment with PBS-subsidised letrozole (Femara) once they have completed Tamoxifen treatment. Bevacizumab (Avastin) which inhibits the growth of bowel cancer has not yet been considered for PBS listing as its manufacturer (Roche) has not yet sought PBAC approval. One couple paying AUD 2750 a month for Avastin to supplement chemotherapy for ovarian cancer and secondary bowel cancer told a journalist in 2008: ‘We have chosen to go into our life savings but quite frankly it will cripple us’ (Remeikis, 2008).

Outside these arrangements, public hospital outpatients may have drugs dispensed at no charge through hospital pharmacies, rather than community pharmacies. There is no dedicated scheme to offset the costs to the States or to public hospitals, however, associated with the use of high-cost medications for either inpatients or outpatients for drugs not on the ‘Section 100’ schedule. Increasingly, state governments are seeking to shift the costs of such drugs dispensing onto Commonwealth schemes, and there is ongoing negotiation on the relative responsibilities of the two levels of government with regard to health.

Exclusions of licensed drugs from subsidy under the statutory system

Drugs licensed by the Therapeutic Goods Administration (TGA) are not all necessarily subsidised by the PBS if an equally effective drug is available at lower cost, or if their cost-effectiveness is not yet proven. In addition, access to some PBS drugs, particularly high-cost medicines, are restricted to patients with the greatest capacity to benefit.

In terms of a licensed but not subsidised drug, this becomes controversial when the Health Minister and Parliament rejects an affirmative PBAC recommendation to list a new drug or decides to amend its coverage. Rejections are rare, but one example is the PBAC recommendation to list Viagra, which was rejected by the Australian Government in 2002 because of cost concerns (Aroni et al., 2003).

Sponsors of a new product that receives a negative recommendation can resubmit later, usually with new data or a lower price. Appeals against the PBAC process (but not the decision) are permitted under the Administrative Decisions (Judicial Review) Act 1977 (Cth). De-listing of drugs or services is not routine and is likely to generate controversy. The Minister has recently created a review mechanism to deal with sponsors who wish to appeal negative PBAC recommendations.

A controversial example in relation to licensing is the abortive drug RU486 (Mifepristone) which in 2006 was removed from the TGA ‘restricted goods’ category.

Such drugs cannot be evaluated, registered, listed or imported without the written approval of the Minister for Health. Since 1996 this category had applied exclusively to medicines ‘intended for use in women as abortifacients’, after an amendment proposed by an influential independent senator to the Therapeutic Goods Amendment Bill, making the Health Minister rather than the TGA responsible for decisions to license these medicines (The Parliament of the Commonwealth of Australia, 2005).

The consequence for a patient of a PBS rejection, or of restricted PBS access, is that the patient usually must pay the full price of the drug. The political consequence from the rejection of a more newsworthy drug, such as Viagra, is considerable media attention.

Policies relating to patients’ ability to ‘top up’ their care under the statutory system

Australia has a long history of patient out-of-pocket co-payments for pharmaceuticals, and thus a tradition of ‘top up’ payments for drug treatments. In general, access to pharmaceutical drugs is tightly controlled. The drug must first be prescribed by an eligible medical practitioner. Patients can pay privately for a prescribed drug not listed on the PBS, or can choose to pay the mark-up for a brand name rather than a generic drug. If a pharmaceutical is listed on the PBS under several brand names, a pharmacist may dispense the least expensive drug unless specifically directed not to do so by the prescribing physician/practitioner. The PBS will only pay for a generic drug, or the least expensive product in a therapeutic class, and a patient must bear the additional costs for a more expensive brand name. The patient can elect to pay a brand premium for items in four therapeutic groups with equivalent prices: H2-receptor antagonists, calcium channel blockers, statins, and ACE inhibitors.

Further, in a mixed public and private health system, there is no regulatory constraint on a patient seeking both private and public treatment (ambulatory consultations and hospital inpatient care) for the treatment of one condition – if patients are prepared to meet the extra cost. For example, a patient may seek a ‘second opinion’ from another general practitioner, but would need a referral for a second specialist consultation. Most doctors, of course, would advise a patient that one doctor should manage their care. Patients frequently also use complementary/alternative treatments – most of which are not covered by Medicare. For example, the 2004-05 National Health Survey found that 3.7% of the adult population had consulted a complementary or alternative health professional in the previous two weeks, and that 46% used alternative treatments, such as vitamins, minerals and herbs (Australian Institute of Health and Welfare, 2006).

It is important to note that, in Australia, the ‘statutory system’ in treatment terms mostly refers to public sector hospitals. Public hospitals, however, treat private as well as public patients, and seek reimbursement from insurers for privately-insured patients treated. Public hospitals employ salaried doctors and most public hospitals also have arrangements with private visiting medical practitioners (surgeons and specialist physicians). General practitioners and most specialists are private practitioners.

A patient choosing to pay privately for a non-PBS listed but licensed drug will remain a patient within the statutory system for the treatment of that condition if the drug was prescribed by the treating doctor. The prescribing doctor will take clinical responsibility for the treatment of that patient, whether a salaried hospital doctor or a private practitioner. If this is community-based treatment the patient would claim a

partial rebate for the consultation from Medicare (or the doctor would accept the schedule fee and bulk-bill Medicare). Consultation claims do not have to be linked to prescribing/administering/monitoring of specific drugs, and doctors are not prohibited to administer drugs that have been legally obtained.

Canada

Background

Canada's health system is governed at federal, provincial/territorial and regional levels. Health care is a provincial responsibility, and the 13 single-payer, universal schemes (known as Medicare) covering health services in each province/territory as defined by the federal Canada Health Act (1984) are predominantly financed from general federal and provincial taxation. The provincial responsibilities cover management, organisation and delivery of hospital and physician services and may include supplementary coverage for other medical goods or services, including out-patient prescription drugs. The federal government, through its health department, Health Canada, transfers funds to the provinces to support the provision of health care on the condition that the provinces will adhere to the principles of the Canada Health Act (Paris and Docteur, 2007a). The federal government also funds and administers health services for specific groups, such as the armed forces, veterans, immigrants and registered First Nations people, and addresses national health issues by providing grants to the provinces or community groups and by funding health research (Ettelt et al., 2008).

Policies on funding of licensed pharmaceuticals under the statutory system

In Canada, drugs for in-patient care are universally covered through the publicly financed Medicare programme. In contrast, for most Canadians, access to out-patient prescription drugs, which are not generally included in the standard services guaranteed by the Canada Health Act, is secured through public and/or private insurance plans. The federal, provincial and territorial governments offer varying levels of coverage, with different eligibility requirements, premiums and deductibles. Two-thirds of the Canadian population rely on private insurance to cover prescription drugs. Private insurance functions as a supplement to Medicare; it is regulated by the ten provinces and three territories so that access to prescription drugs differs across Canada (Box 3).

Publicly funded drug plans (federal, provincial, territorial - F/P/T) define reimbursement prices for drugs under their formularies while private insurers are entitled to use their own formularies (a formulary represents the list of drugs that are reimbursed under a given public or private drug plan).

Box 3 Coverage of prescription drugs under private health plans

The main purchasers of prescription drugs are third-party payers, including privately and publicly financed health plans, patients and hospitals. Private health insurance plans reimburse plan members for the costs of the drugs they have used, and which are included in their plan's formulary. Coverage for prescription drugs under private health insurance tends to be more inclusive in terms of the numbers of products covered for reimbursement compared to the publicly financed programmes. Many private plans offer open access to all the drugs licensed for marketing by Health Canada, while others offer a more restrictive formulary. Some private plans have experimented with using the formularies provided by public drugs plans. Only Québec requires all private plans to offer coverage at least equal to the public formulary.

Until recently, Canada's federal, provincial and territorial drug plans had separate processes for undertaking reviews and making formulary listing recommendations.

As a result, rules for formulary listings tend to differ among provinces and territories, reflecting historical development and policy objectives.

In order to reduce duplication and ensure equal access to ‘high level evidence and advice’ (Canadian Agency for Drugs and Technologies in Health, 2008a) the Common Drug Review (CDR) was created in 2003 as a single process for assessing new drugs⁸ and provide formulary listing recommendations to all publicly funded F/P/T drug plans (except Québec). CDR is part of the Canadian Agency for Drugs and Technology in Health (CADTH) (formerly Canadian Coordinating Office for Health Technology Assessment (CCOHTA), originally established in 1990). It is funded by Canadian federal, provincial and territorial governments.

CDR reviews are considered by the Canadian Expert Drug Advisory Committee (CEDAC), an independent advisory body composed of experts in drug therapy and drug evaluation. CEDAC is appointed by, and reports to, the CADTH Board of Directors. It uses clinical and pharmacoeconomic drug reviews to evaluate the comparative benefits and costs of the drug under review (Canadian Agency for Drugs and Technologies in Health, 2008b). It then makes a common formulary listing recommendation to participating F/P/T drug plans. CEDAC may recommend a drug to be listed, listed under specific requirements or not listed, or that its recommendation is deferred pending clarification of further information.

With the exception of Québec, all Canadian jurisdictions consider CEDAC’s recommendations when developing their own publicly funded drug plans. Final decisions about formularies are taken by the respective provincial or territorial ministry of health. Although economic considerations are often taken into account, these are not necessarily key in determining the outcome of decisions on the provincial/territorial formulary. Economic considerations may refer to simple budget impact analysis or sophisticated cost-effectiveness studies provided by the manufacturer. Only Ontario and British Columbia explicitly consider pharmacoeconomic assessments as part of the decision-making process on the inclusion of a given drug into the provincial drug plan. An explicit cost-effectiveness threshold has not been defined by any jurisdiction.

In general, drugs not included in the formularies will not be covered under the public system, unless there is special permission (see below). At the same time, drug coverage in a given jurisdiction is not necessarily limited to the respective provincial/territorial drug plan. This is illustrated by the availability of new cancer drugs, which varies across jurisdictions. Thus, according to a 2005 report by the Cancer Advocacy Coalition of Canada in Québec, Prince Edward Island, New Brunswick and Nova Scotia intravenous cancer drugs (which are not reviewed by CDR) are covered through hospital global budgets. In contrast, British Columbia, Newfoundland and Saskatchewan fund these drugs through separate cancer agencies’ drugs budgets. Similar variation between provinces can be seen for coverage of oral or take-home new cancer drugs, which are only partially funded by public drug plans in Manitoba, Ontario, Québec and Nova Scotia while Alberta, Prince Edward Island and New Brunswick do not cover these (Khoo et al., 2005). In these cases, new oral and take-home drugs would be accessed through private insurance, direct out-of-pocket payment or compassionate release by pharmaceutical manufacturers (see below).

⁸ Following market authorisation by Health Canada and submission for CDR by manufacturers, the Advisory Committee on Pharmaceuticals at CADTH, or drug plans.

The formularies for in-patient care which are covered by Medicare are determined by the individual hospital. Hospital formularies are established by the relevant hospitals' Pharmaceuticals and Therapeutics Committees (P&T Committee), composed of physicians, pharmacists and, in many cases, nurses. The P&T Committee is usually a sub-committee of the Medical Advisory Committee to whom the P&T makes recommendations. Formularies are adapted to the hospital's activities and patients' profiles. The hospital formulary may include drugs not yet approved by Health Canada, but likely to be accessed by patients through the Special Access Programme (see below).

Special access to medicines

Many plans allow physicians to apply for special-use permission for drugs not listed on a given formulary, which promotes accessibility of drugs not (yet) covered by a patient's insurer. However, the administrative burden associated with obtaining special-use permission has reportedly prevented doctors from doing so in the majority of cases.

The Special Access Programme (SAP) provides access to nonmarketed drugs for practitioners treating patients with serious or life-threatening conditions when conventional therapies have failed, are unsuitable, or unavailable. The SAP authorises a manufacturer to sell a drug that cannot otherwise be sold or distributed in Canada. Drugs considered for release by the SAP include pharmaceutical, biologic, and radio-pharmaceutical products not approved for sale in Canada.

SAP guidelines stipulate that 'a decision to authorise or deny a request is made on a case-by-case basis by taking into consideration the nature of the medical emergency, the availability of marketed alternatives and the information provided in support of the request regarding the use, safety and efficacy of the drug. If access is granted, the practitioner agrees to report on the use of the drug including any adverse events encountered with such use and, upon request, account for all quantities received' (Health Canada, 2008). Accordingly, the SAP is not to be viewed as a mechanism to encourage the early use of drugs and it is not meant to circumvent regulatory review of a submission for marketing of a given drug. Importantly, access to any drug through the SAP is supposed to be limited in duration and quantity to meet emergency needs only.

Policies relating to patients' ability to 'top up' their care under the statutory system

While generally public drug plans place drugs on an 'open formulary', thereby enabling all beneficiaries to access all medicines listed on the formulary, plans also increasingly apply restrictions which limits access to certain medicines to patients who meet explicit criteria upon 'special authorisation' (LeLorier et al., 2008). These criteria are usually a subset of the approved indications, or they are used when other least costly treatments have failed. For example, in Québec, the positive formulary list for public and private prescription drug coverage includes an 'exception section' that lists drugs for selected indications only, requiring prior authorisation.

To obtain special authorisation, physicians submit an application to the drug plan which will normally be reviewed within two weeks following submission. During this period, some patients pay for their own supply of medicines or they obtain a temporary supply from the manufacturer although many (if not most) patients go untreated during the waiting period. The patient or physician is notified of the reimbursement decision in writing. If special authorisation is not granted, patients can

be treated with an approved alternative or they can choose to pay out-of-pocket for the restricted medicine (LeLorier et al., 2008).

As noted above, access to cancer drugs is often through mechanisms outside the relevant provincial drug plan, such as hospital budgets or separate cancer agencies' budgets although restrictions may apply. For example, in Ontario, the New Drug Funding Program (NDFP) funds around 75% of the costs of all intravenous (IV) cancer drugs administered in hospitals (with the remaining 25% covered by the hospitals for older drugs approved before the creation of the NDFP) while those not funded under NDFP or by hospitals' budgets are available for private payment and administered outside of hospitals (The Provincial Working Group, 2006). Patients who have been prescribed one of the unfunded IV cancer drugs would access treatment outside the province or at a private clinic. Some patients are beginning to have these drugs administered at Ontario hospitals under the care of their own oncologists for private payment.

An example for variable access to a specific cancer drug is Sunitinib for the treatment of advanced renal cell carcinoma. According to a 2007 survey by the Cancer Advocacy Coalition of Canada, as of 25 December 2007, it was fully funded in two out of ten provinces only (British Columbia and Québec) although funding is on a case-by-case basis based on disease-specific factors. Other provinces limit access to specific patient groups and/or make the drug available based on private payment (out-of-pocket, through health insurance or manufacturer's compassionate programme) with administration provided by a public cancer centre or hospital (Ontario, Nova Scotia, Alberta, New Brunswick, Newfoundland) whereas it was not funded in Saskatchewan, Manitoba and Prince Edward Island (Khoo et al., 2007). Access to Sunitinib for the treatment of second-line GIST is, in contrast, more evenly across provinces.

Denmark

Background

Health care in Denmark is largely funded through national and local taxation. It provides universal coverage to all Danish residents and access to health care services is largely free at the point of use although there is a modest share of co-payment, mostly for pharmaceuticals and dental services. Health care provision is mainly through general practitioners and specialists in private practices and public hospitals, owned by the regions (Strandberg-Larsen et al., 2007).

The Danish health system is highly decentralised, with five regions and 98 municipalities mainly responsible for organising health care. They are regulated by national legislation and, to some extent, overseen by central bodies.

The five regions were only created in January 2007 as part of the local government reform, replacing the previous 14 counties (Indenrigs- og Sundhedsministeriet, 2005). The number of municipalities was reduced from 275 to 98. The reform constitutes the most recent development in a longer history of decentralisation that has seen a gradual transfer of tasks and responsibilities from the centre to fewer, larger counties/regions and/or municipalities.

Policies on funding of licensed pharmaceuticals under the statutory system

The national government's policy essentially stipulates that 'there must always be funding available in the public health system for all treatments with documented positive effects'. However, because of a rapid growth in drug costs related to the introduction of new drugs and evidence-based broadening of the indications for cancer drugs already in use, the national and regional governments put major emphasis on how new drugs are introduced.

New drugs are licensed by the Danish Medicines Agency (DKMA), an agency under the Danish Ministry for Health and Prevention. DKMA also determines the (level of) reimbursement of drugs. If the evidence is sufficiently convincing, the regions may decide that the drug should be introduced immediately. If regions are uncertain about the benefits associated with the new drug and/or its indications, a national committee with members appointed by, among others, the regions and the medical specialist associations may ask the Danish National Board of Health (the Centre for Evaluation and Health Technology Assessment (DACEHTA) at the National Board of Health) for an evaluation of the new drug, which will prepare a technology assessment of the drug. The question is then referred to a national committee (with members from the ministry, the regions, the medical specialist societies amongst others), which advises the National Board of Health on whether the new drug should be introduced upon which the National Board of Health will prepare a recommendation which all regions will follow. Indeed, the regions have 'promised' not to introduce drugs on their own so as to prevent 'hospital shopping' by patients who are unable obtain a specific drug treatment in their own region.

There is some concern that regions and hospital departments may introduce new drugs and extend indications too slowly for financial reasons, because regions and hospitals pay for the drugs out of their budgets. At the national level, the Government and the Association of the Danish Regions discuss, on an annual basis, the volume of funding the regions may use the following year, and future drug costs have become a major issue in these negotiations. At the regional/hospital/clinical level, one solution has been to defer responsibility for drug costs (or responsibility for the

new drugs or wider indications) from some clinics (especially those in oncology or rheumatology which have experienced the greatest growth in costs – relatively and absolutely) to a fund managed by the region's administrators. Anecdotal evidence suggests that where such a fund has been established, new drugs are introduced more quickly, but that clinics become less cost-conscious, prescribing expensive brand drugs instead of less expensive generics or analogues.

Access to innovative drugs

If all conventional treatments have failed, a patient may apply for 'experimental treatment'. Here, the patient may be included in clinical trials in Denmark or abroad where the evidence for a given treatment is limited and which therefore do not constitute part of the standard treatment, often oncological treatment. Patients applying for experimental treatment are referred by the hospital where they already receive treatment. A committee under the National Board of Health advises hospital doctors on whether a specific experimental treatment warrants referral. Referral to experimental treatment is based on the opinion of several specialists in oncology; this system is not aimed at providing free access to 'alternative' treatment.

Exclusions of licensed drugs from reimbursement/subsidy under the statutory system

On several occasions the Danish authorities have decided not to fund an appropriately licensed drug under the public system. For example, the Association of Regional Councils (local government at the regional level with responsibility for health care provided by hospitals and general practitioners) decided not to introduce treatment of advanced multiple sclerosis with beta-interferon, judging that the benefits of the treatment were marginal compared to its costs. This case was very unusual, however, as the evaluation of the effects of drugs is generally viewed as a question for specialists rather than politicians. However, eventually the national government decided that the regions had to introduce treatment with beta-interferon.

Policies in relation to patients' ability to 'top up' their care under the statutory system

The Danish Health Act (2005) indicates that hospital treatment is provided free at the point of delivery. This right applies to all drugs prescribed at a hospital. The following case illustrates how fundamental this point is to Danish health care:

Bisphosphonates are prescribed by GPs for the treatment of osteoporosis and this treatment is subsidised by the regions as stipulated by law. Most patients take their medication orally but other patients receive intravenous treatment. At least one region was confronted by GPs who were reluctant to treat patients intravenously. The region therefore asked patients to buy the drug as usual and go to a hospital for intravenous treatment. However, the Danish Ministry for Health and Prevention has judged that this approach constitutes a breach of the regulations on payment for hospital treatment and has therefore considered this approach to treatment as illegal.

There have been cases of some cancer patients who have travelled abroad for alternative treatment and have asked their Danish hospital for one or more X-rays to monitor the development of their disease. However, radiology departments have tended to decline such requests because of the lack of an adequate clinical indication (i.e. no referral from a general practitioner or a hospital in Denmark) but also because of capacity constraints.

In general, however, there appears to be a growing acceptance of – and interest in – the possibility of 'topping up' with insurance schemes as a supplement to tax-

financed health care. At the same time, charging patients an additional fee for hospital treatments would probably cause major political and public concern. Some patients travel abroad for treatments which are not provided by Danish hospitals because they are not considered to be evidence-based.

Finland

Background

Finland's highly decentralised health system is mainly funded through local and national taxation plus a National Health Insurance (NHI) scheme based on compulsory insurance fees, which is administered by the Social Insurance Institution (SII, Finnish acronym KELA). Delivery of health care is mainly public, with municipal taxes accounting for almost half of all funding, supplemented by state subsidies, national health insurance contributions and some co-payments (Habl et al., 2006). Health system governance is shared by the centre and the municipalities, with municipalities being responsible for organising primary care and, through participation in hospital districts, secondary and tertiary care (Ettelt et al., 2006). Municipalities provide health and social services independently or in co-operation with neighbouring municipalities; they can purchase services from other municipalities, non-governmental organisations or for-profit providers.

Policies on funding of licensed pharmaceuticals under the statutory system

Pharmaceutical reimbursement is regulated by the NHI legislation and administered by KELA. The main rules are set out in the Health Insurance Act and supplementary provisions are laid down by decrees issued by the Government, the Ministry of Social Affairs and Health (STM) and by decisions made by the Pharmaceutical Pricing Board (PPB).

The Pharmaceutical Pricing Board (PPB) is attached to the Ministry of Social Affairs and Health's insurance department which appoints seven members to the Board: two representatives each from the Ministry of Social Affairs and Health and from KELA as well as one each from the Ministry of Finance, the National Agency for Medicines and the National Research and Development Centre for Welfare and Health (STAKES). The Pharmaceutical Pricing Board provides legal, medical, pharmaceutical, economic and social insurance expertise. Board members serve for three years but can be renominated.

The key responsibilities of the PPB are:

- to decide on the reimbursement status of pharmaceuticals (and their discontinuation),
- to confirm the reasonable wholesale prices of pharmaceuticals and related increases (Peura et al., 2007).

Once a manufacturer has submitted an application for inclusion of a drug in the positive list for reimbursement, the PPB is mandated by law to evaluate the drug based on: therapeutic benefit; patient benefit; economic information (e.g. cost-effectiveness, pharmaceutical companies must submit pharmacoeconomic evaluation of their product); a comparison of wholesale prices of competitive products in Finland; a comparison of prices of the drug in other EU countries; the budgetary impact and manufacturing costs (production and research).

The Ministry of Social Affairs and Health appoints an expert group (maximum of seven members) to inform PPB decisions. Members are nominated on the basis of their expertise and not as representatives of a specific institution or organization; they provide medical, pharmaceutical, health economics and social insurance knowledge. Members of the current expert group come from the Ministry of Social Affairs and

Health, KELA, STAKES, university hospitals and universities. The PPB receives their advice but the advice is not binding.

The PPB takes up to 180 days to make a joint decision on the pricing and reimbursement of drugs. There are three reimbursement categories: basic reimbursement and two sets of special reimbursement, with the lower special category comprising drugs for the treatment of 10 chronic conditions such as asthma and hypertension. The higher special category consists of drugs for the treatment of 34 severe or life-threatening illnesses such as diabetes or cancer. Criteria for the inclusion of drugs in the higher level of reimbursement include the severity of the disease; necessity and cost-effectiveness of the medicinal product; proven therapeutic value of the medicinal product, and funds available for special reimbursement products.

A drug considered to have a reasonable price and to be valid for reimbursement is grouped in the basic reimbursement category (42% reimbursement). For higher reimbursement levels (72% and 100%) a manufacturer must submit evidence on the drug's therapeutic value and cost-effectiveness before it can be considered for one of the special categories. In practice, drugs are usually sold in the basic category for an average of two years, with only few exceptions (Mossialos and Srivastava, 2008). For patients who pay more than the annual limit of EUR 616.72 (in 2006), KELA covers all costs with a co-payment of EUR 1.50 per medicine per purchase.

Pharmaceuticals for in-patient care are free for hospital patients and are not part of the health insurance scheme. Whether or not a specific drug will be included in the hospital's formulary – and thus provided for free to the patient – is decided by the hospital committee.

Policies relating to patients' ability to 'top up' their care under the statutory system

Patients who choose to pay out of pocket for out-patient drugs may do so without any restrictions and they will remain within the statutory system. However, patients cannot pay for drugs for in-patient care; pharmaceutical expenditure is included in the daily cost for care provided in public hospitals. Private hospitals may charge extra but this is not considered a major issue in Finnish health care as only 5% of hospital care is provided in private hospitals. In addition, a major proportion of care provided in private hospitals is purchased by municipalities, which means that policies on patient co-payments are the same as in public hospitals.

New cancer drugs in particular are widely used in public hospitals and there are few cases of patients seeking access to cancer drugs in the private sector if denied under the statutory system. This tends to be occurring only in cases of terminally ill patients. If a patient does seek care in a private hospital and pays for the care out of pocket, s/he will not only have to pay for the medication but will also have to cover most of the costs associated with administering the drug (with the remainder being covered through national health insurance).

In general however, 'topping up' is not a major health policy issue in Finland as it is still quite rare and mainly occurs in cases of treatments that are considered not effective.

France

Background

Health care in France is funded through a combination of social health insurance (SHI) contributions, tax revenues and patients' co-payments, mostly through supplementary private insurance. Health care is delivered through a mix of public and private providers, with generalist and specialist physicians largely working in private practice. Hospitals are public or private (for profit and not-for-profit), with public hospitals being general, regional or local community level, depending on size and level of specialisation (Sandier et al., 2004). Social health insurance covers all residents; patients receive publicly funded care in any facility, independent of its ownership status.

Governance of the health system has traditionally been centralised with the Ministry of Health (and other ministries depending on the division of tasks in the Government of the day), playing a major role in steering and directing the funding and delivery of health care. Parliament also plays an important role in regulating the health system, specifically since a Parliamentary vote on an annual maximum SHI expenditure was introduced in 1996.

Policies on funding of licensed pharmaceuticals under the statutory system

The French health system reimburses drugs according to a positive list which is determined by the Ministry of Health, following technical advice from the Transparency Commission (TC) which is part of the High Health Authority (HAS). Only pharmaceuticals which improve medical services or create savings in the cost of treatment are eligible for reimbursement. The products are evaluated according to the following criteria (van Ganse et al., 2007):

- effectiveness and possible side-effects
- position in the therapeutic spectrum relative to other available treatments
- severity of disease or condition
- clinical profile of the drug
- public health impact.

The pharmaceutical reimbursement rates in the French statutory system are set by the National Union of Complementary Health Insurance Funds (UNCAM), following assessment of the medical service and improvement of medical service by the Transparency Commission, and cost-efficacy assessment and pricing by the Economic Committee for Health Care Products (CEPS).

Access to innovative drugs

New innovative drugs are available to every patient treated in the public or private health care sectors. However, in-patient treatment with innovative drugs is a special case. In France, hospitals are reimbursed through a prospective payment system using prices based on a national tariff per diagnosis-related group (DRG) in combination with budgets and additional payments for some services. Treatment of a limited number of patients with an innovative drug within a given DRG requires

specific approval by the Ministry of Health and the Social Health Insurance in order to ensure the availability of the drug. For example, an expensive innovative drug such as Avastin which is not covered by the existing DRG cost schedule will be reimbursed for in-patient care in addition to the DRG cost, provided that the hospital can justify its appropriate use. Appropriate use is defined by the National Health Authority (HAS) and the National Cancer Institute (INCA) and is based on the medical evidence available.

The list of drugs and devices concerned is available from the Technical Hospitalisation Information Agency (ATIH), an agency supervised by the Ministers of Health and of Social Security (Agence Technique de l'Information sur l'Hospitalisation, 2008).

Specific utilisation guidelines (*référentiels de bon usage*) (Box 4) are used to draw up a contract between the Ministry of Health, sickness funds and hospitals. The guidelines are binding, i.e. they have to be followed by the hospital in order to qualify for reimbursement for the drug. However, because such a system creates an incentive to use the drugs, the Ministry of Health does not publish the price threshold beyond which they will apply this financing mechanism. Patients are not affected financially by this system.

Box 4 Utilisation guidelines: Example bevacizumab (Avastin)

Utilisation guidelines		Bevacizumab (Avastin)
Group 1	Fully validated (appropriate)	First line metastatic non small cell lung carcinoma in combination with platinum
Group 2 (2a, 2b)	Partly validated, requires confirmation (possibly appropriate but preferably administered as part of a research protocol or with systematic monitoring of outcome)	Second line metastatic colorectal cancer in combination with Folinic acid-Fluorouracil-Oxaliplatin (Folfox)
Group 3	Not validated (inappropriate)	Small cell lung carcinoma

The hospital will be fully reimbursed only if the treatment/indication has been classified as group 1 or 2.

Policies relating to patients' ability to 'top up' their care under the statutory system

Topping up is not an issue for the French health system, since the State's official line underlines that every French person should have exactly the same access to healthcare. All legal residents are covered through the SHI and supplemental insurance can be obtained at no or minimal cost for persons below a resource threshold. Illegal residents are covered the State medical assistance (*Aide médicale d'Etat*, AME).

Germany

Background

The German health system is funded largely through mandatory social health insurance (SHI) which covers the majority of the population, while around 15% of the population is covered by three complementary schemes: private health insurance, and two specific government schemes for civil servants.

Ambulatory care is mainly provided by office-based primary and specialist care physicians who have been granted a monopoly to provide care outside hospital. Hospitals are owned and operated by a variety of private for-profit, public and charitable/religious organisations.

Decision-making in the German health sector is shared between the federal government, the states (*Länder*) and corporatist actors, such as the statutory health insurance funds and provider organisations. Responsibilities are set out in the Social Code Book (*Sozialgesetzbuch*), the regulatory framework for the German social health insurance system.

Policies on funding of licensed pharmaceuticals under the statutory system

In Germany's self-governing health system, the Joint Federal Committee (*Gemeinsamer Bundesausschuss*, G-BA) is the highest decision-making body at federal level. Established in 2004, the Joint Federal Committee represents the federal association of statutory health insurance funds, the federal associations of provider groups (SHI physicians, dentists, hospitals), patient organisations and independent members nominated by either the payer or provider side.

The Joint Federal Committee is responsible for defining the services and procedures covered by the statutory health system; these include all aspects of medical care, dental care, medical diagnostics, maternal care, new technologies, pharmaceuticals and medical devices, hospital care and home nursing, medical rehabilitation, fertility treatment, patient transport, palliative care and vaccination. It also groups pharmaceuticals for reference pricing (see below), negotiates rebates with pharmaceutical providers, and has developed a framework contract with the associations of pharmacists for the services provided by pharmacists under SHI.

The Joint Federal Committee is an independent legal entity and is not part of the Ministry of Health. However, it is accountable to the Ministry of Health and its decisions must be submitted to the Ministry of Health. The Ministry of Health has a period of two months to veto any Joint Federal Committee decision, after which decisions will become effective through publication in the Federal Gazette (*Bundesanzeiger*).

Decision-making of the Federal Joint Committee is supported by the Institute of Quality and Efficiency (IQWiG) which commissions health technology assessments (HTA) to inform recommendations on the reimbursement of technologies, including pharmaceuticals. These are generally based on the efficacy and effectiveness of new technologies, using systematic appraisal of published evidence but excluding data submitted with the manufacturers' submission for reimbursement. IQWiG considers four criteria (Sorenson C et al., 2008):

- nature and severity of the disease

- magnitude of the therapeutic effect
- availability of alternative treatments
- side-effects and risk of adverse events

Germany does not use a positive list of drugs reimbursable through SHI. In principle, every (prescription) drug that has received market authorisation is automatically covered by the statutory system, except for drugs for minor illnesses (common colds, drugs for the oral cavity with the exception of antifungals, laxatives and drugs for motion sickness) which are legally excluded (for the insured over 18 years) and lifestyle drugs. In addition, the Social Code Book allows the Federal Minister of Health to exclude drugs considered inefficient, i.e. drugs which are not effective for the desired purpose, or drugs with combinations of active agents, the effect of which cannot be evaluated with certainty.

The coverage of pharmaceuticals under the statutory system is also regulated by the Directive on Pharmaceutical Care of the Federal Joint Committee, which can limit the prescription of some drugs to certain indications and limit the reimbursement level of drugs through reference pricing or defining a maximum reimbursement level (Stargardt et al., 2008).

Access to innovative drugs

There is generally no reimbursement limit for drugs considered innovative (the reimbursement of all other prescription drugs is restricted by the German reference pricing system as stipulated by law). Judgement of a drug as 'innovative' as defined by the Federal Joint Committee is based on an assessment of whether the drug has a therapeutic advantage or fewer side-effects. This regulation has however been amended with the Act to Strengthen Competition in Statutory Health Insurance (2007) which enabled the Federal Joint Committee to also set reimbursement limits for innovative drugs. Thus, from 2008, reviews by the Institute for Quality and Efficiency (IQWiG) will be used by the Federal Joint Committee to ensure that prices for innovative pharmaceuticals are appropriate to their effectiveness (Stargardt et al., 2008).

An issue that has received increasing attention is off-label drug use, raising concerns about access to innovations as well as pharmacovigilance and liability. Generally, drugs that are not licensed for the German pharmaceutical market or are not licensed for the respective indication may not be prescribed by any physician except under clinical trial conditions. Statutory health insurance funds may not fund clinical research and may basically not cover prescriptions of unlicensed drugs or for unlicensed indications. This has led to a series of lawsuits over the reimbursement of off-label prescribing. The legal uncertainty was eventually addressed by the federal social court which defined three criteria that have to be met to allow for the reimbursement of off-label drug use under the statutory system. Thus, decisions of statutory health insurance funds on whether to reimburse the off-label use of a given drug will depend on the following: whether the drug is used (a) for the treatment of a serious illness for which (b) there is no alternative therapy and (c) there are, based on the available evidence, reasonable expectations that the treatment might be effective (Bundesinstitut fuer Arzneimittel und Medizinprodukte, 2006).

In 2002, the federal ministry of health stipulated the creation of an expert committee based at the Federal Institute for Drugs and Medical Devices (BfArM) to clarify rules on off-label use of drugs. There are currently three expert committees, looking into

off-label use of drugs in the areas of oncology, infectious disease (focus: HIV/AIDS) and neurology/psychiatry.

Exclusions of licensed drugs from reimbursement/subsidy under the statutory system

As indicated above, the German statutory system principally reimburses all licensed prescription drugs except those for the treatment of minor illnesses, drugs considered as inefficient as well as so-called lifestyle drugs defined as those which are predominantly aimed at improving the quality of life. One example for the latter are drugs for the treatment of erectile dysfunction (such as sildenafil (Viagra)), which were excluded from reimbursement under the statutory system on grounds that the primary motive for using these drugs is personal lifestyle rather than the treatment of disease (Stargardt et al., 2008). Patients will have to pay for the drug directly (out-of-pocket); however, there is no co-payment for consulting a physician in order to obtain a prescription for the drug.

In the rare case of patients receiving a drug which is excluded from reimbursement under the statutory system in a hospital setting, patients may have to also pay for cost incurred through administering the drug. However, this is a highly theoretical example.

Policies relating to patients' ability to 'top up' their care under the statutory system

Reference pricing for pharmaceuticals (as well as the 'new' maximum reimbursement prices) could be interpreted as 'topping up'. Despite the existence of reference prices, the manufacturer is free to decide on the price of their product. If a drug prescribed by the practitioner is priced above its reference price (i.e. the amount the statutory system would pay for standard care), the patient will have to pay the difference between the retail price and the reference price, in addition to the regular co-payments required for prescription drugs. However, this is rarely the case, as, in 2005, only 7.1% of the pharmaceuticals available on the market were priced above the reference price.

Drugs subject to reference pricing have to be therapeutically comparable to the other drugs in a given drug cluster, amongst other criteria. If drugs are not 'comparable', there will be no reference pricing cluster. One example is the statin reference pricing cluster: this cluster consists of simvastatin, fluvastatin, lovastatin, pravastatin, and atorvastatin for which a reference price was set. Except for atorvastatin, the retail prices of all other statins were either already lower than the reference price or they were reduced to the level of the reference price. Thus, if a patient wishes to receive atorvastatin instead of any other statin, s/he will have to pay the difference between the retail price and the reference price.

The fairly low proportion of pharmaceuticals that are priced above the reference price – and would therefore require 'topping up' from patients who demand that product – is largely because physicians who choose to prescribe a drug which is priced above the reference price are legally required to justify their decision to their patients since all drugs in a reference pricing cluster are judged to be similar by the Federal Joint Committee. Also, all reference prices are determined in a way that at least one drug in each reference pricing cluster is priced at or below its reference price (i.e. does not require additional co-payment). Furthermore, the market share of drugs which required additional co-payments because they were priced above the reference price fell quite rapidly. Returning to the example of statins mentioned above: statins became subject to reference pricing in 2005 and the manufacturer Pfizer kept the

price of atorvastatin above its reference price. However, within one year, atorvastatin had lost 85% of its share in the German pharmaceutical market.

Italy

Background

Public health care in Italy is delivered through a National Health Service (*Servizio Sanitario Nazionale*) and largely funded through national and regional taxes, supplemented by co-payments. The organisation of health care falls within the remit of the 19 regions and two autonomous provinces (Habl et al., 2006).

Central government provides the legislative framework for health care and defines the basic principles and objectives within which the National Health Service operates. Central government also has a constitutional obligation to guarantee access to health care in each of the regions, to reduce health inequalities and to ensure that the health system operates efficiently and transparently. It defines, through the Ministry of Health, the basic basket of health services to be provided by the regions (*Livelli Essenziali di Assistenza*, Essential Levels of Care).

Policies on funding of licensed pharmaceuticals under the statutory system

The main actors in the pharmaceutical system in Italy are: the Italian Medicines Agency (AIFA), the Ministry of Health, the Ministry of Finance and the regions.

The AIFA was created in July 2004; it is responsible for all matters of pharmaceuticals for human use including: market authorisation, pharmacovigilance, and the pricing and reimbursement of all pharmaceuticals. AIFA operates autonomously under the direction and oversight of the ministries of health and of Finance, cooperating with the regions on price setting and reimbursement. The Technical Scientific Committee (CTS) at AIFA is responsible for the assessment of the inclusion of new drugs for reimbursement under the National Health Service.

The Italian pharmaceutical system groups drugs into main reimbursement categories according to a combination of relevance in terms of effectiveness and cost:

- Class A: fully reimbursed under the National Health Service
- Class B: partially reimbursed under the National Health Service with co-payment requirements
- Class C: not reimbursed under the National Health Service.

Further to this, the 19 Regions and two autonomous provinces must provide drugs in scheme A free of charge to all citizens; they may theoretically also decide to fully fund, using their own resources, drugs included in scheme B and so provide these free of charge to their residents.

Reimbursable pharmaceuticals are included in a positive list, the National Pharmaceutical Formulary (PFN), and are administered at the central level by AIFA. The list is updated annually, or every six months if the public pharmaceutical expenditure exceeds a 13% ceiling, and the criteria for the reimbursement granted by AIFA are provided to CTS by the Interministerial Committee for Economic Planning (CIPE).

The use of HTA for regulatory purposes is limited in Italy. Economic analyses are mainly commissioned by pharmaceutical companies, addressed to the regulator,

aiming to support the pricing and the reimbursement process, but also to influence physicians' prescribing patterns.

The inclusion of pharmacoeconomic information in the pricing and reimbursement process is not mandatory, and only a few regions use cost-effectiveness analyses to inform decision-making on the management of a specific disease. However, such analyses are fairly uncommon and have only limited impact on the decision-making process (Martini et al., 2007).

The reimbursement system uses two types of out-of-pocket payments: (1) a fixed amount per prescription and/or per pack (at regional level and only in some regions) and (2) a co-payment for pharmaceuticals (at regional and national level) covering the difference between the price of a more expensive pharmaceutical and a cheaper drug containing the same active substance.

Certain categories of people are excluded from co-payments, such as chronically ill patients, people with rare diseases, disabled people and pregnant women.

Reimbursement for in-patient pharmaceuticals does not differ from the ambulatory sector. Some pharmaceuticals are classified as hospital-only medicines, requiring specialist supervision, and are grouped under class H (a sub-category of class A). These pharmaceuticals, including others that are used in in-patient care, are fully reimbursed according to criteria similar to those used for ambulatory care. The main payer of in-patient pharmaceuticals is the Italian NHS, paid for through the regions (Martini et al., 2007).

Policies in relation to patients' ability to 'top up' their care under the statutory system

Italian patients can 'top up' their treatment if they choose to do so. Thus, it is possible for an Italian citizen to receive in-patient treatment and at the same time pay for a specific drug which is not part of the National Pharmaceutical Formulary. One example is ranibizumab (Lucentis) for the treatment of wet acute macular degeneration (AMD) which has been excluded from reimbursement under the statutory system. Patients with AMD will be able to access Lucentis in most regions if they are willing to pay for the drug out-of pocket. A few regions such as Tuscany have decided to fully fund Lucentis so providing the drug for free to their residents.

Patients wishing to 'top up' their care by paying directly for a given drug which is not reimbursable under the Italian National Health Service will however remain a patient within the statutory system for the treatment of that condition. The clinical responsibility for supervising the treatment rests with the hospital where the treatment is carried out; the relevant region will reimburse the hospital according to standard payment modalities (i.e. DRG).

As regions are free to decide whether or not to fund, from their own resources, a drug which is not reimbursable under the National Pharmaceutical Formulary there is considerable room for regional variation in access to certain drugs, thus raising perceptions of an inequitable system. 'Topping-up' is thus a matter of policy concern in Italy.

The Netherlands

Background

The Dutch health system underwent a major restructuring process following the Health Insurance Act (2006) (ZVV) that made health insurance compulsory for all residents. Under the new framework, all residents are entitled to the same comprehensive core basket of health services, which they purchase from private health insurers. The core health basket includes all acute care provided by hospitals, general practitioners and specialists as well as all drug and devices costs. Residents can take out voluntary health insurance to cover additional services. Health services are generally delivered through private providers in both the ambulatory and hospital sector. Hospitals have traditionally been owned and operated by private not-for-profit organizations.

Policies on funding of licensed pharmaceuticals under the statutory system

The Health Insurance Act does not provide a comprehensive negative or positive list of individual services. Instead, legislation outlines the general types of medical services covered. The Dutch generally use a ‘usual care’ criterion to determine patients’ entitlements to interventions. The criterion is not very restrictive and yields local variations in service provision, which are moderated by practice guidelines.

Implementation of pharmaceutical policy lies with the Ministry of Health, Welfare and Sport, and it is guided by the principle of safe and affordable pharmaceutical care for all. Historically, registration of a drug for market access, which is regulated by the Medicines Evaluation Board (MEB) resulted in most cases in an almost automatic reimbursement by the statutory health insurance funds (Sorenson C et al., 2008). However, under the new Health Insurance Act the Ministry of Health, Welfare and Sport requires evidence of cost-effectiveness in order to include a drug in the core basket of services (a positive list of drugs) covered by the insurance funds. At the same time, the Government may remove ineffective or obsolete drugs from the core service basket.

The reimbursement of drugs is determined by the Health Care Insurance Board’s (CVZ) Pharmaceutical Aid Committee (CFH) which assesses reimbursement of new medications and gives advice to the Ministry on the basis of several fixed criteria. These are:

- therapeutic value
- patient benefit
- cost-effectiveness
- financial impact on the core basket of services, pharmaceutical and health budgets, the insurance funds and the Dutch society.

The Committee also takes into consideration pharmaceutical and/or innovative characteristics, the availability of therapeutic alternatives and social, ethical and other legal criteria. The economic analysis undertaken is from a societal perspective. Assessments are conducted between one or two months up to one year or more.

Access to high-cost medicines

For very expensive drugs, additional coverage depends on the insurer and varies by the type of supplementary insurance chosen and agreements made between hospitals and individual insurers. Expensive in-patient drugs in particular fall under the responsibility of the Dutch Health Care Authority (NZa) which decides on the reimbursement of the costs within hospital budgets.

The introduction of expensive drugs in the Dutch statutory system has been subject to controversial debate since the mid-1990s. For example the introduction of paclitaxel (Taxol) was heavily debated because of its high costs and its relatively low cost-effectiveness. However, the general view was opposed to the idea of withholding this particular treatment from severely ill patients merely on the grounds of cost. The Government therefore decided to contribute towards the costs of taxoids by subsidising the cost of the treatment.

The taxoids case appears to have had set a precedent since in recent years further cancer drugs have been introduced but without a wide public debate concerning the financial implications (Niezen et al., 2006). In addition, the possibilities for funding high-cost treatments were expanded. For example in 2002, the Dutch Government implemented a law that requires health insurers to contribute to the costs of some expensive drugs. As a result, all drugs that are listed under the 'Regulation for Expensive Medicines' receive separate reimbursement rates. Until 2005, Dutch hospitals had to pay between 25% and 100% of the related expenditure, depending on their agreements with the insurers. From 2006 however the reimbursement rate for hospitals has been fixed at 80%. This list includes drugs such as irinotecan (Camptosar), rastuzumab (Herceptin), bevacizumab (Avastin) and bortezomib (Velcade).

It is worthwhile noting that media attention has been sought recently to encourage health insurers to pay for an expensive drug, and, in some cases, health insurers view it as good marketing when they agree to reimburse an expensive drug.

Policies in relation to patients' ability to 'top up' their care under the statutory system

'Top-up' payments within the Dutch statutory health system are quite rare, and as a result 'top-up' payments are not a major policy issue within the Dutch statutory health system.

New Zealand

Background

Health care in New Zealand is largely financed through general taxation with some private payments, supplemented by statutory insurance for accidents and injuries.

The New Zealand health system serves a population of 4.2 million people. Responsibilities in the public health system are defined through a number of laws, most recently through the New Zealand Public Health and Disability Act (2000) (NZPHDA). The NZPHDA established the current structure of the public health system by delegating the organisation of health services to 21 newly created district health boards. The provision of health services is, largely, through publicly owned hospitals and 81 primary health organisations, which co-ordinate primary health services on behalf of the enrolled population.

Pharmac

Pharmac (the Pharmaceutical Management Agency) is a standalone Crown entity and is directly accountable to the Minister of Health. It was set up under the Health and Disabilities Services Act (1993) and it is responsible for managing the community pharmaceutical budget on behalf of District Health Boards (DHBs) and deciding which medicines are funded by the Government.

Pharmac's mandate is to 'secure for eligible people in need of pharmaceuticals, the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the funding provided' as outlined in the NZPHDA.

The majority of prescription medicines used in New Zealand are publicly subsidised via Pharmac. The agency's role in prioritising which medicines are publicly subsidised is crucial to ensuring that the best health outcomes are obtained from medicines and that those outcomes provide the best value for money.

The decision-making process centres on nine criteria (Box 5). The criteria are applied to each funding decision, and weighted as Pharmac considers appropriate. In making its decisions and applying the criteria, three key analyses are required:

- an assessment of the relative clinical effectiveness of the medicine;
- an assessment of the cost-effectiveness of the medicine;
- an assessment of the affordability of the medicine within the budget available.

Box 5 Pharmac's decision-making criteria

1. The health needs of all eligible people within New Zealand
2. The particular health needs of Māori and Pacific peoples
3. The availability and suitability of existing medicines, therapeutic medical devices and related products
4. The clinical benefits and risks of pharmaceuticals
5. The cost-effectiveness of meeting health needs by funding pharmaceuticals rather than using other publicly funded health and disability support services
6. The budgetary impact (in terms of the pharmaceutical budget and the Government's overall health budget) of any changes to the Pharmaceutical Schedule
7. The direct cost to health service users
8. The Government's priorities for health funding, as set out in any objectives notified by the Crown to Pharmac, or in Pharmac's Funding Agreement, or elsewhere
9. Other criteria as Pharmac believes fit – Pharmac will carry out appropriate consultation when it intends to take any such 'other criteria' into account

Currently, funding decisions about high-cost medicines are treated in the same way as other medicines although Pharmac is currently reviewing whether high-cost medicines require a different funding approach to other medicines. Preliminary work on this issue suggests that high-cost medicines should not be treated differently to other medicines.

The Pharmaceutical Schedule

Pharmac's central role is to maintain and manage the Pharmaceutical Schedule, which determines eligibility and criteria for the provision of subsidies throughout New Zealand (NZPHD Act 2000, section 48(a)). The NZPHD Act also requires that DHBs act consistently with the Pharmaceutical Schedule (section 23(7)).

The Pharmaceutical Schedule is a list of over 2000 pharmaceuticals and related products that are publicly subsidised for New Zealanders. It is organised by therapeutic groups (and sub-groups), and Pharmac aims to have a fully subsidised treatment available for each therapeutic sub-group. This approach means that not all medicines that may treat a condition are fully subsidised. The Pharmaceutical Schedule also lists some of the pharmaceuticals purchased by DHBs for use in their hospitals, and includes hospital pharmaceuticals for which Pharmac has negotiated national contracts.

Policies on funding of licensed pharmaceuticals under the statutory system

The Pharmac Board decides which medicines are included on the Pharmaceutical Schedule, the level of subsidy, and any prescribing guidelines and conditions. Pharmac decisions are binding for the DHBs and do not require ministerial approval.

Applications for new pharmaceuticals to be listed on the Pharmaceutical Schedule or for expanding access to pharmaceuticals, once Medsafe has registered the medicine, are usually initiated by pharmaceutical suppliers, although they can also be

initiated by health professionals and members of the public. Following the receipt of a funding proposal, the clinical evidence is reviewed by an independent group of medical practitioners, the Pharmacology and Therapeutic Advisory Committee (PTAC) (Box 6). The committee makes recommendations to the Pharmac Board for assignment of high, medium or low priority of proposals for further evaluation, or that a proposal be declined, referred back to the supplier, or referred to a sub-committee to clarify whether the application meets the criteria for evaluation.

Box 6 Pharmacology and Therapeutics Advisory Committee (PTAC)

PTAC has a central role in providing input to Pharmac's decision-making processes. An advisory committee to Pharmac its statutory purpose is to provide Pharmac with objective advice on pharmaceuticals and their benefits. Its members are medical practitioners with broad experience and a particular interest in pharmaceuticals and their therapeutic indications, and well-developed critical appraisal skills. The Director-General of Health, in consultation with the Pharmac Board, appoints PTAC members.

PTAC recommends to Pharmac whether a drug should be funded, and provides its view on whether the drug should have a low, medium, high or cost-neutral priority for funding. Recommendations are advisory only, and the Pharmac Board is not required to accept its recommendations, although PTAC's recommendations are given serious consideration.

PTAC's recommendations are based on the nine criteria outlined in Box 5 and its deliberations are informed by the detailed information provided by pharmaceutical companies and supplemented by Pharmac staff if required. The committee can also seek advice from other sources if it considers this necessary. In particular, it is important that PTAC has good information in order to be able to make sound recommendations to Pharmac about such matters as:

the condition a pharmaceutical is to treat, the severity of the condition and the clinical indications for treatment, including the stage of disease, co-morbidities, whether the treatment is first-line (the main treatment for an illness) or second-line (alternative treatments that are used due to efficacy or, sometimes, cost) and whether the treatment should be used in combination with other treatments;

whether the treatment is preventive, curative, relieves symptoms or is palliative, and the degree of benefit that could be expected (eg, prevention of premature death, prevention of poor long-term outcomes, or improving immediate outcomes);

availability of alternative treatments, including other medicines, surgery, preventive programmes or mental health services;

the patient population that could be expected to benefit from the treatment and the burden of disease in the community, and the outcomes the affected population could expect from other forms of treatment;

the impact on outcomes for Māori and Pacific peoples that could be expected from the treatment;

the quality of the evidence that supports the application for a treatment to be funded and the applicability of the evidence in the New Zealand setting.

PTAC regularly seeks advice from the sub-committees to ensure there is a sharp focus and high level of expertise in specific health areas/therapeutic groups. At times, ad hoc sub-committees will also be set up to meet a specific purpose.

Furthermore, in some cases, Pharmac sets criteria that need to be met before a medicine will be subsidised. One such mechanism is the Special Authority

arrangement, which can be used to target certain medicines to a particular patient group.

Based on the PTAC prioritisation (and any other relevant criteria such as level of analysis required and stage of the negotiation process with the pharmaceutical supplier), pharmaceuticals are then prioritised ‘in-house’ for cost-utility analysis (CUA). CUA is used as a means to assess the relative cost-effectiveness of a pharmaceutical compared to other funding options. The analysis is undertaken from the perspective of the funder (i.e. for health services provided in the public sector). The majority of new applications require a cost-utility analysis which is done ‘in-house’ by Pharmac staff. Views of patients and consumers are considered in the decision-making process through the Consumer Advisory Committee (CAC). There is no threshold below which a pharmaceutical is considered ‘cost-effective’ or above which it is automatically excluded from the Pharmaceutical Schedule. Cost-effectiveness is only one of the nine criteria Pharmac applies for decision-making. In addition, whether a product is considered cost-effective will also depend on the range of additional pharmaceuticals that could be funded within the current budget and the amount of funding available. This will vary over time. Normally orphan drugs and high-cost medicines are treated no differently to other pharmaceuticals considered by Pharmac. The higher cost of selected medicines should not form justification in itself to adopt a different funding approach (Ettelt et al., 2007).

If Pharmac determines that a medicine cannot be provided through the Pharmaceutical Schedule, it may still be able to be publicly funded in special cases. There are three exceptional circumstances schemes through which public funding may be available:

- The *Community Exceptional Circumstances Scheme* – this is used in circumstances where the provision of a funded medicine in the community is appropriate, but funding from the pharmaceutical budget cannot be provided through the Pharmaceutical Schedule. This scheme requires that the condition (or combination of circumstances) is rare and unusual (occurring in less than 10 cases annually).
- The *Hospital Exceptional Circumstances Scheme* – this is used by DHB hospitals to determine whether a medicine should be funded for use in the community in circumstances where the medication is not on the Pharmaceutical Schedule, cannot be funded through the Community Exceptional Circumstances scheme and is not a Discretionary Community Supply pharmaceutical. There is no rarity criterion for the Hospital Exceptional Circumstances Scheme, but the treatment applied for needs to be cost-effective to the DHB.
- The *Cancer Exceptional Circumstances Scheme* – this is used by DHB hospitals to determine whether they can fund pharmaceuticals for the treatment of cancer in their hospital or as part of hospital outpatient services, in circumstances where the pharmaceutical is not identified as a pharmaceutical cancer treatment in sections A–H of the Pharmaceutical Schedule.

Policies in relation to patients’ ability to ‘top up’ their care under the statutory system

Patients who choose to pay privately for drugs that are not publicly funded may do so in a community setting or in a private hospital setting. However, if they are in a public hospital, patients are generally not allowed to pay for drugs that are not publicly

funded. This is because administration of the drugs and monitoring for any reactions to the drugs will involve public hospital staff time that could otherwise be dedicated to other public services. For example, the administration of Herceptin requires three monthly echocardiograms and weekly or three-weekly infusions of the drug. The annual cost of administering Herceptin is estimated to be more than NZD 20,000 (approx. GBP 7,400; EUR 9,300).

This policy is supported by the Private Involvement Protocols which were approved by Cabinet in 2000. The Private Involvement Protocols contain two clauses that are relevant to this situation:

- a) first and foremost, there is a direct benefit to publicly-funded patients or people with disabilities, i.e., the private involvement leads to an improvement in the clinical quality or efficiency of a service for public patients...
- b) if DHB staff will be directly involved in the delivery of privately-funded services (as opposed to the DHB simply making spare facilities or land available), the services must be part of the range and standard of services (clinical and non-clinical) that are publicly-funded.

However, with the advent of a number of cancer drugs in recent years, there has been pressure to allow the administration of certain privately funded drugs in public facilities. One example is bevacizumab (Avastin) which is used in the treatment of bowel cancer. The Ministry is considering allowing DHBs to approve this under the following circumstances:

- a. Provision of care can be managed within department resources and would not impact on the prioritisation or provision of care to other patients;
- b. There is unlikely to be significant additional cost or resource implication above those required for provision of the treatment to which the patient would be entitled to receive in the public sector;
- c. This is an unusual combination of circumstances that is not a frequent occurrence;
- d. The treatment is time limited and only continues while the patient is showing evidence of maintaining a response;
- e. There is a probability of a useful response that may offset costs of alternative management.

Decisions of particular DHBs may differ due to the proximity of private hospitals capable of caring for certain patients. For example, the lack of private hospitals in the lower South Island capable of treating patients with bowel cancer may lead DHBs in that region to approve administration of Avastin.

Norway

Background

The Norwegian health system is financed through taxation, income related employee and employer contributions, and out-of-pocket payments. All residents are covered by the National Health Insurance Scheme (Folketrygden, NIS), which is managed by the Norwegian Labour and Welfare Organisation (Arbeids- og velferdsvaltningen, NAV).

The overall responsibility for the health care sector in Norway lies at the national level with the Ministry of Health and Care Services (Johnsen, 2006). Responsibility for the provision of health care services has been decentralised with 431 local authorities at municipal level responsible for primary health care. In addition, hospital care was re-centralised in 2002 through the creation of four regional health authorities which control the provision of specialised health services provided by 31 health enterprises by the end of 2003 (i.e. local hospital trusts).

Policies on funding of licensed pharmaceuticals under the statutory system

Funding of new licensed medicines (including expensive drugs) is mandated by the founding law about Patients' Rights. The law stipulates that a patient is entitled to "necessary health assistance". The Ordinance for Priorities Act (paragraph 2) specifies these entitlements further, describing the criteria to be applied for decisions for funding medicines under the statutory system:

- the patient suffers a certain prognostic loss in length of life, or his/her life will be significantly reduced if health assistance from the statutory system is delayed;
- the patient's life will be positively affected by coverage through health insurance;
- the expected costs are reasonable in relation to the effect of the intervention.

The primary goal of national pharmaceutical policy in Norway as set out by a 2004/05 policy document (Frostelid et al., 2007) has been defined as to ensure appropriate use of pharmaceuticals, which is to be achieved as follows:

- medicinal products shall be used correctly, in both medical and economic terms;
- patients shall have secure access to effective medicinal products, regardless of their ability to pay for them;
- medicinal products shall have the lowest possible price.

The Norwegian Medicines Agency (NoMA), an agency subordinate to the Ministry of Health and Care Services (HOD) is the main body responsible for market authorisation, classification, pharmacovigilance, pricing and reimbursement of pharmaceuticals. The Ministry of Health and Care Services holds the general legislative authority. The pricing and reimbursement of pharmaceuticals in Norway is regulated through the Norwegian Act on Medicinal Products (1992) and the

Norwegian Act on Pharmacies (2000), complemented by further regulations of specific areas such as the regulations regarding medicinal products.

Decisions on the reimbursement of drugs under the Norwegian statutory system are based on three criteria: disease severity, service utilisation (annual use of services) and patient type (low income, children). Decisions are further to be based on two fundamental principles related to solidarity and rationality:

1. Everyone should have the same access to necessary pharmaceuticals regardless their ability to pay.
2. The reimbursement system should encourage clinically rational and cost-effective use of pharmaceuticals as a tool to ensure investment in health care services.

Using these criteria, NoMA groups pharmaceuticals into the following reimbursement categories:

- general reimbursement (schedule 9);
- reimbursement on an individual basis (schedules 2 and 9a), where a patient applies for the reimbursement;
- reimbursement for treating serious infectious diseases.

For patients whose treatment involves pharmaceuticals that do not qualify for general reimbursement (category 1 as above) the Norwegian Labour and Welfare Organisation (NAV) will decide, upon application by the patient, whether or not reimbursement will be granted (category 2 as above). In-patient pharmaceuticals are covered by public hospitals and do not require patient co-payment (Frostelid et al., 2007).

Pharmaco-economic evaluation has been mandatory in Norway since 2002. While a drug can obtain market authorisation and a maximum price without pharmaco-economic evaluation, such evaluation is necessary for all pharmaceuticals for which an application for reimbursement under the statutory system has been submitted (Box 7). There are however two exceptions where pharmaceuticals are excluded from a pharmaco-economic evaluation: (1) pharmaceuticals with the same active ingredient as pharmaceuticals for which reimbursement has already been granted; and (2) pharmaceuticals where a new formulation clearly does not change the costs and health outcomes.

Furthermore, if an application for inclusion of a drug under the statutory system concerns a new chemical entity, a new combination or an extension of an indication, and if either (a) the expected annual sales value exceeds 100% of the expenditure on the relevant disease section of the reimbursement list or (b) the annual incremental fiscal impact of approving the application exceeds NOK 5 million (approx. GBP 480,000; EUR 600,000) by the fifth year following approval, NoMA has to reject the application and pass it on to the Ministry of Health and Care Services (HOD) which will consider the matter further (Frostelid et al., 2007).

The Norwegian Medicines Agency (NoMA) may be advised by an external reimbursement committee (National Advisory Committee for Drug Reimbursement) on issues pertaining to the application (i.e. verification of documentation, severity of disease, clinical criteria). For applications passed on to the Ministry of Health and

Care Services (HOD), the HOD may consult the National Council for Health Care Priorities to confirm whether the projected funds 'would be well spent' in comparison to other health challenges. The Ministry may decide to reject the application following further evaluation. However, should it support the approval, it will have to bring the case before Parliament.

Box 7 Principles of pharmacoeconomic evaluation of drugs in Norway

There are few criteria for an application when pharmacoeconomic evaluation is performed. However, the evaluation should make a case for reimbursement. Normally this is achieved by a cost-effectiveness ratio analysis. There is no cut-off ratio determined in Norway. An estimate of the impact on the pharmaceutical budget is required. This means that the pharmacoeconomic analysis should be undertaken from both a societal perspective and the perspective of the payer. In addition, the economic consequences which the illness and any interventions will have for the Norwegian society as a whole and the National Insurance Scheme should be clearly explained throughout the process.

Access to new and high-cost medicines

The Norwegian health authorities are responsible for reviewing new and expensive drugs as soon as possible and indicate their use under specific guidelines communicated to the respective health enterprises. This has however been challenging because of ambiguities in the decision-making process and financing procedures of new and expensive drugs in secondary care. Also, the decentralised nature of the Norwegian health system which has strengthened the role of the regional health authorities has caused regional variation regarding the availability of new and expensive drugs and consequently raised equity issues.

Policies in relation to patients' ability to 'top up' their care under the statutory system

Patients are not able to 'top up' the treatment they receive under the Norwegian statutory system. On several occasions, patients have decided to seek private treatment abroad (cancer patients are a typical example, since there is no private out-patient clinic in Norway), and then sought to receive a refund for their expenses from the Norwegian statutory system. However, no such refund has been granted.

Spain

Background

The Spanish national health system (*Sistema Nacional de Salud*, SNS) offers universal coverage for all residents and provides publicly funded and delivered health services, mainly financed through national taxation. Services are free at the point of use; the only exception is specific non-refundable co-payments for prescription drugs.

National legislation sets out the principles of the SNS, such as the principles of universal coverage (including equal access to care) and of solidarity of public financing. Most funding for publicly financed health care is centrally allocated, through the central tax agency (*Agencia Tributaria*). Since 2001, regions are permitted to levy additional regional taxes for health care, for example through a regional ‘health cent’ on petrol. However, their contribution to public health care financing is small.

Responsibility for organising publicly funded health care largely rests with the 17 regions (Autonomous Communities) (Habl et al., 2006). Regions have their own basic law (Statute of Autonomy), parliaments and governments, and develop regional legislation (Duran et al., 2006).

Policies on funding of licensed pharmaceuticals under the statutory system

Drug policy in Spain (including drug approval, pricing and reimbursement decisions) is one of the few policy areas which remains centralised despite the devolution of power to the Autonomous Communities to manage their own health systems. Thus, decisions regarding the public funding of new drugs (which have already been approved through the established mechanisms) are made at the national level by the Spanish Ministry of Health, in particular the *Dirección General de Farmacia y Productos Sanitarios* (DGF) within the Spanish Ministry of Health.

Ultimately, it is the Director General of the DGF who signs off the decision to accept or deny the public funding of a new drug. Decisions on funding and price setting are made jointly, although for the latter the DGF follows the advice of the Interministerial Commission on Drugs Prices (*Comisión Interministerial de Precios de Medicamentos*), an interdepartmental committee for setting drug prices involving representatives from the Spanish Ministry of Health and Consumer Affairs, the Ministry of Industry, and the Ministry of Finance.

The DGF evaluates the effectiveness of the new drug and assesses its ‘therapeutic value’. The DGF also assigns the drug to one of the following four therapeutic groups:

- Class H drugs: reimbursed by the Spanish NHS at 100%. These are administered at the hospital; they include most of the oral cancer drugs;
- Class R drugs: reimbursed at 90%. This group includes drugs for chronic diseases and some cancer drugs;
- Class N drugs: reimbursed at 60%;

- Non-reimbursable drugs: the ‘negative list’.

Any drug treatment has to be authorised and supervised by a health professional (mainly a doctor). In the case of cancer drugs, an oncology specialist (medical oncologist, haematologist or radiation oncologist) has to authorise its use (including non-reimbursable drugs which are purchased by the patient at the retail pharmacy).

Exclusions of licensed drugs from reimbursement/subsidy under the statutory system

In general, the Spanish national health system has been very generous in terms of public funding of new drugs. There are a number of drugs and health products which are excluded from public funding and require patients to pay the full cost. These are included in a ‘negative list’. The negative list was first introduced in 1993 and tends to include drugs with low therapeutic value or products used for treating minor conditions (e.g. laxatives, vitamins). However, for life-threatening diseases such as cancer, new drugs tend to be always publicly funded.

Box 8 Access to high-cost drugs in Spain in the public debate

In March 2007, the Spanish Society of Medical Oncology (*Sociedad Española de Oncología Médica, SEOM*) published a very critical report ('Barreras de Acceso al paciente de los fármacos oncológicos') which denounced problems of access for patients to new cancer drugs and risks of a postcode lottery as health care management has been devolved to the Autonomous Communities. The Spanish Ministry of Health denied the claim but nevertheless announced measures to speed up the process of new drugs accessing the Spanish market and declared that it would reimburse most new oncology drugs approved by the European Agency EMEA. This was reported in the medical trade journals at the time but there have not been any subsequent (nor more recent) discussions on this matter.

However, the subject does not seem to be an issue any more. In September 2008, a study presented at the annual conference of the SEOM reported the situation with a much less critical tone. Despite some barriers allegedly experienced by patients when trying to access new oncology drugs, Spain is considered to be one of the countries in the EU in which the authorising and marketing of new medicine is fastest.

Policies in relation to patients’ ability to ‘top up’ their care under the statutory system

Out-of-pocket payments for pharmaceuticals are common in Spain. A drug not publicly funded (therefore included in the negative list), but allowed to be marketed in Spain can be purchased by patients. It does however require the prior authorisation and prescription from a practitioner. Such decision to go private does not expel patients from the Spanish NHS, i.e. patients paying for a drug to treat a particular condition remain within the statutory system. Patients are always covered by the statutory health system, regardless of their decision to pay privately for drugs or to receive complementary private treatment.

In cases where the patient is allowed to pay for a drug privately it is the Spanish NHS which takes clinical and financial responsibility for supervising the administration of the drug and for monitoring any side-effects. In case of a cancer patient, the NHS oncologist would therefore administer the drug and monitor the side-effects.

It is indeed common for a patient to ask a doctor to prescribe a treatment (e.g. a drug) so it can be funded by the statutory system, although the treatment has been

provided by a doctor practicing privately. On many occasions it is the same doctor who decides on the treatment and prescribes it – the former on her/his private basis, the latter as a public consultant (see example Box 9). For most people, this is not perceived as misconduct or inappropriate use of public facilities.

Box 9 Vignette: Combining public and private treatment in the Spanish system

A patient (a woman aged 64 years) was referred to a specialist by her general practitioner and subsequently diagnosed with pancreatic cancer at the local hospital. As the cancer was quite advanced (stage 4) and had spread to intestines and spleen, the local surgical team informed her that they could not operate on her. However, the patient underwent surgery to remove the cancer at a private hospital by a leading surgeon two weeks after diagnosis. She then had ‘adjuvant’ chemotherapy back at the local hospital under the supervision of the local oncologist, who liaised with the surgeon and who had full access to her surgery report.

Sweden

Background

The Swedish health system is of a Beveridge type, with counties and municipalities as providers of care. Health care financing is predominantly through taxes at the regional and local level, occasionally supplemented by earmarked grants from the national government; all residents are covered and there is no substitutive private coverage available. A mandatory national-level social insurance system covers sick leave and pensions; it is funded through payroll taxes and administered by the State.

Under the Health and Medical Services Act (1982), the 18 county councils, two regions and 290 municipalities in Sweden are responsible for the financing, organisation and provision of health care and medical services, and for public health services for all residents who are entitled to use the services at subsidised prices. The counties are responsible for primary health care; they own, finance and run the acute care hospitals, including psychiatric care. In addition, financial responsibilities for drugs have gradually been transferred from the state to the counties, although, state subsidies for drugs are still substantial. Municipalities are financially and organisationally responsible for the provision of all forms of nursing care for persons above the age of 65, and also for chronic psychiatric care (Karlberg, 2008)..

Policies on funding of licensed pharmaceuticals under the statutory system

Pricing and reimbursement of pharmaceuticals in Sweden are regulated by the Act on Pharmaceutical Benefits (2002). The Medical Products Agency (*Läkemedelsverket*, MPA) is the Swedish national authority responsible for the regulation and surveillance of the development, manufacturing and sale of pharmaceuticals and other medicinal products (Redman and Hoggard, 2007). The Pharmaceutical Benefits Board (*Läkemedelsförmånsnämnden*, LFN), established in 2002, is the national body responsible for pricing and reimbursement decisions in relation to pharmaceuticals for out-patient care. Pharmaceutical suppliers apply to LFN for their products to be included in the reimbursement system at a set price. LFN then decides which drugs are accepted or rejected for reimbursement under the statutory system. If a product is rejected, the industry may resubmit an application for the same product at a lower price. Both the MPA and LFN are independent authorities answering to the Ministry of Health and Social Affairs.

Decisions about public reimbursement of services are based on three principles of priority setting in health care, as determined by the Swedish Parliament in 1997 (Ettelt et al., 2007):

- The principle of human dignity: health care shall be provided in a spirit of respect for the equal value of all human beings.
- The principle of need and solidarity: those with the greatest medical need take precedence over those with less severe conditions as it relates to health care resources, for example the reimbursement of drugs.
- The cost-effectiveness principle: the costs for using a medicine should be reasonable from a medical, humanitarian, and socioeconomic perspective.

All reimbursed outpatient drugs are included in the Pharmaceutical Reimbursement Scheme (a positive list of drugs) which is regularly updated by LFN (Redman and

Hoggard, 2007). If a drug is accepted by the LFN, subsidy is mandatory and the cost will be covered by the county in which the patient resides with co-payments applying. User charges, which are gradually decreasing until a ceiling is reached, are set nationally. However, for in-hospital drugs, the counties decide on the use. This will cover most of the new and expensive drugs.

Access to high-cost medicines

Access to expensive new drugs (especially cancer drugs) has received considerable media attention due to the differences in the availability of these products among the counties. Such differences are the consequence of the decentralised character of the Swedish political system, which enables counties to set their own priorities. For example, if a county employs more oncologists, more cancer drugs will be prescribed and used.

Cost-effectiveness is an important factor in evaluating new expensive drugs, and the norm is that drugs with low cost-effectiveness are generally not reimbursed under the statutory system. Ultimately, decision-making about whether to not fund and use a new expensive licensed drug under the statutory system will be made by the clinicians within a clinical department, a hospital and a county. Clinicians ought to take into account opportunity costs when they make their decisions. The decision not to use drug-eluting stents is one example. For cancer drugs the approach tends to be one of dilution rather than not to using the drug at all. However, evidence-based national guidelines and regional clinical practice guidelines are aimed at assisting clinicians in their decision-making.

Policies in relation to patients' ability to 'top up' their care under the statutory system

Within the Swedish statutory health system, patients cannot purchase drugs privately. However, patients may access drugs (mainly out-patient) which have market approval but that are not part of the pharmaceutical reimbursement scheme (for example expensive cancer drugs) privately by paying the full price out-of-pocket. This usually is the case for a small part of the Swedish population: there are private providers, who deliver services to a very small group of wealthy people, or people who have private health insurance (a small percentage of all cancer patients). In addition, cancer patients may choose to receive treatment abroad (e.g. in Germany), where alternative treatments may be purchased, including expensive drugs.

A single patient in need of an expensive life-prolonging drug will practically have unlimited access within the public system without any 'top-ups'. 'Top-up' of treatments within the statutory system is generally not permitted.

There are however borderline cases which could be interpreted as 'topping-up' care under the statutory system. For example, in 2007, the Pharmaceutical Benefits Board decided to subsidise HPV vaccination for girls aged 13-17 years in Sweden. However, at the time of writing, it had not yet been decided, at the national level, whether the HPV vaccine should be part of the national vaccination package and provided free of charge. In the meantime, the vaccine is available for private purchase for the targeted population group which will then be administered at the Primary Care Centre.

In general however, the combination of county-provided care and a private drug is not possible for patients in the Swedish public system. 'Topping-up' is not a political issue; if the use of a drug is evidence based and within acceptable cost-utility limits, it should be used and provided by the system; if not, it should not be used.

Switzerland

Background

The 1996 Swiss Law on Health Insurance (LAMal) stipulates that all Swiss residents purchase basic health insurance which covers a basket of goods and services defined at the federal level. Private health insurance held by the majority of the population (around 80%) covers additional services to the basic basket of services funded under the statutory system (Paris and Docteur, 2007b). There are several cost-sharing arrangements within the Swiss mandatory health insurance with all individuals contributing to the cost of health services through a deductible, co-insurance and co-payments.

Switzerland is a confederation of 26 Cantons, characterised by a high level of political decentralisation. Cantons have their own constitution, parliament, government and courts. The federal government legislates in areas such as public health, social insurance, professional qualifications and others, while the Cantons are responsible for disease prevention and health education and for the provision of health care. They also partially finance hospital costs (European Observatory on Health Care Systems, 2000).

Policies on funding of licensed pharmaceuticals under the statutory system

The Swiss universal basket of goods and services covered under the basic insurance includes drug coverage (*Ordonnance sur les prestations dans l'assurance obligatoire des soins en cas de maladie*, OPAS). Basic health insurance covers drugs included in a positive list when dispensed in community pharmacies, hospital pharmacies or by doctors.

The Swiss Agency for Therapeutic Products (Swissmedic) is responsible for registration and market-entry authorisation of pharmaceuticals and medical devices. Its duty is to assess and certify that drugs and medical devices entering the Swiss market are of high quality, safe and effective. Following market authorisation, the manufacturer may apply for inclusion of the drug in the positive list of reimbursed products (*Liste des spécialités*, SL) to the Federal Office for Public Health (OFSP) which decides whether a drug is eligible for reimbursement, as well as its maximum reimbursement price.

The principles of inclusion of newly licensed drugs in the positive list are established by ordinances issued by the Federal Council (i.e. the Swiss Government) and the Department of Home Affairs. The Swiss authorities do not carry out their own cost-benefit analysis but they require manufacturers to provide evidence of effectiveness and therapeutic value and that the drug offers value-for-money. However, the Federal Office of Public Health does undertake comparative assessments with other countries (external reference) as well as with equivalent products on the therapeutic level. If a given agent or drug is claimed to be innovative, it must demonstrate improved effectiveness or a better risk-profile (i.e. fewer undesirable side-effects). The assessment of effectiveness must be based on controlled clinical trials for allopathic drugs, and the Federal Drug Commission is expected to draw on Swissmedic's work on assessing effectiveness. However, it may require additional data (Paris and Docteur, 2007b).

Once a drug has been included in the positive list (established by the Federal Office of Public Health) and reimbursement decisions, informed by the advice of the Federal

Drug Commission) have been made, insurers reimburse the price of the drug, minus the required user charges (deductibles and co-insurance) (Box 10).

Box 10 User charge arrangements for pharmaceuticals in the Swiss mandatory health insurance system

Deductibles: there are 6 possible levels:

- Adults: 300, 500; 1 000; 1 500; 2 000; 2 500 CHF per year
- Children: 0, 100; 200; 300; 400; 500; 600 CHF per year

Co-insurance: 10% of the cost of health goods and services beyond the value of the deductible

Co-payments: for each in-patient day, 10 CHF per day

Ceiling: the 10% co-insurance may not exceed an annual cumulated individual amount of 700 CHF for adults and 350 CHF for children

Since January 2006, co-insurance requirements have been adjusted to encourage uptake of generics instead of branded drugs for which there are generic substitutes.

There is generally no difference in terms of cost-sharing arrangements for drugs within ambulatory care and hospital settings for the services covered by the mandatory health insurance. Thus, drugs used for in-patient care are funded by basic health insurance through hospital payment schemes defined in each Canton. Patients may have to contribute to the total cost of their hospital stay, but they are not invoiced separately for the cost of the in-patient drugs. In principle, when a hospital stay is not covered by basic health insurance, either because the patient has private health insurance or because the hospital stay is not covered by the basic health insurance, the hospital produces an itemised bill, and may charge drugs at any price. However, private health insurance companies which provide supplementary schemes tend to sign agreements with hospitals including price arrangements.

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Annex

Questionnaire: Right to pay to ‘top up’ treatment under the statutory system

The issue

The advent of new and expensive drugs, especially for the treatment of cancer, creates challenges for health systems about whether and how to make these new treatments available under the statutory system given inevitably limited resources. While these new treatments have the potential to be effective for some individuals, through for example extending their life by months or even years, their overall cost-effectiveness may be questionable and health systems may decide not to pay for these under the statutory system. Yet, patients may still wish to access these drugs and be willing to pay out of pocket to have these (i.e. to ‘top up’ their care under the statutory system).

Effectively, current legislation in England stipulates that patients cannot ‘top up’ their NHS care with additional private treatment. Specifically, a patient cannot be both a private and a NHS patient for the treatment of one condition during the same visit to an NHS organisation. Applying this rule has led to a small number of cases where patients who chose to pay out of pocket for treatments that are not funded under the NHS were then considered private patients and denied all of their NHS care for the specific condition, thereby forcing them to pay for all their treatment for that condition privately.

We attach three case vignettes of (hypothetical) cancer patients in England to illustrate this situation.

Our request

The Department of Health is reviewing this arrangement and is interested to learn how ‘topping up’ is handled in other countries. Specifically we are interested to learn:

1. What is the policy, in your country, on deciding on whether or not to fund new (but expensive) licensed medicines under the public/statutory system?

2. Has your country ever decided to not fund an appropriately licensed drug under the public/statutory system? (Please provide an example if appropriate)

3. What were the consequences of this decision?

4. What is the policy, in your country, for patients who choose to pay privately for drugs that are not funded within the statutory health system?

5. If a patient is allowed to pay for a drug to treat a particular condition privately, will s/he still remain a patient within the statutory system for treatment of that condition?

6. Is it possible for a patient to receive, in a single visit to one institution, a simultaneous administration of both private and public (statutory) drugs or treatment?
 7. If a patient is allowed to pay for the drug privately, who takes clinical responsibility for supervising the treatment and who pays for the clinical time required to administer the drug and monitor side-effects, etc.?
 8. If possible and relevant, please use one (or more) of the three clinical vignettes attached to this email to illustrate what would have happened in your country. What would have been the same or similar, and what would have been different?
 9. What are the consequences of allowing patients to pay out of pocket to top up what is provided by the statutory health system? (e.g. does it encourage the use of low value drugs, increase drug use, lead to perceptions of an inequitable system, controversy about responsibility and funding etc?)
10. Is the issue of 'topping up' a matter of policy concern in your country?