Review

Decision-making in priority setting for medicines—A review of empirical studies

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Abstract

Rising pharmaceutical expenditure leads to an increased need for priority setting in medicinal care. The objective of this paper is to review studies that empirically analyse a macro- and meso-level decision-making process for including drugs in and/or excluding drugs from reimbursement lists and drug formularies in industrialized countries. We identified six separate studies analysing a decision-making process as a whole. According to them, the most important groups in decision-making were experts and administrative persons. The decision-makers had an explicitly or implicitly defined set of criteria that were considered in decision-making, with clinical evidence on the benefit and the costs being the main criteria used. However, formal pharmacoeconomic analyses were given a rather small role. The criteria used varied between studies, and also between decisions. The decisions seemed inevitably to be partly value-based in their nature, as the scientific or other exact evidence did not give a firm foundation on which the decisions could be solely based. The majority of the studies concentrated on descriptive analysis on how things are rather than on explicitly analysing how decision-making processes perform against defined principles or goals. To facilitate decision-making by clearly defined principles and methods, more analytic studies on decision-making are especially needed.

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Keywords: Resource allocation; Decision-making; Priority setting; Drug formulary; Pharmaceuticals

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0168-8510/5 – see front matter © 2007 Elsevier Ireland Ltd. All rights reserved.
doi:10.1016/j.healthpol.2007.09.007
1. Introduction

Pharmaceutical expenditure is rising as new drugs are developed for diseases for which there was no previous drug therapy and the price of new drugs are relatively higher. Cost containment can be partly achieved by price regulation, co-payments, physician-level drug budgets, reference pricing and generic substitution. However, it is increasingly clear that cost containment cannot be achieved without more or less explicit priority setting for drugs.

In the registration of a medicine, the costs and cost effectiveness are not usually assessed and therefore priority-setting decisions are now made by a separate process. Health care systems have varying methods to prioritise drugs at a national or institutional level. These methods include for example negative and positive drug lists, clinical guidelines, and reimbursement restrictions for patient subgroups. Several industrialized countries have national-level decision-making processes to select the outpatient drugs to be covered by the public health care system (for example Australia, Finland, France, New Zealand and Sweden). In the hospital sector, it is common that hospitals have committees that decide about the inclusions of drugs into the hospital drug formulary [1–4]. However, there is a considerable variance in how strictly clinicians have to comply with the formulary.

The search for good methods in priority setting in health care has shown that there are no simple solutions. A complete and non-contradictory set of rational decision rules alone does not solve the problems encountered in priority setting [5]. Priority setting in health care is partly subjective and value-based in nature and perhaps stakeholders other than experts are also needed in decision-making [6]. A solution is to combine different approaches where both explicit and implicit methods are used as complementary tools [7].

The objective of this paper is to review studies that empirically analyse a macro- and meso-level decision-making process for including drugs in and/or excluding drugs from reimbursement lists and drug formularies in industrialized countries. These studies explored questions such as what technical methods are used, who are the decision-makers, what are the criteria behind the decisions and what are the procedural frameworks for decision-making. This summary of the key findings of the studies on existing decision-making processes discloses similarities and differences between the processes, which can be useful for guiding the development of priority-setting processes in a more rational and fair direction [8] and for directing future research.

2. Materials and methods

We searched for empirical studies published in English that explored the decision-making process in the priority-setting of medicines either in outpatient or inpatient settings in industrialized countries. MEDLINE was searched from 1990 to February 2007 with the following keywords: “pharmaceutical preparations” or “pharmaceutical services” or “pharmacy and therapeutics committees” or “pharmacy administration” or “formularies” or “drug costs” or “drug approval” and “health care rationing” or “health priorities” or “health policy” or “policy making” or “decision-making”. In addition we checked all the references from the relevant articles found.

In order to focus on the analyses of actual decision-making processes and to gain the best insight into the decision-making process as a whole, we included only qualitative studies or studies that focused on one decision-making body. We excluded surveys that did not analyse the actual processes, such as surveys targeted to a large number of hospitals, purely descriptive articles that did not include systematic data gathering and analysis, and studies concentrating on a single decision-making criterion such as pharmacoeconomic analyses.

The review was made qualitatively as the majority of the qualitative studies. The estimated relative importance of the decision-making criteria in the studies is based on our interpretation of the study results.

3. Results

For the review we identified six original research articles. Three of those six studies were conducted in Canada, and the rest were conducted in the United Kingdom, France and Finland (Tables 1 and 2). Four studies concerned drugs to be used in outpatient care and two concerned a hospital context.

A qualitative study from Canada [9] explored decision-making in a Drug Quality and Therapeutics
Table 1
Description of the studies

<table>
<thead>
<tr>
<th>Study</th>
<th>Setting</th>
<th>Country</th>
<th>Objectives</th>
<th>Materials and methods</th>
<th>Decision-makers</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. PausJenssen et al. [9]</td>
<td>Outpatient</td>
<td>Canada</td>
<td>To describe how listing decisions are made, and specifically the role of pharmacoeconomic analysis</td>
<td>Meeting transcripts (9) and interviews with members (7) of the committee</td>
<td>A provincial level committee including experts and government employees</td>
</tr>
<tr>
<td>2. Martin et al. [10]</td>
<td>Outpatient</td>
<td>Canada</td>
<td>To describe the rationales used by a committee making funding decisions for new cancer drugs</td>
<td>Interviews of committee members (11), observation of meetings (12) and documents produced</td>
<td>A provincial level committee including administrators, experts, patients and members of the public</td>
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<tr>
<td>3. Le Pen et al. [13]</td>
<td>Outpatient</td>
<td>France</td>
<td>To analyze the concepts and methods used in drug evaluation in the drug reimbursement system</td>
<td>Statistical analysis of classification system used to evaluate new drugs</td>
<td>A national-level committee including administrators and experts</td>
</tr>
<tr>
<td>4. Vuorenkoski et al. [14]</td>
<td>Outpatient</td>
<td>Finland</td>
<td>To examine stakeholders’ perspectives on prioritisation decisions made in the national drug reimbursement system</td>
<td>Interviews of key informants (18)</td>
<td>Administrators and experts in a governmental system</td>
</tr>
<tr>
<td>5. Martin et al. [15]</td>
<td>Hospital</td>
<td>Canada</td>
<td>To describe the process of priority setting for new drugs in a hospital formulary and evaluate it using “Accountability for Reasonableness” framework</td>
<td>Minutes of committee meetings (20), interviews of key informants (18) and observation of meetings (3)</td>
<td>A committee including experts</td>
</tr>
<tr>
<td>6. Jenkings and Barber [17]</td>
<td>Hospital</td>
<td>UK</td>
<td>To determine what was considered as evidence and how it was used in decision-making</td>
<td>Observation and tape recording of committee meetings (8)</td>
<td>Two committees including experts</td>
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<tr>
<td>Study</td>
<td>Important criteria in decision-making</td>
<td>Expenses</td>
<td>Conclusions</td>
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<tr>
<td>1. PausJenssen et al. [9]</td>
<td>Clinical benefit, costs, quality of data and past decisions</td>
<td>More higher the costs, more carefully evaluated; pharmacoeconomic analyses seldom used</td>
<td>Pharmacoeconomic analyses had a limited role</td>
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<td></td>
</tr>
<tr>
<td>2. Martin et al. [10]</td>
<td>Clinical benefit was most important criteria; number of patients, costs, quality of evidence, access to treatment, pressure from physician and patient groups and past decisions</td>
<td>Value-for-money was considered, but formal pharmacoeconomic analyses was not used; predefined amount of money to spend</td>
<td>Rationales varied according to decision and involved a cluster of criteria</td>
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<tr>
<td>3. Le Pen et al. [13]</td>
<td>Efficacy and disease severity</td>
<td>No economic considerations in the classification system</td>
<td>Economic and public health aspects of drugs should have more impact when deciding which drugs should be publicly covered</td>
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<tr>
<td>4. Vuorenkoski et al. [14]</td>
<td>Scientific evidence and budget impact</td>
<td>Budget impact and price of the drug were very important criteria in decision-making</td>
<td>Decision-making process is not transparent; unofficial stakeholders have many ways to influence decision-making</td>
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<td>5. Martin et al. [15]</td>
<td>Benefits, adverse effects, quality of evidence, number of patients requiring the drug, availability of alternative drugs, drug use in other hospitals and costs of the drug</td>
<td>No formal pharmacoeconomical analyses, but informal cost-comparisons</td>
<td>Evaluation using “Accountability for Reasonableness” framework led to several recommendations to improve the fairness of priority setting</td>
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<tr>
<td>6. Jenkins and Barber [17]</td>
<td>Clinical trial data and costs most important; patient demand, pre-existing prescribing of the drug, company activities, clinicians excitement and decisions in other hospitals</td>
<td>Costs were considered, but high costs were not a sufficient reason alone for rejection</td>
<td>Both abstract scientific rationality and the local rationality of practical health care provision were present in the decisions</td>
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Committee (DQTC) working at the provincial level, which makes recommendations for listing drugs in a positive list for the Ontario Drug Benefit Program. The committee included eight physicians, one pharmacist, one pharmacologist and two government employees (12 members). Ontario has required a pharmacoeconomic evaluation from the applicants from 1996 onwards. The study found that central to all decisions was the perceived clinical merit of the product. The committee spent the largest amount of time in discussing clinical claims made by the manufacturer. The quality of the data was evaluated carefully and the committee often believed that manufacturers overstated the benefits.

Only after considering the clinical merits was the cost of the product considered. Formal pharmacoeconomic analyses were rather seldom used, partly because of the poor quality of the analysis and the committee members’ lack of knowledge of pharmacoeconomic analysis. However, the committee did discuss economic issues and often performed informal economic analyses. The data were examined more carefully if the costs for the program were expected to be high. The use of value judgements in the decision-making was not explicit, and if present, judgements came ad hoc from individual committee members.

The second study from Canada assessed the decision-making of a committee deciding which cancer drugs should be funded at the provincial level through a new public program in Ontario [10]. The same data are used also in two other papers including additional data from a committee that was planning the use of some specific acute cardiac care treatments [11,12]. The committee contained administrators, oncologists, oncology researchers, a pharmacist, an ethicist, patients and members of the public (15 members). It had a pre-defined amount of money to spend on new cancer drugs, but more money could be negotiated. The committee did not use formal pharmacoeconomic analyses in its decision-making, although the value-for-money concept was used. The clinical benefit of the drug had the most important role in the decision-making. In addition to this, the number of patients, costs, quality of evidence, access to treatment, pressure from physician and patient groups and past decisions influenced the decision-making. The study found that decisions were not based on any individual criterion, but on changing the combination of several criteria, which varied according to, for example, the cost of individual treatment or size of the budget. One strategy in the decision-making was to compare previous and current decisions.

Le Pen et al. [13] analysed priority-setting decisions in France. After licensing, the Transparency Commission (TC) assesses whether a drug should be included in the positive list of the reimbursement system. The committee includes representatives of government, public insurance funds, physicians and other experts. From 1999 onwards the TC has defined the “medical service rendered” (MSR) classification to describe the value of the drug (low, moderate, important). MSR is defined by evaluating seven specific dimensions of the drug (severity of the disease, efficacy and safety, nature of treatment, place in therapeutic strategy, existence of alternative treatments, public health value, and conditions of use). This system was designed to make the decision-making more objective and based on explicitly stated criteria. Le Pen et al. analysed how different dimensions predicted the final decision. They found that efficacy and disease severity had a major impact on decisions and public health value and economic considerations had little or no impact. In addition, there were significant differences between therapeutic classes in dimensions best predicting the decisions. The authors’ conclusion was that the economic and public health aspects of drugs should have more impact when deciding which drugs should be publicly covered.

Vuorenkoski et al. [14] analyzed the decision-making process in the Finnish drug reimbursement system from the perspective of stakeholders. This study examined the decision-making process to select drugs for the higher reimbursement category, which would give a higher monetary compensation to the patient. The results revealed that the process was much more complicated than that stated in legislation. Even though the decisions formally were made by the Government, they resulted from a complex interplay between administrators, experts, and politicians in a non-transparent process. Administrators tried to make decisions based on scientific evidence alone. However, achieving this was not an easy task, and there appeared to be hidden non-scientific criteria behind the decisions, such as the budget impact of the decision. Stakeholders outside the public administration – such as pharmaceutical companies, patients and the public – had few opportunities to openly participate in decision-making. Despite
this they tried to influence decisions concerning their interests through several methods, such as lobbying the media, the parliament or other stakeholders.

Two of the studies analysed decision-making in a hospital setting. The first study explored decision-making on including drugs in the drug formulary in a network of three teaching hospitals in Canada [15]. In these hospitals an expert committee (consisting of 14 physicians, two pharmacists and one nurse from three hospitals) made recommendations to the Medical Advisory Council (MAC) on which drugs to include in the drug formulary. Proposals for the drugs to be listed could be made by physicians and pharmacists in these hospitals. The study evaluated the decision-making process using an “Accountability for Reasonableness” framework developed by Daniels and Sabin [16]. According to that framework, fair and legitimate priority setting decisions can be achieved by satisfying four conditions: the rationales behind decisions are publicly accessible; rationales are accepted as relevant and reasonable; there is an opportunity to appeal the decisions; and there is regulation to ensure that the above conditions are met.

The study revealed that in practice the expert committee had a significant influence on the final decisions, because the MAC rarely rejected the committee’s recommendations. The decisions of the expert committee were based on a cluster of criteria such as benefits, adverse effects, quality of evidence, number of patients requiring the drug, availability of alternative drugs, drug use in other hospitals and costs of the drug. Formal pharmacoeconomic analyses were seldom used. Lay-people did not participate in the decision-making, although several interviewees thought it to be necessary. In the light of the “Accountability for Reasonableness” framework the authors found several problems in decision-making: the goals of the committee were not clear (especially the question on cost considerations), grounds for decisions were not made public, and in practice there was no opportunity for appeal.

The second study analysed the activities of drug and therapeutics committees in two British hospitals [17]. The committees contained clinicians from a range of specialties, pharmacists, members from nursing, and in one committee, a financing officer. The study explored what the committee members considered as evidence as well as how it was used in decision-making to include drugs in the formulary. The authors found that clinical trial data and costs were the most important criteria in decision-making. In addition to this, patient demand, pharmaceutical company activities, pre-existing prescribing of the drug, clinicians’ excitement and decisions in other hospitals influenced the decision-making. Although costs were an important theme in discussions, it alone was not a sufficient reason to reject a drug. The authors concluded that both the formal rationality of science and the local rationality of health care should be recognised, and there should be more open discussion on the different sources and types of evidence used in decision-making.

3.1. Synthesis of results

According to the studies the decision-makers have explicitly or implicitly defined the set of criteria used in the decision-making process. In addition, some external factors can be seen to influence the decision-making. The criteria used varied between decision-making processes, and also between the decisions of a single decision-making process. For example the costs and the type of drug can be seen to have influenced what other criteria were used in decision-making.

According to the review, the clinical evidence on benefit and the quality of that evidence were the main criteria used in priority setting concerning medicines. In some cases the clinical benefit was explicitly divided into efficacy and adverse effects. The costs of the drug emerged as the second major criteria in the decision-making. Cost containment and keeping the budget balanced is supposedly in the background of every decision-making system explored in the studies. However, only one study [10] indicated that the decision-makers had a predefined amount of money to cover drug expenses, which explicitly sets cost as an important criterion in decision-making. Formal pharmacoeconomic analyses had a rather minor role in decision-making. In some instances simpler comparisons of costs and benefits were used.

Other criteria used by decision-makers were alternative treatments available, decisions in other hospitals/systems, size of population affected, severity of disease and past decisions. External factors mentioned as influencing decision-making were patient demand,
pharmaceutical company activities and clinicians’ excitement.

In the decision-making processes analysed in this review the most influential stakeholder groups were experts, such as physicians and pharmacists. The second most influential were administrative persons. The interests of the other stakeholders were mediated more implicitly. Only one study found that lay-people were directly involved in decision-making [10–12]. In one study, inclusion of lay members in decision-making was proposed as one possibility to improve decision-making [15].

The transparency of the decision-making was discussed in three studies [11,14,15]. According to these studies the criteria and reasoning used in individual formulary decisions were not easily accessible to anyone else except the actual decision-makers. The conclusion was that greater publicity could improve priority setting by engaging all stakeholders in a discussion about the appropriate principles used in priority setting and by promoting accountability and democracy in decision-making.

Only one of the studies reviewed had evaluated the performance of the decision-making processes against predefined principles or goals, and it revealed several possibilities for how to improve the fairness of priority setting. The same framework has been used to make international comparisons of the fairness of four centralized drug review agencies [18]. That study also found many possibilities for improving the fairness of drug review processes.

4. Discussion

We found only six studies that matched our criteria. One reason for the lack for studies could be that qualitative studies on the subject are not easy to conduct as the majority of publicly funded health care systems would like to give the image that all necessary care is provided. Another reason is that decision-making can be influenced by factors which perhaps would not be easily accepted by the public or other stakeholders as such.

The review found that there is considerable variability in decision-making processes in terms of criteria used and decision-makers involved. The criteria found to be used in these studies have also been found in survey studies dealing with decision-making criteria related to drugs [19–21]. Clinical benefit as shown in clinical trials was the most important criterion. A considerable amount of scientific knowledge exists on medicines compared to other forms of therapy. However, there are some limitations in the use of clinical trial data in decision-making: trials may have too short a follow-up, they can have an inappropriate comparator or the sample size can be too small [20]. Furthermore, much of the evidence is created by pharmaceutical companies whose economic interests might cast doubt on the reliability or robustness of the scientific evidence they produce.

Formal pharmacoeconomic analyses could partly solve the conflict between the two major criteria of benefit and cost. The first countries to officially require pharmacoeconomic analyses before accepting a drug to the reimbursement system were Australia in 1993 and some provinces of Canada (Ontario and British Columbia) in 1994–1995 [22]. Later, for example, Finland and the Netherlands have started requiring pharmacoeconomic analyses when companies apply for their drugs to become reimbursable [23,24]. Some studies have found a linkage between actual drug coverage decisions and cost-effectiveness estimates [25,26].

However, according to this review pharmacoeconomic analyses had a limited explicit role in decision-making. Other studies have found, like this review, that decision-makers have many problems in using pharmacoeconomic data in decision-making [9,27,28]: problems in the quality of pharmacoeconomic evaluations, decision-makers’ poor knowledge of economic analyses and manufacturers’ use of economic analysis as a marketing tool. For example in Australia, 67% of pharmacoeconomic analyses provided by pharmaceutical companies to decision-makers in 1994–1997 had significant methodological problems [29] and in British Columbia, Canada, a majority of the pharmacoeconomic evaluations did not comply with existing guidelines [30].

Because the scientific and other exact evidence does not give a sufficiently firm foundation on which the decisions could be solely based, other partly subjective and value-based criteria can be rational and even legitimate from the perspective of real-life priority setting. As one of the analysed studies [17] stated, the criteria used in the decision-making could be divided into either a formal or a practical rationality. The formal rationality includes scientific evidence of
clinical benefit in addition to cost and budget considerations. The practical rationality includes criteria which in many cases are implicitly substituting for a lack of scientific evidence. It can for example take account of local situations and mediate the influence of different pressure groups. A study from the United Kingdom [31] focused on the lesser-discussed personal and political aspects in decision-making for medicines at the local level of the UK health care system. The authors concluded that these aspects should not be treated as contaminants of an otherwise rational decision-making process. But on the contrary the authors suggested that they seem to be relevant, reasonable and also of substantial importance in decision-making from the perspective of real-life decision-making.

It was suggested in some of the studies analysed that transparency could improve the quality of decision-making. There have been some developments in this direction already. Since 2003 short summaries of the decisions for including drugs into the Australian Pharmaceutical Benefits Scheme have started to be published on the internet [32], and the same type of policies have been adopted for example in Sweden, Canada and France also. Another possibility is that taking into account lay preferences – through public participation for example – could increase transparency and lead to more accountable decision-making [6,33–35]. However, there is no simple solution to the question of how the public should participate in practice.

The majority of the studies concentrated on descriptive analysis on how things are rather than on explicitly analysing how decision-making processes perform against some defined principles or goals. One such method is to evaluate the fairness and legitimacy of the decision-making through use of the “Accountability for Reasonableness” framework [16]. To facilitate decision-making by clearly defined principles, analytic studies on real-life decision-making on the priority setting of drugs are especially needed.

References


