Health services research at work for national health policy

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AT WORK FOR
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Thesis, Academic Medical Centre, University of Amsterdam

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HEALTH SERVICES RESEARCH
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NATIONAL HEALTH POLICY

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Faculteit der Geneeskunde
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Chapter 1

Introduction
Introduction

This thesis addresses the issue of how health services research can produce useful knowledge for policymakers. It is based on research carried out at the department of Social Medicine of the Academic Medical Center (University of Amsterdam) from 1998 to 2005 and at the Institute of Medical Technology Assessment of the Erasmus Medical Center (Erasmus University Rotterdam) from 2001 to 2004.

This introduction consists of five sections. Firstly, we describe the domain of health services research. Secondly, we address the relationship between health policymaking and health services research and focus on factors that influence the use of research by policymakers. Thirdly, we present the analytical model we use in this thesis and which incorporates four concepts: content, context, process and actors. This third section also presents the central research question. Fourthly, we introduce three cases of health services research for national health policy. These cases provide the empirical material for this thesis. Lastly, we describe the outline of the thesis.

Health services research

Health services research is an area of applied research that is multidisciplinary in nature and covers a wide range of topics in the field of health care [1;2]. There are many definitions of health services research that point to the same defining elements. Although the ultimate outcomes of health service utilization and provision are in the area of health, health services research often only analyses health care utilization or health service provision [3]. Health services research is usually policy-oriented and multidisciplinary. Some definitions point to the role of health services research in increasing the effectiveness of health care interventions or health care organization. Black et al. [4] emphasized the evaluation of advantages and disadvantages of health care interventions. Academy Health emphasized the identification of effective ways to organize, manage, finance and deliver high-quality care, reduce medical errors and improve patient safety. In sum, health services research is the multidisciplinary field of scientific investigation that studies how social factors, financing systems, organizational structures and processes, health technologies and personal behaviours affect access to health care, the quality and cost of health care and ultimately health and well-being [5]. In this thesis the focus is on health services research for national health policy.

Health policymaking and health services research

Like all policymakers, those who make national health policies need information for their policymaking process. Sometimes this information is scientific knowledge produced by health services research [6]. Health services research for national health policy aims to produce knowledge that policymakers can use in this process. We consider health services research to be a social process of scientific knowledge production. This process takes place in an environment that often measures research output by the number of publications in scientific journals, a number that is often a factor in the financial incentive structure for researchers. To
be considered for publication, the research results need to have a high level of generalizability. Although this emphasizes the generalizable content of a research topic, it does not automatically result in better national health policy. The strength of evidence can be a weakness when it comes to usefulness [6]. The road from research to policy is not one of consecutive steps whereby the final, generalizable product of health services research is a ready-to-use building block for national health policy [7]. Our view is that health services research and policymaking are often simultaneous processes taking place in interaction [8;9]. Consequently, to be effective, the interaction of the two processes needs to be anticipated and facilitated, especially since policymakers and researchers seem to have conflicting interests [10;11].

Both international [12-14] and national authors [15;16] have addressed policymakers’ use of information and knowledge as well as the interface between health services research and policymaking [9;17-20]. Innaer et al. summarized the facilitators and barriers for using research evidence in policymaking in 24 studies and surveys with health-policy decision-makers [14].

<table>
<thead>
<tr>
<th>Facilitators</th>
<th>Barriers</th>
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<tr>
<td>Personal contact between researchers and policymakers</td>
<td>Absence of personal contact between researchers and policymakers</td>
</tr>
<tr>
<td>Timeliness of research</td>
<td>Lack of timeliness of research</td>
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<tr>
<td>Relevance of research</td>
<td>Lack of relevance of research</td>
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<td>Good-quality research</td>
<td>Mutual mistrust, including perceived political naivety of scientists and scientific naivety of policymakers</td>
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<td>Research that confirms current policy or endorsed self-interest</td>
<td>Power and budget struggles</td>
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<tr>
<td>Community pressure or client demand for research</td>
<td>Poor-quality research</td>
</tr>
<tr>
<td>Research that includes effectiveness data</td>
<td>Political instability or high turnover of policymaking staff</td>
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In this thesis, although we do not test the work already done by many others, we do aim to contribute to the field of research that addresses the relationship between research and policy, including usefulness for and utilization of health services research by policymakers. We do this by presenting and reflecting on practical examples of knowledge production by health services research for national health policy. In presenting these examples, we focus on two of the above factors that influence the use of research by policymakers: the quality of research and the interaction between researchers and policymakers.

**A model for policy analysis ... and health services research**

This thesis is inspired by a model for policy analysis in health care – and more specifically, health sector reforms – provided by Walt and Gilson (1994) [21]. According to the authors, ‘The model is a highly simplified model of an extremely complex set of interrelationships, and gives the impression that each can be considered separately. In reality actors are influenced
(as individuals and as members of interest groups or professional associations) by the context within which they live and work, at both the macro-government level and the micro-institutional level. Context is affected by many factors such as instability or uncertainty created by changes in political regime or war; by neo-liberal or socialist ideology; by historical experience and culture. The process of policymaking (how issues get on the political agenda, how they fare once there) in turn is affected by actors, their position in power structures, their own values and expectations. And the content of policy will reflect some or all of the above dimensions.’ They argue ‘... that the traditional focus on the content of policy neglects the other dimensions of process, actors and context which can make the difference between effective and ineffective policy choice and implementation.’ [21] The model appreciates a specific content, but also identifies the context and processes that are related to this content. Furthermore, it positions actors in the centre of the model, indicating their central role.

**Figure 1.** A model for policy analysis. G.Walt and L. Gilson (1994)

This model for health policy analysis can also include health services research itself as information used by the actors. If health services researchers understand policy and the policymaking process, they can produce more useful information for policymakers [10;22]. Likewise, policymakers will be better informed if they understand a specific health services content – e.g. the cost effectiveness of an intervention or the design of a new instrument – within its context and processes and with its actors.

The emphasis on generalizable content by researchers described above is often paralleled by the interest of the contractors of health services research (like national health policymakers) to invest in the study of a specific content. The research proposals that are awarded funding by policymakers are likely to reflect this emphasis on specific content. While we as researchers were interested in answering such content-focused questions, we also hypothesized that to increase the quality of the research – and consequently its usefulness – we should address other questions as well. It is not just answering the generalizable question of ‘What works?’ but answering the additional questions of ‘What works: how, why, where, when and for
whom?’ that can improve the quality of the research and provide a better understanding of specific results so that policymakers can be better informed. Of course, these types of questions are not unique and can also be found in studies on effective implementation of interventions in health care. However, these commonly address changes in practices of care professionals [23-26]. Here we chose to use the perspective of policy analysis. Doing so, we attempted to align the perspective of health services research with that of health policy. Consequently, the question of ‘What works: how, why, when and for whom?’ can be operationalized in a combination of researchable questions (in addition to the initial research question) regarding content as well as context and process, with actors playing the central role in all of them. The challenge of the model lies in its application: How can we address these additional questions in health services research for national health policy?

In conclusion, we explore two research questions in this thesis in order to increase the usefulness of health services research for national health policy. Firstly, how can health services research include content-, context- and process-focused research questions (in addition to the initial research question), and secondly, how can health services researchers participate in an interaction process with policymakers?

Health services research at work for national health policy

This thesis presents three cases of health services research at work. All are research projects in which a content-focused research question was posed by national and international health policymakers: one by a health minister, a second by the World Health Organization (WHO), who was advising a director-general for health services, and a third question by a director-general of health care [27]. All cases concerned national health policies: two in the Netherlands and one in Nepal. In two cases the initial research question was formulated as a cost-effectiveness question regarding a new health services intervention [28;29]. The decision to continue the interventions on a nationwide scale depended in part on the outcomes of our studies. In the third case a new instrument for performance measurement of a country’s entire health system was requested. In all cases we initiated research activities to address a combination of additional content-, context- and process-focused questions, i.e. in addition to the initial content-focused questions that national health policymakers were interested in. The chapters presented in this thesis address most of the additionally formulated research questions. The choice of chapters was based on the personal involvement of the author of this thesis in addressing these questions.

The three cases are briefly presented in this introduction by describing the interventions and their general backgrounds. Also, the initial research question and its history are described, as well as the combination of content-, context- and process-focused research questions (in addition to the initial research question) and the researchers’ reflections that led to them. ‘Actors’ play an important role in all chapters. Table 2 provides an overview of the chapters in this thesis.
Case I. A national screening programme for familial hypercholesterolaemia?

The first case in this thesis concerns the genetic screening programme for familial hypercholesterolaemia in the Netherlands (see Box 1). The programme started in 1994 as an experiment and was funded by the Ministry of Health, Welfare and Sport (MoH) for a period of four years. The decision on structural nationwide implementation and long-term funding by the MoH depended in part on an evaluation of the effects of the programme in its experimental phase.

Box 1. Screening for Familial Hypercholesterolaemia

Screening for familial hypercholesterolaemia

Familial hypercholesterolaemia (FH) predisposes for coronary artery disease (CAD) and premature cardiac death [30]. The discovery of Low Density Lipoprotein (LDL) receptor gene mutations in clinically diagnosed FH patients and the subsequent development of DNA tests to detect these mutations enables the detection of FH patients before a first CAD event [31]. This is particularly relevant for relatives of confirmed FH patients, who are at much higher risk of having an LDL receptor gene mutation than the general population. Therefore, a family-based genetic screening programme for FH was implemented in the Netherlands by the Foundation for the Identification of Persons with Inherited Hypercholesterolaemia (StOEH) [32;33].

The Foundation for the Identification of Persons with Inherited Hypercholesterolaemia (StOEH) initiated an evaluation of the programme. Researchers from the Department of Social Medicine of the Academic Medical Center to the University of Amsterdam, together with the StOEH, wrote a research proposal for an evaluation study which was funded by the Netherlands Organisation for Health Research and Development (ZonMw) [28]. The proposal included three research questions: 1) What are the expected costs and health effects of the screening programme? 2) What is the predictive value of DNA tests used to diagnose FH? and 3) To what extent is participation in the screening and treatment of FH disturbing?

The evaluation study was carried out from 1997 to 2000 [34]. The case in this thesis focuses on research related to the question about costs and effects (Question 1). Questions 2 and 3 are both addressed in the final evaluation report [34]. In addition, Question 3 is addressed in detail by Van Maarle [35].

Reflections of the involved health services researchers that led to identifying additional content-, context- and process-focused research questions.

Some of the implicit assumptions about the disease, the programme’s context and the processes involved were challenging. Firstly, the documents of the Foundation for the Identification of Persons with Inherited Hypercholesterolaemia (StOEH) presented FH as a monogenetic, probably 100% penetrant disorder [36]. A mutation in the LDL receptor gene...
leads to very high blood cholesterol levels. To better understand the potential impact of the screening programme, we were curious to learn more about the correlation between the prevalence of a mutation and the prevalence of hypercholesterolemia in screened persons. We addressed this question in an additional study presented in Chapter 2.

Secondly, it was assumed the programme would detect persons with FH who were not yet known to have FH. The potential benefit of the screening programme will also depend on whether existing hypercholesterolemia is already diagnosed. Therefore, we wanted to know how many screened persons with FH had already been identified with hypercholesterolaemia in general practice prior to screening. This was also relevant, as GPs might give suboptimal treatment to persons with hypercholesterolaemia caused by an LDL receptor gene mutation and not by lifestyle factors, for example. This question is addressed in Chapter 3.

Thirdly, the chosen methodological approach for the screening was based on a pedigree analysis – ‘cascade screening’ [32] – for which previously screened persons provided the genealogical information. We hypothesized that the identification of an ‘index case’ and the subsequent methodological rigor to attain the completeness of the pedigree was important for identifying all FH mutation carriers. If an index case was missed the family would not be considered at risk, and if there were ‘blank spaces’ in the pedigree or in the programme follow-up, the potential effect of the overall programme could be influenced. We concluded that an analysis of the programme’s potential in terms of identifying all mutation carriers was important for estimating and understanding the programme’s effects.

In relation to the third initial research question (‘To what extent is participation in the screening and treatment of FH disturbing?’), the research group discussed the potential social consequences of participating in the screening programme. The discussion focused on whether participants faced difficulties when applying for life insurance. Although the existing guidelines for insurers regarding questions about health risks were clear [37] and in theory did not seem to create a barrier for applicants screened for FH, the research group felt it was important to investigate how those people who had been screened experienced trying to get insurance.

In summary, our discussions focused on the assumptions underlying the programme and we formulated the following research questions in addition to the question of the cost-effectiveness of the screening programme:

(These questions are included in this thesis.)

1. What is the prevalence of a mutation among screened persons with hypercholesterolaemia and the prevalence of hypercholesterolaemia among persons with an LDL receptor gene mutation? (presented in Chapter 2 [38])

2. What proportion of patients with FH were identified with hypercholesterolaemia in general practice prior to genetic screening? (presented in Chapter 3 [39])

(These questions are not included in this thesis.)

3. What is the potential of the current methodology of pedigree analysis and follow-up: How many family members can be reached by the programme and how many have already been reached? [40]
4. When applying for insurance, do screened persons (and persons with positive screening results) face consequences because they participated in the screening programme? [41]

The screening programme’s cost-effectiveness evaluation (Question 1 of the research proposal) is addressed in Chapter 4 [42].

Case II. Nationwide implementation of respiratory guidelines?

The second case in this thesis concerns the implementation of respiratory care guidelines in Nepal. His Majesty’s Government of Nepal, Ministry of Health (HMG-MoH) receives assistance from the WHO to improve the quality of care for children (over 5 years of age), youths, and adults with respiratory symptoms. For this specific topic, WHO is developing the Practical Approach to Lung Health (PAL)--initiative together with Nepal and other member-states (see Box 2.). Elements of PAL are being field-tested and evaluated in several countries to guide the further development of the package and to support policy decisions on PAL’s uptake [43].

In 1998, the WHO invited researchers to evaluate the costs and effects of PAL in order to provide support for decision-making about implementing it in participating countries. The researchers developed proposals for studying the costs and effects of PAL in Nepal, one of the countries participating in its development and pilot implementation. This resulted in two proposals: one for assessing the training’s effectiveness and one for assessing the costs and effects of developing and implementing PAL in Nepal, using a cluster randomized trial design. Only the latter proposal was funded [29].

Box 2. Practical Approach to Lung Health.

**Practical Approach to Lung Health (PAL)**

Respiratory diseases are an important cause of morbidity and mortality in all ages everywhere. In middle- and low-income countries there is the suspicion that primary care facilities may provide inadequate care for respiratory diseases. With the exception of case management of tuberculosis, many health systems have no standard strategy for dealing with respiratory diseases in adults [44]. In 1997, the WHO started developing integrated clinical care guidelines for adult lung health in primary care settings in middle- and low-income countries. This initiative was called the Adult Lung Health Initiative (ALHI), later renamed the Practical Approach to Lung Health (PAL), because PAL was a more appropriate acronym in Islamic cultures [45].

The Practical Approach to Lung Health initiative aims to improve the syndromic management of lung diseases in children (over 5 years of age), youths and adults in middle- and low-income countries [45]. PAL is presented as a package consisting of a generic clinical practice guideline and accompanying training materials. It targets ‘multipurpose’ health workers at peripheral primary and secondary care facilities.

The following were the core objectives of the cost-effectiveness study:

1. To compare the costs of implementing PAL in government facilities to the costs of maintaining the standard array of medical services.
2. To compare at the primary care level the effects for patients and facilities in PAL areas to the effects in areas using the standard array of medical services.

3. To allow comparison of the cost effectiveness of the PAL strategy to other potential investments in a population’s health.

These objectives were addressed in a cluster randomized trial. Some of the results have been published, others are forthcoming [46-48]. The research questions that can be considered to be part of the initial cost-effectiveness study (such as studies on PAL’s effects on prescribing drugs [49] and on the burden of respiratory diseases in Nepal) are not presented in this case. Neither are studies presented that validated measurement instruments necessary to assess the effects of PAL at patient level. This was particularly important in measuring participants’ respiratory conditions and their quality of life. Reference values for peak expiratory flow in the Nepalese population had to be collected [50] and quality-of-life instruments had to be validated within the Nepalese context [51].

Reflections of the involved health services researchers that led to identifying additional content-, context- and process-focused research questions.

PAL was presented by the WHO as a package of interventions and strategies. To better understand the possible effects of PAL, it was necessary to open the package and study its elements and underlying assumptions that were related to the process and context in Nepal. As a result, the team developed three research questions in addition to the cost-effectiveness evaluation.

Firstly, we observed that the study proposal did not explicitly address the context in which PAL was going to be implemented in Nepal. Much attention was given to the international importance of PAL. Only two context-specific conditions were mentioned: the absence of diagnostic guidelines and the enormous logistic difficulties existing in Nepal. We anticipated scarce resources and consequently erratic availability of specific drugs in rural areas. The team had long discussions with the WHO counterpart as to whether WHO should provide drugs to the health services while PAL was being evaluated. Such additional measures would need the full support of several counterparts in Nepal. The uncertainty about the local situation and outcome of discussions as described created the need for assessing the broader context and processes of adapting and implementing PAL. We hypothesized that with such information, the outcome of the cost-effectiveness assessment could be better understood and could potentially guide policy changes. Results of this additional study are presented in Chapter 6 [52].

Secondly, we observed that during the adaptation process, the participants in the adaptation working group shared their views on and knowledge of the primary care health services in rural areas. In the proposal, however, no opportunity was created for assessing the functioning of the primary care health services in Nepal. Nor were patient views incorporated into the adaptation process or evaluation study. We argued that an understanding of how the health services functioned should be based on information not only from the health care providers,
financiers and policymakers, but also from the users of these health services. We developed a proposal to study the health care context from the patient’s perspectives and experiences. This study is presented in Chapter 7 [53].

Thirdly, the results of the cost-effectiveness evaluation were expected to guide the policymaking process. How the results would be used was unclear. Because methods for priority-setting have attracted increasing interest in the international literature, an opportunity arose for applying a rational approach to prioritizing health programmes, including PAL in Nepal. To explore possibilities for a rational approach to priority-setting for resource allocation in low-income countries, we assessed the relative weights of different characteristics of health care programmes. The results of this exploration in Nepal are presented in Chapter 8 [54].

In summary, in addition to the question of the policymakers on the cost-effectiveness of the implementation of respiratory care guidelines, we formulated the following research questions:

(All of these questions are included in this thesis.)

1. What is, ex ante, the implementability of PAL in Nepal based on the quality of the guidelines, the implementation strategy and the social context in which PAL is going to be implemented? (presented in Chapter 6 [52])

2. What route is used by tuberculosis patients within the health care context from the moment their complaints first begin until the start of tuberculosis treatment? and What is the role of the governmental primary care facilities in this journey? (presented in Chapter 7 [53])

3. What is the relative importance of PAL in Nepal compared to other programmes? (presented in Chapter 8 [54])

Case III. An instrument for national health system performance measurement?

The third case concerns health services research for developing a tool to measure the performance of the Dutch health system. In 2001, the need for international comparisons of health system performance and the development of a major health system reform in the Netherlands were the driving forces behind the Dutch MoH’s development of indicators for the performance of the entire health system [55;56]. Performance measurement should inform the monitoring and control functions of the MoH. Previously, primarily volume and cost data about health care provision were collected and reported in health sector reports [57]. Inspired by activities of the Organisation for Economic Co-operation and Development (OECD) in the field of performance and quality measurement, the MoH formulated a request for research assistance for developing a performance indicator framework for the entire Dutch health system [58] [59].
Reflections of the concerned health services researchers that led to identifying additional content-, context- and process-focused research questions.

By responding to the request of the MoH, we were given an opportunity to build a new instrument that would be used in the policy practice of the MoH to monitor and steer the health system. We realized that this tool had to meet many requirements that were linked to its intended use and function (see Chapter 10 [60]). Also, we realized this tool was going to be used in an environment with dynamic organizational and policy context and processes. We hypothesized that whether or not this new tool would actually be used would depend largely on the context and processes that coexisted. Therefore, we formulated the following additional question: ‘What organizational and policy contexts and processes influence the development of the performance indicator framework?’ This additional question is addressed in Chapter 11 [61].

In this study the researchers collaborated closely with the MoH in an interactive research and development process. During this process we realized that for certain potential indicators, an extra boost in terms of additional studies might help the MoH develop these indicators. As a result, we formulated additional research questions for three specific indicators. One regarded the efficiency of allocation of resources, the second, the performance of mental health care and the third, measuring patient experiences with health care.

In summary, we addressed the following research questions in addition to the initial question of the policymakers to develop a tool for health system performance:

1. What are the organizational and policy context and processes that influence, and potentially interact with, the development of the performance indicator framework? (presented in Chapter 11, [61])

2. To what extent are decisions about reimbursement by the Dutch Sickness Fund based on cost-effectiveness information? [62]

3. What are useful and feasible performance indicators for mental health? [63]

4. How can patient experiences with health care be measured as an indicator of the health care system’s responsiveness? [64]

In Chapter 10, the proposed conceptual framework for performance indicators is presented [60].

Outline of this thesis

Following this introduction, the thesis consists of three case-specific sections, each containing multiple chapters. For each case, the chapters present the results of several of the research questions mentioned above (see Table 2). At the end of each case, a retrospective chapter ‘Health Services Research at work in Case …’ addresses the position of the researchers in relation to other actors and the inclusion of the combination of content-, context- and process-focused questions (in addition to the initial research question), the interaction of the
researchers with policymakers, and describes the follow-up of events and developments in the three cases.

The thesis concludes with a discussion in Chapter 13 in which a reflection is given on the lessons learned for health services research by answering the central question in this thesis: In order to increase the usefulness of health services research for national health policy, (a) How can health services research include content-, context- and process-focused research questions (in addition to the initial research question)? and (b) How can health services researchers participate in an interaction process with policymakers? What was the added value of including the combination of content-, context- and process-focused research questions (in addition to the initial research question)? Suggestions are also made as to how we can include Walt and Gilson’s model in a structural way in health services research.

Table 2 gives an overview of the chapters in this thesis and shows which domain (content, context or process) is addressed in each chapter. Also, the involved actors are listed for each case, according to the following categories: a) intended users, b) contractors, c) subject of study and d) academic disciplines of the actors in the health services research group.
Table 2. Overview of cases and chapters in this thesis: a combination of additional content- and process-focused research questions and involved actors.

<table>
<thead>
<tr>
<th>Chapter</th>
<th>Content</th>
<th>Context</th>
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<tr>
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<td>Introduction</td>
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<td>2</td>
<td>Results from a family- and DNA-based screening programme for FH</td>
<td>Genetic screening for familial hypercholesterolaemia in 1992-1997: primarily younger patients in the care of general practitioners</td>
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<td>3</td>
<td>A national screening programme for familial hypercholesterolaemia?</td>
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<td>4</td>
<td>Cost-effectiveness of a family- and DNA-based screening programme on familial hypercholesterolaemia in the Netherlands</td>
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<td>5</td>
<td>HSR(^2) at work in Case I</td>
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<td>6</td>
<td>How did you get here? Twenty-six journeys on the road to tuberculosis treatment in rural Nepal</td>
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<td>7</td>
<td>Nationwide implementation of respiratory care guidelines?</td>
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<td>HSR at work in Case II</td>
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<td>Developing a national performance indicator framework for the Dutch health system</td>
<td>The Performance Indicator Framework of the Dutch Health System: A Progress Report(^3)</td>
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<td>13</td>
<td>Discussion</td>
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1 a=intended users, b=contractor, c=subject of study, d=involved disciplines in health services research group
2 HSR=health services research
3 This chapter addresses both the context as well as the process in which the performance indicator framework was developed.
<table>
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<th>Process</th>
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| A rational multi-criteria approach to priority setting: Should a lung health programme be implemented in Nepal? | a. MoH Nepal, WHO,  
b. NWO-WOTRO  
c. Health care providers, patients, policymakers  
d. Health economists, epidemiologists, public health specialists, health scientists, medical doctor, political scientist |
| Implementing global knowledge in local practice: a WHO lung health initiative in Nepal | a. MoH, StOEH  
b. ZonMw  
c. StOEH, general practitioners  
d. Epidemiologists, psychologists, health scientist, medical doctors |
| Developing a national performance indicator framework for the Dutch health system | a. MoH  
b. MoH  
c. MoH  
d. Epidemiologists, public health specialists, health scientists, medical doctors, political scientist |
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A national screening programme for familial hypercholesterolaemia?
Results from a family and DNA based active identification programme for familial hypercholesterolaemia


J Epidemiol Community Health 2001 55 500-502
Introduction

Heterozygous familial hypercholesterolaemia (FH) is a common inborn error of lipoprotein metabolism, which strongly predisposes for coronary artery disease (CAD) and premature cardiac death [1]. In 1994, a family based active identification programme for FH was implemented in the Netherlands [2]. It is based on DNA diagnosis of the LDL-receptor gene mutation which enables us to search selectively for patients in a high-risk population. The programme initially targets first and second-degree relatives of FH probands (diagnosed at Lipid Research Clinics throughout the country) and extends further into the family only when new patients are identified. The programme aims to identify mutation carriers and to refer them to Lipid Research Clinics for extensive individual risk assessment and, if necessary, treatment. Since no carefully collected data are available for cholesterol levels among the general population of LDL-receptor gene mutation carriers, the large majority of whom are asymptomatic, we studied the prevalence of hypercholesterolaemia among screenees with a proved LDL-receptor gene mutation.

Methods and Results

Between 1994 and 1998 2814 adults were screened. The estimated response rate was constant over the years at 90% [3;4]. For reasons of comparison with available population data for total serum cholesterol levels [5], we selected those who were between 20 and 60 years of age (1856 screenees). Depending on the available funds in the screening programme, which were lacking in certain periods, single cholesterol measurements were taken at the time of screening. Therefore, we analysed the data of all 1005 persons who had DNA test results as well as cholesterol measurements. These were a non-selective sample of the 1856 screenees between 20 and 60 years of age. Cholesterol was measured using commercially available kits (Boehringer Mannheim, Mannheim, Germany). Genomic DNA was isolated from the leukocyte fraction of 10 ml of freshly collected blood, followed by polymerase chain reaction (PCR) and restriction enzyme analysis.

From the perspective of the screening programme, the screenees that are already treated can not be considered as new cases and they do not benefit from the screening programme in the same manner as newly identified cases. Therefore, we present the prevalence of hypercholesterolaemia among all screenees as well as the prevalence of hypercholesterolaemia among those not yet treated with HMG-CoA reductase inhibitors.
Hypercholesterolaemia was defined as either an untreated total cholesterol (TC) level above the 95th percentile for age and sex in the Dutch population (table 1) [5], or as receiving HMG Co-A reductase inhibitors. We also show the total cholesterol distribution for the untreated screenees using conventional cut-off points (<6.5, 6.5-7.9, >=8 mmol/l). All LDL-receptor

### Table 1. MORGEN Project Data 1996-1997: mean total serum cholesterol (TC) and standard deviation (SD) as well as 95th percentile (P95) in the general Dutch population by sex and age group.

<table>
<thead>
<tr>
<th>MEN</th>
<th>Age group (years)</th>
<th>N</th>
<th>Mean TC (SD)</th>
<th>P95</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>20-29</td>
<td>589</td>
<td>4.4 (0.9)</td>
<td>5.8</td>
</tr>
<tr>
<td></td>
<td>30-39</td>
<td>890</td>
<td>5.0 (1.0)</td>
<td>6.7</td>
</tr>
<tr>
<td></td>
<td>40-49</td>
<td>1210</td>
<td>5.5 (1.0)</td>
<td>7.2</td>
</tr>
<tr>
<td></td>
<td>50-59</td>
<td>1030</td>
<td>5.5 (1.0)</td>
<td>7.1</td>
</tr>
<tr>
<td>WOMEN</td>
<td>Age group (years)</td>
<td>N</td>
<td>Mean TC (SD)</td>
<td>P95</td>
</tr>
<tr>
<td></td>
<td>20-29</td>
<td>843</td>
<td>4.6 (0.8)</td>
<td>6.1</td>
</tr>
<tr>
<td></td>
<td>30-39</td>
<td>1140</td>
<td>4.8 (0.8)</td>
<td>6.3</td>
</tr>
<tr>
<td></td>
<td>40-49</td>
<td>1450</td>
<td>5.1 (0.9)</td>
<td>6.7</td>
</tr>
<tr>
<td></td>
<td>50-59</td>
<td>1139</td>
<td>5.7 (1.0)</td>
<td>7.4</td>
</tr>
</tbody>
</table>

### Table 2. Prevalence of hypercholesterolaemia (HC) by sex, age group and DNA test result in all screenees and prevalence of hypercholesterolaemia, mean total serum cholesterol (TC), standard deviation (SD) and total serum cholesterol distribution, using conventional cut-off values (6.5 and 8.0 mmol/l) as well as the 95th percentile (P95) in untreated screenees.

<table>
<thead>
<tr>
<th>MEN</th>
<th>20-29 years</th>
<th>30-39 years</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>all</td>
<td>fh+</td>
</tr>
<tr>
<td>All ¹</td>
<td>105 (100)</td>
<td>35 (33.3)</td>
</tr>
<tr>
<td>% HC</td>
<td>39.0</td>
<td>82.9</td>
</tr>
<tr>
<td>Untreated</td>
<td>N (%)</td>
<td>101 (100)</td>
</tr>
<tr>
<td>Mean TC (SD)</td>
<td>5.5 (1.4)</td>
<td>6.9 (1.3)</td>
</tr>
<tr>
<td>TC&lt; 6.5 (%)</td>
<td>81 (80.2)</td>
<td>13 (41.9)</td>
</tr>
<tr>
<td>6.5 &lt;= TC&lt; 8.0 (%)</td>
<td>11 (10.9)</td>
<td>9 (29.0)</td>
</tr>
<tr>
<td>TC &gt;= 8.0 (%)</td>
<td>9 (8.9)</td>
<td>9 (29.0)</td>
</tr>
<tr>
<td>% &gt; P95</td>
<td>36.6</td>
<td>80.6</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>WOMEN</th>
<th>20-29 years</th>
<th>30-39 years</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>all</td>
<td>fh+</td>
</tr>
<tr>
<td>All ²</td>
<td>103 (100)</td>
<td>40 (38.8)</td>
</tr>
<tr>
<td>% HC</td>
<td>44.7</td>
<td>90.0</td>
</tr>
<tr>
<td>Untreated</td>
<td>N (%)</td>
<td>89 (100)</td>
</tr>
<tr>
<td>Mean TC (SD)</td>
<td>5.7 (1.7)</td>
<td>7.5 (1.5)</td>
</tr>
<tr>
<td>TC&lt; 6.5 (%)</td>
<td>65 (73.0)</td>
<td>8 (30.8)</td>
</tr>
<tr>
<td>6.5 &lt;= TC&lt; 8.0 (%)</td>
<td>12 (13.5)</td>
<td>8 (30.8)</td>
</tr>
<tr>
<td>TC &gt;= 8.0 (%)</td>
<td>12 (13.5)</td>
<td>10 (38.5)</td>
</tr>
<tr>
<td>% &gt; P95</td>
<td>36.0</td>
<td>84.6</td>
</tr>
</tbody>
</table>

¹ Hypercholesterolaemia was defined as either an untreated total cholesterol (TC) level above the 95th percentile for age and sex in the Dutch population (table 1), or as receiving HMGco-A reductase inhibitors.
gene mutation carriers were heterozygotes. None of the screenees had been tested for a LDL-receptor gene mutation before. Table 2 shows the results for the screened population. It is evident that each age category contains LDL-receptor gene mutation carriers who do not have hypercholesterolaemia: 19.8% in all men, 32.3% in untreated men and 16.7% in women, 28.7% in untreated women. Furthermore, it is shown that the prevalence of mutation carriers among all screenees tends to be lower in the older age groups. This is probably the result of selective mortality. However, the prevalence of mutation carriers among untreated screenees is also lower in the older age groups. This is not purely the result of selective mortality but it is mainly due to the fact that an increasing proportion of those screened in the older age groups is already treated with cholesterol lowering drugs, and more in mutation carriers than in those without a mutation since they have generally higher cholesterol levels. This might also explain why the prevalence of hypercholesterolaemia in untreated female mutation carriers is lower in the older age groups.

**Discussion**

These data have not been shown before in such a large and well-defined cohort, and have important consequences for case finding strategies for inherited disorders of lipoprotein metabolism but it is mainly due to the fact that an increasing proportion of those screened in the older age groups is already treated with cholesterol lowering drugs, and more in mutation carriers than in those without a mutation since they have generally higher cholesterol levels. This might also explain why the prevalence of hypercholesterolaemia in untreated female mutation carriers is lower in the older age groups.

![Results from an identification programme for FH](image-url)

- Table 2 shows the results for the screened population.
- It is evident that each age category contains LDL-receptor gene mutation carriers who do not have hypercholesterolaemia: 19.8% in all men, 32.3% in untreated men and 16.7% in women, 28.7% in untreated women. Furthermore, it is shown that the prevalence of mutation carriers among all screenees tends to be lower in the older age groups. This is probably the result of selective mortality. However, the prevalence of mutation carriers among untreated screenees is also lower in the older age groups. This is not purely the result of selective mortality but it is mainly due to the fact that an increasing proportion of those screened in the older age groups is already treated with cholesterol lowering drugs, and more in mutation carriers than in those without a mutation since they have generally higher cholesterol levels. This might also explain why the prevalence of hypercholesterolaemia in untreated female mutation carriers is lower in the older age groups.

## Discussion

These data have not been shown before in such a large and well-defined cohort, and have important consequences for case finding strategies for inherited disorders of lipoprotein metabolism.

<table>
<thead>
<tr>
<th>40-49 years</th>
<th>50-59 years</th>
<th>All ages (20-59 years)</th>
</tr>
</thead>
<tbody>
<tr>
<td>all</td>
<td>fh+</td>
<td>fh-</td>
</tr>
<tr>
<td>134 (100)</td>
<td>38 (28.4)</td>
<td>96 (71.6)</td>
</tr>
<tr>
<td>35.8</td>
<td>76.3</td>
<td>19.8</td>
</tr>
<tr>
<td>109 (100)</td>
<td>17 (15.6)</td>
<td>92 (84.4)</td>
</tr>
<tr>
<td>6.2 (1.2)</td>
<td>7.4 (1.0)</td>
<td>6.0 (1.1)</td>
</tr>
<tr>
<td>71 (65.1)</td>
<td>4 (23.5)</td>
<td>67 (72.8)</td>
</tr>
<tr>
<td>30 (27.5)</td>
<td>8 (47.1)</td>
<td>22 (23.9)</td>
</tr>
<tr>
<td>8 (7.3)</td>
<td>5 (29.4)</td>
<td>3 (3.3)</td>
</tr>
<tr>
<td>21.1</td>
<td>47.1</td>
<td>16.3</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>40-49 years</th>
<th>50-59 years</th>
<th>All ages (20-59 years)</th>
</tr>
</thead>
<tbody>
<tr>
<td>all</td>
<td>fh+</td>
<td>fh-</td>
</tr>
<tr>
<td>146 (100)</td>
<td>43 (29.5)</td>
<td>103 (70.5)</td>
</tr>
<tr>
<td>34.2</td>
<td>83.7</td>
<td>13.6</td>
</tr>
<tr>
<td>124 (100)</td>
<td>22 (17.7)</td>
<td>102 (82.3)</td>
</tr>
<tr>
<td>5.9 (1.2)</td>
<td>7.3 (1.5)</td>
<td>5.6 (0.9)</td>
</tr>
<tr>
<td>93 (75.0)</td>
<td>7 (31.8)</td>
<td>86 (84.3)</td>
</tr>
<tr>
<td>26 (21.0)</td>
<td>10 (45.5)</td>
<td>16 (15.7)</td>
</tr>
<tr>
<td>5 (4.0)</td>
<td>5 (22.7)</td>
<td>0</td>
</tr>
<tr>
<td>22.6</td>
<td>68.2</td>
<td>12.7</td>
</tr>
</tbody>
</table>

2 95th percentile for total cholesterol in the Dutch general population as observed in the MORGEN-study (Source: RIVM Bilthoven The Netherlands) (see table 1).

3 All = treated and untreated screenees
metabolism with a known molecular basis. Our study shows that if in a high risk population of yet untreated, mainly asymptomatic mutation carriers, a single TC level would be used for the diagnosis of FH rather than the current gold standard, i.e. the presence of a LDL-receptor gene mutation, the diagnosis would be missed in more than a quarter of the FH patients. As is shown by others, FH is not fully penetrant from birth onwards [6]. It has not been documented before, however, to which extent the genetic disorder causes hypercholesterolaemia in a population of mainly asymptomatic adult relatives of genetically diagnosed patients as is shown by the present study.

The high prevalence of “lower” TC levels in our cohort may well reflect a combination of factors like patients’ adherence to low calorie diets for weight loss, intercurrent illness of infectious nature, a better general health of participants in a screening programme, or a protective genetic constitution. In addition, because cholesterol levels vary with LDL-receptor mutation [7], we cannot exclude that our findings of normal cholesterol levels in LDL-receptor gene mutation carriers are the result of screening for mutations that may result in a milder than expected phenotype as reported elsewhere, albeit very unlikely. Nevertheless, even though a single measurement of cholesterol is not very reliable when assessing the individual cardiovascular disease risk, for the purpose of assessing the cholesterol values on a population level a single measurement can be used [8]. The prevalence of hypercholesterolaemia in those without a LDL-receptor gene mutation is higher than in the general population, which indicates that in this high-risk population probably also other factors than the LDL-receptor gene mutation contribute to the prevalence of hypercholesterolaemia.

The importance of our findings depends largely on whether patients with a LDL-receptor gene mutation but without hypercholesterolaemia, experience an increased risk of coronary heart disease and whether they need the same rigorous treatment as other FH patients. This is currently unknown. Since damage to the vascular wall in FH patients is likely to be the result of the number of cholesterol-years [9], a single TC level below the 95th percentile could be falsely reassuring. Comprehensive appraisal of cardiovascular disease risk and cholesterol screening at regular intervals is advised. In a follow up study, we plan to further explore explanations for our findings and to assess whether the risk of coronary heart disease is increased in this group of mutation carriers in whom FH does not seem to be fully penetrant.

Reference List


Results from an identification programme for FH


Chapter 3

Genetic screening for familial hypercholesterolaemia in 1992-1997: primarily younger patients in the care of general practitioners

A.H.A. ten Asbroek, S. van Lunsen, P.J. Marang-van de Mheen, L.J. Gunning-Schepers

Ned Tijdschr Geneeskd 2000 144(3) 125-129

Translation by C. Higgins.
Abstract

Objective
To estimate the proportion of patients with familial hypercholesterolaemia (FH) who were identified with hypercholesterolaemia in general practice prior to screening by means of pedigree research and DNA analysis by the National Foundation for the Identification of Persons with Familial Hypercholesterolaemia (StOEH).

Design
Retrospective.

Method
General practice files of FH patients, diagnosed through genetic screening by the StOEH in 1992-1997 whose general practitioner’s (GP’s) practice in Amsterdam, Haarlem or Alkmaar, were studied for cholesterol and FH related information documented in the period prior to the screening.

Results
Out of the 121 persons selected 80 agreed to the study; one GP refused to co-operate. There was no difference between respondents and non respondents with regard to age, sex or domicile of the GP. In 48 of 79 (61%) general practice files studied, cholesterol measurements were reported prior to screening; 39 patients (49%) had hypercholesterolaemia and 29 (37%) were being treated with cholesterol lowering drugs. Mean age of the FH patients who had no record of their cholesterol levels was 25.1 years (SD: 17.0) at the time of screening, 22 years younger than the mean age of FH patients who did have cholesterol levels on record prior to screening (47.1 (SD: 18.4); p < 0.0001).

Conclusion.
Of the FH patients identified through family based genetic screening especially the younger FH patients are newly brought to the attention of their GP.
Introduction

Familial hypercholesterolaemia (FH) is an autosomal dominant disorder characterized by extremely high cholesterol, which commonly leads to premature coronary heart disease (CHD) [1-4]. The prevalence of the heterozygous form is 1:400 [5]; the homozygous form is more rare (1:1,000,000) and is clinically more severe [1;3]. Here we will discuss data of patients with heterozygous FH. The diagnosis of FH is usually made on clinical grounds after the appearance of CHD. FH is caused by a mutation in the low-density lipoprotein receptor gene. Using genetic diagnostics, a mutation can be detected before the first clinical symptoms appear [1]. Considering their sharply increased risk of FH and also CHD, this is particularly relevant for relatives of people with FH.

Treatment of FH is aimed at lowering cholesterol. Until the end of the 1980s, this meant lifestyle recommendations and medicinal therapy using fibrates and bile-acid-binding resins. With the introduction of cholesterol synthesis inhibitors – statins – the therapy became more effective and can achieve a significant decrease in the risk of CHD [6;7], also among FH patients [8-10]. The combination of genetic diagnostics with effective cholesterol-lowering therapy makes it advisable to screen populations with a sharply increased risk of FH. Relatives of patients with genetically confirmed FH form just such a high-risk group.

Since 1992, in the Netherlands, FH patients have been identified using a combination of family research and DNA analysis. In 1994, this led to the establishment of the ‘National Foundation for the Identification of Persons with Familial Hypercholesterolaemia’ (StOEH) [11;12]. FH patients who have been clinically diagnosed and genetically confirmed (referred to as ‘index patients’) and known to the StOEH through a lipid outpatient clinic form the starting point of the family research. Together with the index patient, the StOEH draws up a pedigree of his or her family. Relatives are then approached for DNA testing using the cascade principle: only when a first-degree relative has been shown to have FH are that person’s first-degree relatives approached. If first-degree relatives are dead (or do not want to participate), second-degree relatives are approached. As a result, although the FH patients identified are always first- or second-degree relatives of a genetically diagnosed FH patient, they are sometimes only ‘distant’ relatives of the index patient with whom the family study began. Those who have been screened receive the test results in writing, and people who test positive are referred to their general practitioners (GPs). The GP is advised to refer the patient to a lipid outpatient clinic for specialized care, which includes monitoring the lipid profile, followed by treatment if necessary.

Within the framework of a formal evaluation of the identification programme, it is important to estimate the health benefits that result from screening. In order to do this, it is important to know how many of the identified FH patients were already known to have hypercholesterolaemia. To answer this question, we examined the GP patient files of the FH patients for cholesterol and FH-related information. The main question was divided into four sub-questions:
Using the information in the GP patient files, how many of the identified FH patients had ever had their cholesterol levels checked prior to the genetic screening?

Of the FH patients identified, how many were already known to their GPs as having hypercholesterolaemia?

How many of the identified FH patients were already being treated for hypercholesterolaemia?

Of those identified as FH patients, for how many of these patients had their GPs recorded a positive family medical history or actually mentioned ‘familial hypercholesterolaemia’ in their patient files?

For the first sub-question, we studied whether the results were age-related. The study was approved by the AMC’s Medical Ethics Commission.

Methods

Participants

Included in the study were all screened relatives of index patients who had been selected based on having a) a genetic mutation caused by FH, and b) a GP living in Amsterdam, Haarlem or Alkmaar. One-hundred twenty-one (121) people met these criteria, and all of them had been screened between 1992 and 1998. In this period, the StOEH drew up a total of 196 pedigrees and identified 1336 FH patients. The 121 people selected came from 48 pedigrees. Those selected were evenly distributed with regard to sex and age. Participants gave permission for access to their GP patient files; this permission was limited to information related to FH.

Analysis of patient records

The GP patient files were examined using an ‘item list’. All cholesterol-related information (including cholesterol measurements, cholesterol-lowering therapy, record of relevant family medical history or actual mention of ‘familial hypercholesterolaemia’) was included in the study, irrespective of the date the information was recorded. The data on consultations and what had been done were used to make it possible to select information prior to the genetic screening (and where necessary also prior to treatment with medicine). When analysing the cholesterol measurements, only those measurements taken prior to treatment were used. If there were multiple cholesterol measurements, an average of the cholesterol values during the untreated period was calculated for further analysis. ‘Treatment’ is understood to be treatment with statins. The information obtained came from handwritten information on the patient’s consultation card, in correspondence included in the GP patient files, and from automated GP patient files.

Because this study looks at information available from GPs, for defining hypercholesterolaemia we used cut-off points for total cholesterol of 6.5 mmol/l and 8.0 mmol/l from the 1991
standards of the Dutch College of General Practitioners. For comparison, the 95th percentile (P95) for total cholesterol was used, an internationally accepted cut-off point for clinical diagnosis of FH. This percentile is calculated from unpublished information gathered within the framework of the ‘Monitoring Project on Risk Factors and Health in the Netherlands’ (MORGEN project) in 1996 and 1997 [13]. Because there was no reference information for people younger than 20 and older than 59 years of age, for these age groups the 95th percentile was used for 20 to 25 year olds and 55 to 60 year olds respectively.

**Statistical analysis**

For analysis of the differences in average age we used Student’s t-test; for differences in percentage between sub-groups we used an $X^2$ test. Finally, we looked at whether the results of this study were influenced by the participation of patients screened before StOEH’s formal establishment in 1994.

**Results**

Eighty (80) of the 121 FH patients who were approached agreed to participate in the study. One patient’s GP was not willing to cooperate, which meant that in the end, 79 (65.3%) GP patient files were examined.

There was no statistically significant difference between respondents and non-respondents with regard to distribution according to sex ($X^2$=0.02, $p=0.88$), average age ($t=0.53$, $p=0.69$) (table 1), or GP address ($X^2$=1.2, $p=0.56$) (information not shown).

**Table 1.** Age and sex of respondents and non-respondents.

<table>
<thead>
<tr>
<th></th>
<th>Total</th>
<th>Men</th>
<th></th>
<th>Women</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>No.</td>
<td>%</td>
<td>Av. age ± SD</td>
<td>No.</td>
<td>%</td>
</tr>
<tr>
<td>Respondents</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>No.</td>
<td>79</td>
<td>65.3</td>
<td>38.6 ± 20.8</td>
<td>35</td>
<td>66.0</td>
</tr>
<tr>
<td>Non-respondents</td>
<td>42</td>
<td>34.7</td>
<td>40.7 ± 21.5</td>
<td>18</td>
<td>34.0</td>
</tr>
<tr>
<td>Total</td>
<td>121</td>
<td>100</td>
<td>39.3 ± 21.0</td>
<td>53</td>
<td>100</td>
</tr>
</tbody>
</table>

For 48 (60.8%) of the 79 patients, their patient files showed cholesterol had been measured prior to the genetic screening (table 2). The average age of these patients was 47.1 years (SD 18.4). The average age of the remaining patients was 25.1 years (SD 17.0). This 22.0-year difference is statistically significant ($t=5.32$, $p<0.0001$).

Thirty-nine patients (39; 49.4%) patients had average cholesterol values higher than 6.5 mmol/l (untreated). Twenty-seven (27) of them had values higher than 8.0 mmol/l (table 3). Likewise, 39 of the 79 patients had cholesterol values higher than the P95. However, some of these were different patients, in particular patients younger than those diagnosed when using cut-off points of 6.5 mmol/l and 8.0 mmol/l.
Twenty-nine (29; 36.7%) of the 79 patients were already being treated for hypercholesterolaemia prior to the genetic screening. The difference in the percentage of men among treated and untreated patients was not statistically significant ($X^2=0.01, p=0.94$).

In 27 of the 79 GP patient files, we found a positive family medical history or actual mention of ‘familial hypercholesterolaemia’. In 9 of these 27 GP patient files, we found both. The results found are no different than the results that would have been found if we had only used the information from those patients screened since the establishment of StOEH in 1994 (n=62) (separate information not shown here).

**Table 2.** Measurement of cholesterol prior to genetic screening.

<table>
<thead>
<tr>
<th></th>
<th>Total</th>
<th></th>
<th></th>
<th>Men</th>
<th></th>
<th></th>
<th>Women</th>
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</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>No.</td>
<td>%</td>
<td>Av. age ± SD</td>
<td>No.</td>
<td>%</td>
<td>Av. age ± SD</td>
<td>No.</td>
<td>%</td>
<td>Av. age ± SD</td>
</tr>
<tr>
<td>Cholesterol</td>
<td></td>
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<td></td>
<td></td>
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<td></td>
<td></td>
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<td></td>
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<tr>
<td>measurement</td>
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<td>48</td>
<td>60.8</td>
<td>47.1 ± 18.4</td>
<td>21</td>
<td>60.0</td>
<td>45.7 ± 17.8</td>
<td>27</td>
<td>61.4</td>
<td>48.1 ± 19.2</td>
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<td>31</td>
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<td>25.1 ± 17.0</td>
<td>14</td>
<td>40.0</td>
<td>22.5 ± 16.9</td>
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<td>100</td>
<td>38.6 ± 20.8</td>
<td>35</td>
<td>100</td>
<td>36.5 ± 20.6</td>
<td>44</td>
<td>100</td>
<td>40.3 ± 21.0</td>
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</table>

**Table 3.** Hypercholesterolaemia defined by cut-off points of 6.5 and 8.0 mmol/l and 95th percentile (P95) for total cholesterol.

<table>
<thead>
<tr>
<th></th>
<th>Total</th>
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<th></th>
<th>Men</th>
<th></th>
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<td>%</td>
<td>No.</td>
<td>%</td>
<td>No.</td>
<td>%</td>
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<tr>
<td>&lt; 6.5 mmol/l</td>
<td>31</td>
<td>39.2</td>
<td>14</td>
<td>40.0</td>
<td>17</td>
<td>38.6</td>
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<tr>
<td>6.5-8.0 mmol/l</td>
<td>39</td>
<td>49.4</td>
<td>18</td>
<td>51.4</td>
<td>21</td>
<td>47.7</td>
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<tr>
<td>&gt; 8.0 mmol/l</td>
<td>47</td>
<td>58.4</td>
<td>21</td>
<td>61.6</td>
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<td>35</td>
<td>100</td>
<td>44</td>
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</table>

**Discussion**

This study investigated how many FH patients were already known to their GPs as having hypercholesterolaemia prior to the genetic screening. In 61% of the GP patient files examined, at least one cholesterol measurement was reported to have been done prior to screening. On average, these patients were 22 years older than the FH patients who had never had
their cholesterol measured. Forty-nine (49) percent were known to their GPs as having hypercholesterolaemia. Thirty-seven (37) percent were being treated with cholesterol-lowering medication prior to the genetic screening.

The GP patient files of 79 of the 121 selected FH patients were examined (65% response). The difference in average age and distribution according to sex between respondents and non-respondents was not statistically significant. There was also no difference in distribution according to GP address. Because of this, there is no reason to assume the respondents are not representative of all those selected.

Considering the prevalence of specific mutation types differs according to region [14] and that the level of total cholesterol depends on the mutation type [1;15], it is possible that the geographic concentration of the GPs (living in Amsterdam, Haarlem and Alkmaar) influenced the results. It is possible that in other regions, a different prevalence of previously diagnosed hypercholesterolaemia would be found among FH patients. There is as of yet no population information on the extent of the variation of cholesterol levels according to mutation type, nor is there a geographical description of the mutation types found in the Netherlands.

In the MORGEN project, the age- and sex-specific 95th percentile (P95) as cut-off point for hypercholesterolaemia is not available for those younger than 20 and older than 60 years of age. Because of this, for these groups the P95 for 20 to 25 year olds and for 55 to 60 year olds were used respectively. This could mean that the prevalence of hypercholesterolaemia is underestimated in the lowest age group and overestimated in the highest age group. However, when available percentiles for cholesterol are used from the Canadian population [16], for example – which strongly correspond to those in the Netherlands in the 20 to 60 year age group – it appears the method we chose scarcely influences the prevalence of hypercholesterolaemia: only one patient younger than 20 years of age was diagnosed with hypercholesterolaemia with the Canadian P95, but not with the MORGEN P95.

It is possible that not all cholesterol-related information was included in the GP patient files, for example, when cholesterol measurements were taken as part of a company physical examination or during a hospital stay. However, it is clear that markedly high cholesterol will be reported to the GP. Because of this, we expect this kind of underreporting to have had no effect on the estimate of the percentage of FH patients with hypercholesterolaemia.

This study shows that the FH patients with no record of cholesterol measurements in their GP patient files prior to the screening were on average 22 years younger than the other FH patients. This means that of the identified FH patients, it was particularly the young FH patients who were being brought to the attention of the GPs for the first time. As a result, they can be referred for specialized care at an early stage, including monitoring of the lipid profile followed by treatment if necessary. Nevertheless, the underlying assumption is that the cholesterol distribution among FH patients already known to their GPs is the same as among FH patients identified for the first time through screening. Data from another study among 215 untreated FH patients confirm this (data not shown). In this study, patient information was selected from the same database, in a period in which cholesterol was determined and a DNA test was also carried out.
Considering the increased risk of CHD (especially fatal CHD) for young FH patients [17], this study confirms the potential added value of genetic screening for individual FH patients over passive identification by GPs. Now, asymptomatic carriers of gene mutations can be closely followed, properly monitored and if necessary treated in order to minimize the risk of CHD.

Acknowledgements

We like to thank M.A.W. Umans-Eckenhausen and staff members of the StOEH for their cooperation during this study. We also thank P.J.E. Bindels, J.C. Defesche, J.J.P. Kastelein and E. Schadé for their comments on earlier drafts of this article. We are grateful for the opportunity to use unpublished data from the MORGEN-project. These were kindly made available to us by D. Kromhout, W.M.M. Verschuren en S. Houterman of the National Institute for Public Health and the Environment.

Reference List


Cost-effectiveness of a family and DNA based screening programme on familial hypercholesterolaemia in The Netherlands

P.J. Marang-van de Mheen, A.H.A. ten Asbroek, L. Bonneux, G.J. Bonsel, N.S. Klazinga

European Heart Journal 2002 23 1922-1930
Abstract

Aims
To estimate the cost-effectiveness of the current screening programme on familial hypercholesterolaemia (FH) in relatives of diagnosed FH-patients in the Netherlands.

Methods and results
Data from 2229 screened FH-relatives, including age, sex, risk factor status and screening outcome, were combined with the Framingham risk function and national disease-specific cost data to arrive at a model-based comparison of survival and costs, with and without the screening programme. Cost-effectiveness ratios were computed for various treatment strategies, with no screening as reference. Costs per life year gained varied between 25,5 and 32 thousand Euros, depending upon the precise treatment strategy after a positive screen. The costs for screening (tracing the FH-positive individuals) were much lower than the follow-up costs (treatment), of which 80% were costs for statins. Consequently, the costs per life year gained of alternative screening programmes are about the same.

Conclusion
The cost-effectiveness ratio of FH screening is within the range requiring explicit political consideration in the Netherlands. As the costs of statin treatment are the single most important determinant of costs, policy decisions reduce to decisions on the acceptability of statin treatment for this risk group. Pending major changes in statin price, clear guidelines should be developed on how screen positive individuals should be treated, since not all of them have an elevated cholesterol level.
Introduction

Statin treatment is now widely used as lipid lowering therapy: the EUROASPIRE II study carried out in 15 European countries in 1999-2000, showed that the proportion of patients taking a statin varied from 30.7% (Greece) to 75.1% (the Netherlands) [1]. In recent years a number of cost-effectiveness analyses have been carried out regarding statin treatment in cardiovascular disease [2-5]. Assuming lifelong simvastatin treatment for individuals at risk, Pickin and others conclude that treatment is cost-effective for individuals with an annual CHD risk of 3% or higher, for whom the costs are £ 8200 per life year gained [5]. Studies so far addressed patients with heterozygous familial hypercholesterolaemia (FH) [6]. Patients of this autosomal dominant genetic disease, with a prevalence of 1 in 500 in most Western countries [7-9], usually develop severe dyslipidaemia, characterised by elevated cholesterol levels (mainly the Low Density Lipoprotein (LDL) cholesterol), often in combination with the presence of tendon xanthomata, corneal arcus, xanthelasmata and a family (or personal) history of early CHD [10]. Patients experience a cholesterol-related increased risk for CHD and subsequent mortality especially at a young age [10;11]. From this, Goldman and others considered low to moderate doses of statins to be efficient for primary prevention in these patients [6].

The discovery of LDL receptor gene mutations in clinically diagnosed FH patients allowed for the subsequent development of DNA diagnostic tests, to be used for suspected FH patients. In view of the much higher risk and the availability of treatment, a family based genetic screening programme for familial hypercholesterolaemia was implemented in the Netherlands from 1994 onwards [12]. The programme targets first and second degree relatives of probands diagnosed with FH and a LDL receptor gene mutation at Lipid Research Clinics throughout the country. These relatives with a 50 and 25% prior probability of FH respectively, are tested for the same LDL receptor gene mutation as found in the FH proband. The screening extends further into the family if new patients are identified. Individuals with a mutation for FH are advised to contact their GP to be referred to a Lipid Research Clinic.

The above screening programme started with government approval in a provisional setting. To decide upon nation-wide implementation of the programme, an evaluation study was carried out including a cost-effectiveness analysis (from 1997 until 2000). This is relevant in particular since not all mutation carriers have an elevated cholesterol level [13], so that part of the screen positives are not likely to experience any health gain due to the screening programme but do account for part of the cost. In the absence of trial data, the effectiveness of the Dutch FH screening programme was estimated by combining data on risk factor status and screening outcomes for a large cohort of FH relatives with the Framingham risk function and national disease-specific cost data, to arrive at a comparison of survival and costs with and without the screening programme.
Methods

Population
Data were subtracted for a closed cohort of individuals screened for a LDL receptor gene mutation in the period 1994-1997, aged 16 years and older. In this period 2814 individuals were screened, of which 363 aged younger than 16 years and 222 who were screened on the apoB mutation, leaving 2229 individuals to be included in the present analysis. These individuals were found through 137 FH probands.

General structure of analysis
Primary outcomes of the analysis were life years gained and life time costs of the screened cohort of relatives, theoretically subjected to various strategies of treatment and to a strategy without screening. Life years gained were estimated assuming that all effects of screening and subsequent treatment are mediated by cholesterol level. Empirical data on the cholesterol level and on the treatment effects in terms of cholesterol reduction, were combined with the Framingham risk function to derive a CHD-specific mortality risk under various treatment strategies. All other causes of mortality were assumed to be unaffected. Life time costs were estimated by combining the empirical data on screening costs, with the estimated life years gained by screening, assuming that these will be years with treatment, and the available age and sex specific Dutch costs data [14]. Both computational procedures are presented in detail below. A lifetime horizon was chosen because 1) the start of statin treatment usually implies lifelong treatment, and 2) the life years gained of a preventive intervention (such as screening) are usually in the future (especially with young individuals) while costs are being made from the start onwards. Therefore the time horizon should be long enough to show the full effect regarding the life years gained.

Calculation of life years gained
The following disease model was assumed.
This model is supported by the following facts:
1. FH, defined as the presence of a LDL receptor gene mutation, is associated with an elevated LDL cholesterol and total cholesterol level [10].
2. An elevated cholesterol level causes CHD and thereby excess mortality [15-17].
The presence of a LDL receptor gene mutation is assumed to carry the increased risk through the elevated cholesterol level. Furthermore, we assumed that the screened population only differed from the general Dutch population on their cholesterol values, but not in any other CHD risk factor (blood pressure, obesity). As a consequence, we assumed that any impact of a screening programme would be observed in CHD mortality, leaving the non-CHD mortality unaltered.

The method used to calculate cumulative survival of the above cohort of individuals with and without screening, has been described before [18]. We first used the Framingham risk function [19] to estimate the CHD mortality risk in the general Dutch population, using the observed average CHD risk factor prevalences in the Netherlands [20-23]. The estimated CHD mortality was then compared with the observed CHD mortality [24], and the Framingham risk function was fitted on the observed CHD mortality by adjusting age. Subsequently, the observed total cholesterol / HDL cholesterol ratios from the screened population were entered in this fitted Framingham function, to estimate the (increased) CHD mortality in the screened population. This was done separately for men and women grouped into 5-year age groups as risk profiles and consequent mortality risks differ between each of these groups. The mortality risks calculated with the Framingham function were then added to the observed non-CHD mortality risk in the general Dutch population [24, 25], and the resulting total mortality risk was entered into a life table to calculate the life expectancy at the time of screening. Calculations were repeated for the situation in which part of the population is treated with statins, causing a lowering of the cholesterol level in this group. The difference in the life years lived by the cohort in the untreated and the treated situation is the number of life years gained due to screening.

Cholesterol levels were known for 1295 individuals out of the 2229 screened. The lack of cholesterol data for other screenees was due to financial reasons (it was no longer covered by the programme), and we therefore could not see any reason why it would give rise to any systematic bias. We therefore applied the calculations regarding the years of life gained among the 1295 individuals, to the other screenees without cholesterol data, thereby assuming that the cholesterol distribution in these individuals would be the same.

Since no clear guidelines exist at this point with respect to eligibility for treatment for the screened population, different treatment strategies were considered:

1. All individuals with a mutation for FH are eligible for treatment.
2. All individuals with a mutation for FH and a cholesterol level above the 95th percentile of the general Dutch population are eligible for treatment.
3. All individuals with a mutation for FH that fulfil the treatment criteria in the national CBO consensus guideline on hypercholesterolaemia [26], are eligible for treatment.
4. See 1. but only if untreated at screening.
5. See 2. but only if untreated at screening.
6. See 3. but only if untreated at screening.

For all strategies we assumed that all persons with a mutation for FH were adequately treated, once traced by the screening programme, without side effects or non-compliance, causing a
proportional reduction in cholesterol level that persists lifelong (on average 21% reduction of total cholesterol level and 5% increase of HDL cholesterol level) [27]. This was done by using the observed (cross-sectional) cholesterol ratios in the screened population to calculate the difference in cholesterol ratio with the general Dutch population at screening. This difference was then assumed to remain throughout life (thus assuming lifelong treatment and 100% compliance). Lifelong treatment was defined as treatment until 85 years of age, since treatment at higher ages does not seem to be indicated.

**Calculation of costs**

We distinguished between the screening process (tracing the individuals) and the follow-up process, including the direct medical costs and the indirect medical costs during the life years gained due to treatment. We used actual costs when possible.

The cost components for the screening process are: a DNA test in FH probands to find the mutation for FH to enable the family based screening, the programme costs of the family based screening (administration, field work personnel), and DNA tests for all individuals screened for the mutation as found in the FH proband. For the FH probands the national tariff of a DNA test was assumed to reflect the actual costs.

The cost components for the follow-up process are: a single GP consult, a single consult at a Lipid Research Clinic, a single measurement of the lipid profile, treatment with cholesterol lowering drugs for part of the screenees, and control at the Lipid Research Clinic for part of the screenees. Consult costs and lab measurements were calculated assuming full compliance of FH-positives to the diagnostic and follow up protocol, and using published unit costs.

Costs of FH-negatives who go to their GP, because they nevertheless have become worried, or because they wanted to have their cholesterol tested, were taken into account. Based on clinical practice we assumed 4 control visits during the first treatment year, 2 visits during the second treatment year, and 1 visit (for ¾ of the treated persons) and 2 visits (for ¼ of the treated persons) during consecutive treatment years.

The costs for statin treatment were calculated by multiplying the number of extra treatment years by the price of cholesterol lowering drugs per person per year. The number of extra treatment years was estimated from the life years gained due to screening, assuming that these will be years with treatment. To estimate the price of cholesterol lowering drugs, we used the observed frequency distribution of drugs in our study (50% atorvastatin, 45% simvastatin and 5% pravastatin). Similarly, we used the observed average dose as prescribed in 2 leading hospitals (45,9 mg atorvastatin, 30,7 mg simvastatin and 27,6 mg pravastatin).

Since the average dose exceeded the Standard Daily Dose, which is reported annually in the Dutch formulary (Pharmaceutical Compass) issued by the insurance companies, we have assumed that a doubling in dose causes a 1,5 increase in price, based on the price data per tablet. The price is estimated including 4% administration costs and 4,97 Euros per prescription line, assuming 1 prescription per year.

To estimate the induced costs during the life years gained, the number of deaths prevented due to screening were multiplied by the average direct medical costs per death [14]. This was
done separately for men and women, and for each 5-year age group. Since screening may shift mortality to higher ages and to other causes of death, we distinguished between CHD mortality and non-CHD mortality. This shift may have consequences in terms of costs for CHD that are saved, but are spent on other diseases.

**Technical cost assumptions**
Cost data were collected in the period 1994-1998. Since both the costs of the programme and the number of individuals screened by the programme increased considerably in this period, we calculated the programme-related costs per screenee in 1998 and applied these costs to all screenees to standardize scale effects.
Costs are presented as differential costs (screening minus non-screening). Cost results are presented without discounting. Prices are expressed in Euros. One Euro equals 0,62 British pounds (currency exchange date 18-03-2002).

**Effect and cost estimations in an alternative screening programme**
The considered alternative screening programme differs from the current screening strategy by taking the lipid profile into account. The following treatment strategies will be considered:
1. All individuals with a cholesterol level above the 95th percentile of the general Dutch population, are eligible for treatment.
2. All individuals that fulfil the treatment criteria in the national CBO consensus guideline on hypercholesterolaemia [26], are eligible for treatment.
3. See 1. but only if untreated at screening.
4. See 2. but only if untreated at screening.
The calculation of effects and costs for this alternative screening is done in the same way as for the current screening programme.

**Sensitivity analysis**
The relative importance of our key assumptions was checked through sensitivity analysis.
1. Family history (first degree CHD death before age 60) was not systematically recorded in our study, and ignored in our baseline analysis. In a sensitivity analysis we assume that all screenees have a positive family history, which implies an increased absolute CHD risk and therefore a changed eligibility for treatment according to the Dutch cholesterol consensus.
2. Our Framingham based CHD survival estimates rest on a risk set of which cholesterol level is but one. In the absence of complete detailed data on other risk factors, our baseline analysis assumed the average risk factor levels as have been published for the general Dutch population. In a sensitivity analysis we assume all screenees to have a 20% higher blood pressure compared with the general Dutch population.
3. The baseline analysis assumed that treatment would lead to an average lowering of the cholesterol level as reported in the clinical trials. However, these trials did not include FH patients, so that the generalisation of this quantitative response to our cohort is uncertain.
In a sensitivity analysis we therefore assume that treatment leads to a reduction in the cholesterol level of 30% and an increase in the HDL cholesterol of 6% (instead of 21% and 5% respectively in the baseline analysis), which should be regarded as the most optimistic variant given the observed reductions in a trial [27].

Table 1 Costs of current screening programme

<table>
<thead>
<tr>
<th>Cost components</th>
<th>N</th>
<th>Unit price</th>
<th>Costs</th>
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<td>Screening</td>
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<tr>
<td>Programme</td>
<td>288 484</td>
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<tr>
<td>DNA test on mutation</td>
<td>2229</td>
<td>125.70</td>
<td>280 179</td>
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<tr>
<td>Multiple DNA test</td>
<td>137</td>
<td>540</td>
<td>73 980</td>
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<tr>
<td>Total screening</td>
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<td>642 642</td>
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<tr>
<td>Follow-up</td>
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<tr>
<td>Single consult GP</td>
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<tr>
<td>FH+</td>
<td>759</td>
<td>16.59</td>
<td>12 596</td>
</tr>
<tr>
<td>FH-</td>
<td>121</td>
<td>16.59</td>
<td>2 000</td>
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<tr>
<td>Single consult specialist</td>
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<tr>
<td>FH+</td>
<td>759</td>
<td>72.60</td>
<td>55 112</td>
</tr>
<tr>
<td>FH-</td>
<td>50</td>
<td>72.60</td>
<td>3 629</td>
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<tr>
<td>Single lipid profile measurement</td>
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<tr>
<td>FH+</td>
<td>759</td>
<td>14.79</td>
<td>11 229</td>
</tr>
<tr>
<td>FH-</td>
<td>213</td>
<td>14.79</td>
<td>3 153</td>
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<tr>
<td>Cholesterol lowering drugs</td>
<td>29 119</td>
<td>714.46</td>
<td>20 804 347</td>
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<td>Controls during treatment years</td>
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<td>1st year</td>
<td>3 036</td>
<td>72.60</td>
<td>220 447</td>
</tr>
<tr>
<td>2nd year</td>
<td>1 518</td>
<td>72.60</td>
<td>110 224</td>
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<tr>
<td>3rd year and onwards</td>
<td>34 501</td>
<td>72.60</td>
<td>2 504 929</td>
</tr>
<tr>
<td>Costs during life years gained</td>
<td></td>
<td></td>
<td>2 678 255</td>
</tr>
<tr>
<td>Total follow-up</td>
<td></td>
<td></td>
<td>26 405 920</td>
</tr>
<tr>
<td>Total screening + follow-up</td>
<td></td>
<td></td>
<td>27 048 563</td>
</tr>
</tbody>
</table>

Number screened: 2229
Number FH+: 759
Number treated: 759
Screening costs per screenee: €288
Screening costs per FH+: €847
Follow-up costs per treated individual: €34 787
1 Costs for follow-up in case of strategy 1: treat all FH+
2 N refers to the number of treatment years for all individuals treated.

Price is calculated per treatment year.

Results

Cost-effectiveness of current screening

Table 1 shows the costs of the screening and the follow-up process in the current screening. The total screening costs are approximately 640 thousand Euros. Per individual screened the screening costs are 288 Euros.
Furthermore, the follow-up costs are shown according to the first screening strategy (treat all FH positives). In this strategy 26 million Euros are spent on treatment for the screened population, which considerably exceeds the screening costs. The costs per person treated are almost 35 thousand Euros. About 80% of the total costs are costs for cholesterol lowering drugs. The reason that additional costs are being made during the years of life gained is that although CHD events are prevented due to treatment, thereby saving CHD-related costs, these individuals survive and have other diseases at older ages, thereby increasing the on average more costly non-CHD related costs. Table 2 shows the balance between costs and years of life gained for various treatment strategies.

Table 2 Difference in cost-effectiveness between treatment strategies (current screening)

<table>
<thead>
<tr>
<th>Treatment strategy</th>
<th>N treated</th>
<th>Costs</th>
<th>Years of life gained</th>
<th>Costs per year of life gained</th>
</tr>
</thead>
<tbody>
<tr>
<td>All FH+</td>
<td>759</td>
<td>27 048 563</td>
<td>865</td>
<td>31 260</td>
</tr>
<tr>
<td>FH+ with elevated cholesterol level</td>
<td>461</td>
<td>18 247 529</td>
<td>610</td>
<td>29 918</td>
</tr>
<tr>
<td>FH+ as in cholesterol consensus</td>
<td>265</td>
<td>9 251 537</td>
<td>361</td>
<td>25 613</td>
</tr>
<tr>
<td>Untreated FH+</td>
<td>430</td>
<td>16 704 039</td>
<td>519</td>
<td>32 164</td>
</tr>
<tr>
<td>Untreated FH+ with elevated cholesterol level</td>
<td>303</td>
<td>12 554 834</td>
<td>407</td>
<td>30 843</td>
</tr>
<tr>
<td>Untreated FH+ as in cholesterol consensus</td>
<td>133</td>
<td>5 637 424</td>
<td>204</td>
<td>27 700</td>
</tr>
</tbody>
</table>

The costs per year of life gained vary between 25,5 and 32 thousand Euros. The lowest costs per year of life gained are made if treatment follows the Dutch cholesterol consensus guideline. These guidelines are based on a cut-off point of 18.151 Euros per year of life gained. The costs being higher here is due to 1) the costs during the years of life gained are not taken into account in the consensus calculations, and 2) relatively more young individuals in the screened population are eligible for treatment due to their highly elevated cholesterol level. The higher costs in the other strategies are due to more young individuals being treated. At these young ages the absolute mortality probabilities are small, so that the number of life years gained due to treatment (in absolute terms) is small, against the high costs of lifelong treatment.

Cost-effectiveness of alternative screening

Additional cholesterol level measurement in the screening results in about similar cost-effectiveness (table 3).

Table 3 Cost-effectiveness between treatment strategies in case of alternative screening

<table>
<thead>
<tr>
<th>Treatment strategy</th>
<th>N treated</th>
<th>Costs</th>
<th>Years of life gained</th>
<th>Costs per year of life gained</th>
</tr>
</thead>
<tbody>
<tr>
<td>All with elevated cholesterol level</td>
<td>658</td>
<td>25 030 932</td>
<td>836</td>
<td>29 957</td>
</tr>
<tr>
<td>All as in cholesterol consensus</td>
<td>406</td>
<td>12 368 034</td>
<td>507</td>
<td>24 376</td>
</tr>
<tr>
<td>Untreated with elevated cholesterol level</td>
<td>489</td>
<td>19 038 280</td>
<td>623</td>
<td>30 558</td>
</tr>
<tr>
<td>Untreated as in cholesterol consensus</td>
<td>263</td>
<td>8 555 188</td>
<td>337</td>
<td>25 360</td>
</tr>
</tbody>
</table>
Although more individuals are treated, resulting in higher costs, more life years are also gained, resulting in similar cost-effectiveness ratios. Note that the cost-effectiveness ratios of comparable treatment strategies are similar or better in table 3 compared with table 2, due to the fact that relatively more elderly individuals are being treated. The absolute mortality probabilities are higher at older ages, so that treatment leads to a greater increase in the total number of years of life gained. On average these individuals are treated for a shorter amount of time because of their shorter remaining life-expectancy, resulting in lower costs and thereby in a more favourable cost-effectiveness ratio.

**Sensitivity analyses**

Table 4 shows the results of the sensitivity analysis.

<table>
<thead>
<tr>
<th>Strategy</th>
<th>Baseline analysis</th>
<th>Sensitivity analysis (1)</th>
<th>Sensitivity analysis (2)</th>
<th>Sensitivity analysis (3)</th>
</tr>
</thead>
<tbody>
<tr>
<td>FH+ as in cholesterol consensus</td>
<td>25 613</td>
<td>25 312</td>
<td>19 636</td>
<td>19 328</td>
</tr>
<tr>
<td>Untreated FH+ as in cholesterol consensus</td>
<td>27 700</td>
<td>27 330</td>
<td>21 117</td>
<td>20 770</td>
</tr>
</tbody>
</table>

1 Assuming that all screened individuals have a positive family history
2 Assuming that all screened individuals have a 20% higher blood pressure than the general Dutch population
3 Assuming that treatment results in a 30% reduction of total cholesterol level and a 6% increase in the HDL cholesterol, causing a 35% reduction of the cholesterol ratio

The assumption regarding a positive family history does not affect our results. An assumed 20% higher blood pressure in the screened population (thereby increasing the baseline CHD-risk) results in a more favourable cost-effectiveness ratio, as with higher baseline risk the absolute mortality reduction will be greater. Combined with an on average smaller number of treatment years per person, due to the lower remaining life-expectancy, this results in a better cost-effectiveness ratio. An assumed greater effect of treatment on lowering the cholesterol level results in a lower, more favourable, cost-effectiveness ratio. More life years are gained due to the assumed greater effect of treatment. Although these life years will be years with treatment, thereby increasing costs, the relative increase in the number of life years gained is greater than the relative increase in the number of treatment years.

**Discussion**

The present study has shown that the cost-effectiveness of a family based screening programme for FH in the Netherlands is between 25,5 and 32 thousand Euros per year of life gained, depending on the treatment strategy after a positive screen. The screening costs were much smaller than the follow-up costs, of which 80% consisted of costs for cholesterol
lowering drugs. Consequently, the cost-effectiveness of an alternative screening including cholesterol level measurement is about the same.

In the Netherlands systematic screening of serum cholesterol levels is not recommended by national guidelines [26]. Screening for the phenotype (increased serum cholesterol level in affected families) might be an alternative strategy for screening for the genotype (the affected gene). We limited our analysis of screening for the genotype to comparisons with the existing policy (no screening). No data exist about the efficiency of such alternative policies. Analysing costs and effects of these alternative screening policies require a separate analysis, going beyond the scope of this paper.

A model-based approach was used in this study because of lack of available longitudinal data concerning the screened population. The Framingham risk function that was used has been shown to be applicable to a European population [28]. Furthermore, this risk model was used since it contains the cholesterol level as one of the risk factors, and since the screened population mainly consists of asymptomatic carriers who for their cholesterol levels are at CHD risk much alike non-FH patients of similar age and lipid profile. Evidence is lacking concerning more damage and/or higher risks of FH mutation carriers compared with age and cholesterol matched counterparts without FH e.g. due to a greater number of cholesterol years. Jensen et al have shown that a model using the cholesterol-year score to predict the CHD risk in FH patients has the worst correlation with vessel damage [29]. A model with traditional risk factors did not predict the CHD risk very well, but gave a better prediction than the model with the cholesterol-year score. Therefore, the model we used containing all traditional risk factors seems to be the best alternative available.

However, extrapolating the Framingham function to younger and older ages may cause bias. The Framingham function was based on persons aged 30 to 55 years without pre-existing cardiovascular disease at the start of the study. Stamler et al have recently shown that the relationship between cholesterol level and CHD at younger ages is comparable to the relationship in the age group 30 to 55 years of age [30], so that the extrapolation of the Framingham function to younger ages seems justifiable. Extrapolation to older ages seems more hazardous since the relationship between the cholesterol level (or other risk factors) and CHD attenuates at older ages [31;32]. As a result we might have overestimated the life years gained due to screening.

Sensitivity analysis showed that the life years gained may have been underestimated if the effectiveness of treatment in the screened population exceeds the average effectiveness reported in clinical trials[27]. A greater reduction is found in trials carried out in populations with pre-existing cardiovascular disease and a higher baseline cholesterol level (e.g. 26% reduction in the 4S study), compared with trials carried out in populations mainly without cardiovascular disease and a lower baseline cholesterol level (e.g. 20% reduction in the WOSCOPS study). With respect to the presence of cardiovascular disease and baseline cholesterol level, the screened population resembles the latter population more, although the cause of an elevated cholesterol level in the screened population is likely to be quite different. Furthermore, the question is whether the effect of statin treatment as found in the trials is an
accurate estimate of the lifetime impact, given the limited follow-up of the trials (smaller than 10 years). No evidence exists on the effect of statin treatment beyond 10 years. From available knowledge on the way its effects are mediated, the extrapolation based on trial effects seems rational and defendable. In particular, no signs exist on attenuation of its effects. Therefore, it does not seem likely that we underestimated the life years gained.

An issue of general concern is the implicit assumption that the CHD risk is related to a certain cholesterol level, regardless of whether this cholesterol level is the result of treatment or not. We are not aware of any study that has investigated this issue directly. However, since the reduction in mortality risk as found in the trials is greater than the reduction expected based on observational studies [33], this might be regarded as indirect evidence that we have underestimated the risk reduction in the present study in this respect.

On the other hand, our compliance assumptions (100% follow-up, 100% treatment and 100% compliance) were probably too generous. In another study we found that 87% of the individuals with a mutation for FH consult a medical doctor due to the screening and 82% have their cholesterol level checked [34;35]. However, only 21% started taking cholesterol-lowering drugs after the screening, while the remainder was already taking these when they were screened. The compliance was estimated to be 90% rather than 100%. This will have overestimated the absolute number of life years gained. Furthermore, this compliance assumption may also have overestimated the costs if it would mean that individuals get fewer pills on a yearly basis (from the pharmacist) than prescribed because they sometimes forget taking them. The result of both effects on the cost effectiveness ratio is difficult to calculate since it will depend on how often individuals forget. Furthermore, assuming a different percentage of compliance brings up the question who is compliant and who is not. Are individuals at higher risks more compliant or not? And what is the effect on their cholesterol level if individuals are temporarily not compliant? Since we did not have individual data on compliance for the population studied, other assumptions could not be explored.

We assumed that the screened population only differed from the general population in their cholesterol levels, and not in e.g. other risk factors. This assumption followed from our main assumption that the increased CHD risk is entirely determined by the raised cholesterol level. In that context it seems likely that the average level of other risk factors is not different from that in the general population. For smoking this is supported by data from another study on the same screened population [34]. Since we did not have individual data on other cardiovascular risk factors, this could not be checked empirically. We have tested the effect of this assumption on the cost effectiveness ratio through sensitivity analyses, which showed a more favourable cost-effectiveness in case of a 20% higher average blood pressure in the screened population. Further research is needed to investigate to what extent the screened population has a different risk profile apart from the cholesterol level.

The results were presented without discounting. Discounting is appropriate if costs and effects show different flows in future time. However, in this cohort lifetable model, both costs and effects are determined by the same dimension: future life years gained. As this
same dimension is discounted both in numerator and denominator, the effect of discounting on the cost-effectiveness ratio is marginal.

Other studies have shown different results with respect to cost-effectiveness. The study of Goldman did also use a model based on the Framingham risk function, but considered lovastatin rather than the newer statins used in this study, and defined FH patients as persons with an extremely elevated CHD risk (equivalent to the risk of persons with a cholesterol level of 15,6 mmol/l) [6]. Also lovastatin was added as primary prevention to already available secondary prevention measures. Pickin et al have shown more favourable cost-effectiveness ratios of lifelong treatment with statins than the present study [5]. Part of the explanation is that only the saved CHD-related health care costs were taken into account but not the shift of costs to older ages and non-CHD causes. When assuming lifelong treatment, the same perspective should also be applied when assessing the health care costs saved. Excluding the costs during the life years gained in our study, resulted in estimated costs of between 23 and 29 thousand Euros per year of life gained, depending on the treatment strategy. Although the difference is smaller, these estimates are still higher. This may have been caused by the younger average age in our screened population (38 years in case of the first treatment strategy compared with 55 to 58 years in the study of Pickin et al). Another reason may be that the screened individuals have fewer other CHD risk factors. Sensitivity analysis showed that a higher baseline CHD risk would lead to a more favourable cost-effectiveness ratio.

Within a couple of years some statins will be out of patent, which would lower the costs of statins and would thereby result in a more favourable cost-effectiveness ratio. However, history has shown that pharmaceutical companies usually have a new drug (a statin in this case) ready, which is in fact more expensive than its predecessor. We therefore doubt that in the long run this would lower the statin costs.

In conclusion, the cost-effectiveness ratio of this family based screening on FH exceeds the cut-off point of 18,151 Euros per year of life gained as set by the Dutch cholesterol consensus guideline. It therefore requires explicit political consideration. As the costs of statin treatment are the single most important determinant of costs, policy decisions reduce to decisions on the acceptability of statin treatment. Pending major changes in statin price, clear guidelines should be developed on how screen positive individuals should be treated, since not all of them have an elevated cholesterol level. If a mutation for FH does not add to the cholesterol-related CHD risk, mutation carriers should be treated depending on their cholesterol level. Since evidence is lacking in this respect, a final answer cannot be given. For now it seems best to treat screened individuals based on their cholesterol level. Following the guidelines from the Dutch cholesterol consensus and cost-effectiveness considerations, this may exclude many young individuals from treatment although their health gain due to treatment may be much higher than in many other screening programmes. A possibility may be to monitor their cholesterol level regularly and to start treatment when the risks become higher.
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Health services research at work in Case I
The first case of health services research at work for national health policy concerned the genetic screening programme for familial hypercholesterolaemia (FH) in the Netherlands implemented by the Foundation for the Identification of Persons with Familial Hypercholesterolaemia (StOEH). The initiative for an evaluation was developed by the StOEH together with health services researchers and focused on the costs and effects of the screening programme. The Ministry of Health, Welfare, and Sport (MoH) used the results in the decision-making process regarding the implementation of the screening as a national programme. One of the initial content questions regarded the cost effectiveness of the screening programme. We developed a combination of four content-, context- and process-focused research questions, in addition to the initial research questions formulated in the research proposal:

1) What is the prevalence of a mutation among screened persons with hypercholesterolaemia and the prevalence of hypercholesterolaemia among persons with an LDL receptor gene mutation (FH+)? (presented in Chapter 2 [1])

2) What proportion of patients with FH were identified with hypercholesterolaemia in general practice prior to genetic screening? (presented in Chapter 3)

3) What is the potential of the current methodology of pedigree analysis and follow-up: How many family members can be reached by the programme and how many have already been reached? [2] and

4) Do screened persons (and persons with a positive screening result) face consequences when applying for insurance because of their participation in the screening programme? [3]

In this case, we presented the results of the first and second additional research question in Chapters 2 and 3 respectively. Furthermore, in Chapter 4 we presented the results of the cost-effectiveness study of the programme [4].

The research group consisted of epidemiologists, public health specialists, medical doctors, a health scientist and a psychologist. The work of the research group was supervised by a scientific committee consisting of senior academic researchers [5]. During the evaluation, in terms of frequency of interaction, our most important counterparts were the staff and initiators of the organization that implemented the genetic screening programme, the StOEH [6]. Besides being a counterpart, the StOEH was also a subject of the evaluation, as our evaluation included its approach, programme costs and results. The evaluation study was contracted by the Netherlands Organisation for Health Research and Development (ZonMw).

**Inclusion of additional research questions**

We used different strategies to pursue all four additional research questions formulated in this evaluation study. It was possible to answer the first additional question – What is the prevalence of hypercholesterolaemia among the screened persons? (Chapter 2) – within the financial boundaries of the initial research proposal. We perceived this study as a necessary content-related intermediate analysis. We received the full cooperation of the StOEH
in linking the results from DNA analysis to the results from cholesterol measurements. No additional data or staff were needed other than those already available.

The second question – What proportion of patients with FH were identified with hypercholesterolaemia in general practice prior to screening? (Chapter 3) – could be addressed through student involvement. A medical student carried out the data collection as part of his obligatory research internship. In addition, the StOEH facilitated in contacting screened persons who were identified as FH+. The StOEH asked these persons for permission to review their medical records in the general practitioner’s (GP) practice regarding FH-related matters, such as cholesterol measurements and hypercholesterolaemia treatment. Once permission was obtained, the StOEH had no further involvement in this study. Supervising the student’s data analysis and reporting of the study results was done within the framework of the initial proposal and as part of the academic training of the medical student. No additional funds or other resources were used.

We developed a third additional question regarding the analysis of the pedigree and the completeness of the family tree drawn up by the StOEH (not shown in this thesis). A graduate student in medical informatics designed a model for analysing the FH+ population that could theoretically be identified with the chosen approach. Supervision was provided by researchers in the evaluation study as well as by an assistant professor in medical informatics as part of the academic training of the student.

The fourth additional research question – Do screened persons and persons with a positive screening result face consequences when applying for insurance because of their participation in the screening programme? [3] – could also be addressed by involving a medical or other student, much like the second and third additional questions. The StOEH facilitated the informed consent and the mailing of questionnaires. Supervision was provided by one of the senior researchers in the evaluation study. No additional funds were needed.

**Interactions with policymakers and other actors**

Our experiences with policymakers and other actors in achieving these additional research goals varied with each sub-study. The interaction in the studies mentioned here focused on the StOEH, members of the steering committee and GPs. The policymakers at the ministry did not interact with the researchers in the beginning of the research period but became involved towards the end, when the results were discussed.

Our analysis of the prevalence of hypercholesterolaemia among screened persons showed that 17% of FH+ persons did not have hypercholesterolaemia. The StOEH received this with some reluctance, as this was perceived as a rather high percentage. The very first response to our finding was, ‘Your data are wrong.’ (Actually, the data had been obtained from the StOEH database.) This response may have been the result of the difference between our findings and the assumptions of the staff involved in executing the screening programme. The policy document for the screening programme stated: ‘FH is a disease that is probably 100% penetrant’ [7]. Our findings challenged the statement about the penetrance of the LDL receptor gene mutations and stressed the importance of measuring serum cholesterol levels.
in addition to a DNA analysis. By working closely together with the initiators of the StOEH on
the research paper presented in Chapter 2, we could present our results of the analysis of the
StOEH database, point out the importance of more research about the genotype-phenotype
mechanisms in FH and at the same time facilitate constructive cooperation between the
health services researchers and those involved in the StOEH.

During the study of the second additional question (What proportion of patients with FH
were identified with hypercholesterolaemia in general practice prior to genetic screening?),
the interaction of the health services researchers (including the medical student) focused on
GPs. We experienced good cooperation and GPs expressed interest in the findings of the
study. The contribution from the GPs was limited to allowing access to their patient records,
which was very often facilitated by their practice assistants.

The interaction during the exploration of the third additional question (What is the potential
of the current methodology of pedigree analysis and follow-up: How many family members
can be reached by the programme and how many have already been reached?) proved more
difficult. As analysis of the genealogic information (collected and owned by the StOEH) was
not perceived by the StOEH as being part of the initial research question, the StOEH had to
approve of this study in a separate decision-making process. Sharing this confidential informa-
tion with a third party (health services researchers) needed utmost consideration. A document
in which we presented the importance of the analysis for the evaluation of the programme
and in which we proved to be able to transform a confidential, family-specific database into
an anonymous database failed to persuade the StOEH [8]. The StOEH decided not to approve
this study on legal grounds (the informed consent did not cover linking pedigree information
and DNA diagnoses) and quality grounds (the pedigree database could not be guaranteed to
be of sufficient quality for the analysis). After intensive communication and explanations, we
had to abandon the plan to study this aspect of the screening programme [9]. The student
graduated with a thesis on the theoretical approach of the pedigree analysis and the analysis
of a test database that contained a mock family tree [2].

Studying the consequences of screening when applying for insurance (additional research
question 4) was well received by both the StOEH and policymakers. A patient-support organi-

Health services research at work in Case I
zation dealing with issues related to insurance and labour contributed to the study [10]. This
organization provided data on the number of complaints their help-desk had received regard-
ing genetic testing and applying for insurance in the years prior to our evaluation. The study
showed (data not shown in this thesis) that participants of a genetic screening programme still
encounter unanticipated insurance problems. It is not clear why these problems occur: whether
insurance companies ask questions regarding genetic testing or questions that can be inter-
preted as such, or whether individuals themselves give more information than they have been
asked for. The researchers concluded that guidelines and legislation on genetic information are
just a prerequisite and that education of all those involved is equally important [3].

The interaction with the StOEH and its initiators was most explicit when studying the cost
effectiveness of the screening programme, the content question in this case (Chapter 4).
Whereas the screening programme was developed and implemented from the perspective
of microbiologists and internal medicine specialists, the evaluation study was developed from the perspective of public health (rather than the health of the individual patient): what are the costs and effects for the total population, the population of screened persons and the population of persons at risk of having familial hypercholesterolaemia? We consequently chose what health economists call the societal perspective for the cost-effectiveness analysis [11]. Our technical approach of the analysis was debated by those involved in the StOEH. To create an opportunity to discuss the findings, a workshop was held at the initiative of ZonMw [12]. Horstman [13] studied the evolvement of the debate on the implementation of the screening programme and she stressed the importance of the different perspectives from the involved actors, including our health services research group and the StOEH. This difference is also shown in another cost-effectiveness study of the same screening programme published by a group of researchers from the UK and the StOEH [14]. Their analysis showed a cost of $8,700.00 per life year gained, compared to $32,000.00 in our study. (At the time, the exchange rate for dollars to euros was 1:1.) In both analyses, the risks of cardiovascular disease mortality were modelled using different studies on which these models were based. As a consequence, neither of the two cost-effectiveness analyses represents ‘reality’. The difference between the two cost-effectiveness ratios is primarily based on three factors. Firstly, in our study we chose a population model (based on persons from the general population with unknown FH status) for calculating the risk of cardiovascular disease (CVD) mortality [15]. Wonderling et al. chose a model based on the mortality risk in a cohort of clinically diagnosed FH patients (with clinical symptoms of hypercholesterolaemia) documented in the Simone Broome Register [16]. This resulted in a lower risk estimation and fewer life years gained in our study. Secondly, we estimated the costs of treatment till the age of 85 instead of 60, as in the study by Wonderling et al. This led to a higher estimated cost of treatment in our study. Thirdly, we included the costs of diseases (other than CVD) that would occur in the life years gained after the screening. This debate of perspectives, methods and techniques, including the cost-effectiveness publications and an accompanying editorial [17], indicates not only that evaluation perspectives differed, but also that actors are convinced that scientific arguments contribute to the decision-making process on the funding and continuation of the programme.

**Follow-up of events and developments**

Several developments have occurred since the publication of the evaluation report [5] and the workshop on the evaluation results [12]. Firstly, the results from the study on getting insurance after screening for FH [18;19] received much attention in the media (e.g. [20;21]) and led to questions being asked in parliament [22;23]. As a result, the minister for Health, Welfare and Sport initiated further investigations by the Health Council of the Netherlands [24;25] and delayed the decision on continuation and expansion of the screening programme [26]. Depending on agreement with the insurers about the protocol for insuring FH patients [27], the minister decided that although the costs per life year gained were high (as we had shown), the importance of avoiding the health effects of FH weighted heavier. Therefore,
the decision was taken to continue the programme and incorporate it into the general health care package covered by the Dutch Sickness Fund [27]. The Health Care Insurance Board (CVZ) was appointed to coordinate the nationwide implementation of the programme. A scientific expert committee was appointed to advise the CVZ [28].

Secondly, in December 2004 the CVZ defined an additional evaluation of the acceptability of the programmes’ approach, and of the potential negative consequences of a genetic diagnosis on obtaining insurance [29]. These evaluation studies were carried out in 2005 [30].

Thirdly, the importance of further studying the risk for cardiovascular disease in persons with a genetic mutation for the LDL receptor gene but without an increased LDL level was formulated by the Health Council of the Netherlands [25], supporting our findings presented in Chapter 2 [1].

Fourthly, based on the findings of our evaluation, we recommended considering the inclusion of the treatment of FH in the new clinical practice guidelines on cardiovascular risk management for GPs [31]. Attempts to achieve this led to a separate publication of a specific guideline for diagnosis and treatment of FH [32] and a position statement regarding this issue from the Dutch Association of General Practitioners NHG [33].

In 2005, the coordination of the screening for familial hypercholesterolaemia was transferred from the CVZ to the National Institute for Public Health and the Environment (RIVM), where the coordination of all national screening and prevention programmes is positioned [34]. It is unclear whether this shift in coordination leaves the follow-up of the recent evaluation results [30] as well as the overall scientific guidance of the screening programme in a vacuum.

In conclusion, the developments regarding FH screening in the Netherlands show that the issue is no longer whether or not a family- and DNA-based screening is desirable, but rather how the conditions under which the programme is implemented can be redesigned. With the transfer of the coordination of the screening programme from the research-based StOEH to the Health Care Insurance Board and again to the RIVM, the screening programme (and the discussion about it) has been put firmly into the domain of public health services. Our health services research has contributed to this evolution by producing relevant scientific knowledge, by interaction and by open debate with other scientists and involved actors. Several results of our efforts have been incorporated into the policymaking process, which led to the health minister’s decision to expand the screening programme, continue its evaluation and encourage further research.

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Nationwide implementation of respiratory care guidelines?
Implementing global knowledge in local practice: a WHO lung health initiative in Nepal

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Abstract

Clinical practice guidelines are widely used to improve the quality of primary health care in different health systems, including those of low-income countries. Often developed at international level and adapted to national contexts to increase the feasibility of effective uptake, guideline initiatives aim to transfer global scientific knowledge into local practice. The WHO’s Practical Approach to Lung Health¹ (PAL) is an example of such an initiative and is currently being developed to improve quality of care for youths and adults with respiratory diseases. We assessed ex-ante the feasibility of successful implementation of PAL in a pilot programme in rural Nepal, studying three components: the quality of the innovation (i.e. the guideline), the effectiveness of the implementation strategy (i.e. training) and the receptiveness of the social system of health staff at all levels (i.e. social and organizational characteristics). We assessed the guideline innovation with the AGREE instrument for guidelines, the intended implementation strategy by critical comparison with literature on effective strategies, and the social system with both a stakeholder analysis and a descriptive analysis of the health care system at district level.

This ex-ante assessment of an adaptive local implementation of international WHO guidelines showed that in July 2002 the ‘implementability’ of the package was challenged on the three components studied. To increase the chances of successful implementation, the national guideline development process should be improved and the implementation strategy needs to be upgraded. In order to successfully transfer global knowledge into local practice, we need to develop additional multifactorial sustained interventions that tackle other culture- and health system-specific barriers as well. The primary health workers are key informants for these barriers.

¹ Practical Approach to Lung Health (PAL) was initially titled Adult Lung Health Initiative. The name was changed in 2001.
Introduction

Multilateral efforts to improve the quality of primary health care in varying national systems often include the development and introduction of standard clinical practice guidelines. This process typically starts out with the development of generic tools at global level as can be seen in a variety of programmes for essential drugs and disease control [1-3]. The tools are not health system-specific and hence, the potential for instantaneous implementation in many health systems is limited. Consequently, generic tools are meant to be adapted to the specific local context. Overall, the ultimate challenge lies in transferring global scientific knowledge into local practices. Especially in low-income countries, these circumstances are frequently characterized by a chronic lack of sufficiently competent health workers and other limited resources.

Success or failure in the promotion of changes in clinical practice in developed countries with well-trained professionals depends on the characteristics of the implementation process [4-6]. Given the multitude of international initiatives and further globalization of efforts, there is a need for more systematic study of how generic guidelines are implemented through national health systems in developing countries.

This paper takes the example of the ‘Practical Approach to Lung Health’ (PAL),1 a World Health Organization (WHO) initiative to improve the syndromic management of lung diseases in youths (over 5 years of age) and adults in middle- and low-income countries [7]. PAL is presented as a package that consists of a generic clinical practice guideline and accompanying training materials. It targets the multi-purpose health worker at peripheral primary and

Box 1 Description of generic and Nepal specific PAL guideline development

The global, generic PAL guideline was designed and developed at supranational level by expert panels on the initiative of WHO in 1997-1998. [16] The guideline contains algorithms that follow a syndromic approach of disease. Primary care health workers with little training in lung health receive guidelines that comprehensively cover respiratory disease case management. The guideline guides the health worker stepwise through the assessment of a patient and results in a classification. A specific classification leads to specific management and, if necessary, to treatment. The clinical algorithms are presented as flow-charts in topic specific modules. The complete document is 60 pages in length and includes recommendations for follow-up and counselling. Expected outcomes include an increase in rational use of drugs, adequate referrals, shortening of delay in TB diagnosis and treatment, decrease in number and severity of asthma attacks in chronic patients.

The local, context specific PAL-NEPAL adaptation and implementation on the basis of the generic PAL guideline started in November 1999. Nepalese experts and potential stakeholders from the national, regional, district and local health system participated in the Adaptation Working Group (AWG) to produce a context specific PAL guideline. Among the twenty-three participants were eighteen senior health officials representing governmental health offices, clinical departments of tertiary hospitals and academics. Five participants represented the peripheral health care level: 2 district health officers, 2 hospital-based health workers and 1 health worker from a Primary Health Care Centre. The AWG focused its work on the treatment of diseases and specifically addressed the perceived problems with recommended drug therapies in the generic guideline. Their findings and suggestions were presented in October 2001 [18]. This concluded the adaptation into the PAL-NEPAL guideline that was now ready for its first pilot implementation that started in July 2002 in the rural low-land district Nawalparasi.
secondary care facilities. In several countries, elements of PAL are currently being field tested and evaluated [8].

In Nepal, the global, generic PAL package was adapted to the specific Nepalese health care context as part of the guideline development process and also to facilitate its further introduction (Box 1) [9-12]. A pilot implementation of the adapted PAL package, called PAL-Nepal, started in governmental primary health care centres, health posts and sub health posts of Nawalparasi, a rural lowland district (Box 2). The pilot implementation of PAL-Nepal is subject to an evaluation of effects, including costs, organizational effects and health outcomes, using a cluster randomized trial design. Also, it is subject to a qualitative assessment of the development and implementation process itself.

This article presents the latter of the two studies and answers the research question: ‘What are the chances of successful implementation of PAL-Nepal given the characteristics of the guideline, the planned implementation strategy and the social system?’.

The study was carried out by external researchers (AtA, LN, CvdH) who were observers during PAL-Nepal activities and had no active involvement in its development and implementation.

**Methods**

Adapted from Rogers [13], we identified and assessed three components in the development and implementation process of PAL-Nepal: the innovation (the guideline), the implementation strategy (training), and the social system (the social and organizational context of health
workers at the various levels in the Nepalese health care system. We perceived that successful implementation depends on the quality of the innovation itself, the effectiveness of the implementation strategy and the receptiveness of the social system in conjunction with each other. The first two components are elements of the new health policy and the latter determines the context for adoption and diffusion. For each component we selected specific assessment instruments.

**Assessment of innovation**

We used the generic and standardized Appraisal of Guidelines Research & Evaluation (AGREE) Instrument [14] to assess whether one may expect the guideline to be effective in producing the desired changes in outcomes. The AGREE instrument appraises the methods used for developing the guidelines, the content of the final recommendations, and the factors linked to their uptake.

The material for this assessment consisted of the background documents for the generic PAL clinical practice guideline [15;16], the minutes of the adaptation working group meetings held in Nepal [17;18], personal observation reports and the context-specific PAL-Nepal algorithms. Three independent reviewers familiar with developing and assessing clinical practice guidelines assessed the PAL-Nepal documents with the AGREE instrument. For multi-rater Kappa analysis we used SPSS statistical software and additional macros [19;20]

**Assessment of implementation strategy**

For the implementation strategy we established whether — given the available evidence from international literature — we expected the strategy offered to be effective. To assess this, we critically analysed the planned implementation strategy using the framework suggested by Hulscher et al. [21], which is based on evaluation research literature and theories on implementation and behaviour change, and on the data collection checklist developed by the Cochrane collaboration [22]. Additionally, we compared the planned implementation strategy with characteristics of strategies that enhance effective implementation as summarised by Grol and Grimshaw [23;24], Grimshaw et al. [6] and Hulscher et al. [25].

Input for this analysis was derived from the minutes of the Adaptation Working Group meetings [17;18], training plans and training manuals.

**Assessment of the social system**

Finally, we assessed the receptiveness of the social and organizational context of health workers at the various levels in the Nepalese health care system. We perceived the social system at national level foremost as a political system and carried out a stakeholder analysis to assess the feasibility of the political acceptance of PAL-Nepal [26;27]. For this analysis, three respondents (senior health officials) rated the stakeholders’ position towards the PAL-Nepal goals and mechanisms and the anticipated power of the stakeholders to influence the achievement of these goals.
In addition, we described factors at district and local level that influence the system’s ability to take on central guidance. Input came from the literature, discussions with health care workers and personal observations during meetings at national and district/local level in 2001 and 2002 (AtA, LN, RS), the researcher’s diary (AtA) and e-mail communication during the research period 2000 – 2002 (LN, AtA) and field visits totalling seven months over a period of two years.

**Time frame of assessment**
The first phase in the development and implementation of PAL-Nepal started in November 1999, building forth on the work of the international expert meetings that started the construction of the generic PAL package. It ended in July 2002, when the implementation phase started, just before the actual introduction of the PAL-Nepal guideline package to the targeted health workers, which marked the start of the implementation phase. The time locus for this paper is July 1, 2002.

**Results**

**Innovation**
The results of the assessment of the PAL-Nepal guideline with the AGREE instrument are shown in Table 1. The ‘Comments of the reviewers’ reflect the specific findings per item of the AGREE instrument. The inter-rater agreement was low: multi-rater Kappa was 0.29. We hypothesized that part of the variation could be explained by variation in ‘agree’ and ‘strongly

**Table 1** Quality of PAL-NEPAL guidelines by domain [14]

<table>
<thead>
<tr>
<th>Domain</th>
<th>Reviewers’ comments</th>
<th>Summary Domain Score (0-100%)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Scope and purpose</strong></td>
<td>The overall objectives of the guideline are described. The clinical questions covered by the guideline are specifically described. The patients to whom the guideline is meant to apply are described. The guideline does not describe specifically the social and geographic characteristics of the target population.</td>
<td>78%</td>
</tr>
<tr>
<td>(Overall aim of the guideline, the specific clinical questions and the target patient population)</td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Clarity and presentation</strong></td>
<td>The recommendations in the PAL-Nepal guidelines are specific and unambiguous, and the options for management of the condition are clearly presented. Alternative treatment options are rarely given. It is difficult to identify the key recommendations in this extensive document package. The guideline is supported with tools for application.</td>
<td>69%</td>
</tr>
</tbody>
</table>
### Domain Reviewers’ comments Summary Domain Score (0-100%)

**Stakeholder involvement**  
(The extent to which the guideline represents the views of its intended users)  
The target users, i.e. health professionals from rural health facilities, did not participate in the development process. Professionals from higher professional groups were represented in the development groups of both the generic and country-specific guidelines. Patient views were not explicitly included in the guideline development process. The target users of the guideline are clearly defined and the guideline is piloted among target users.  
56%

**Applicability**  
(The likely organizational, behavioural and cost implications of the guideline)  
The potential organizational barriers in applying the recommendations have been discussed in the generic guideline document in a general manner. Specific mentioning of organizational barriers did not occur in the PAL-Nepal guideline. The cost implications are subject of study in the first pilot implementation of PAL-Nepal. At present the cost implications are unclear, yet are studied. The PAL-Nepal guideline does not present key review criteria for monitoring and/or audit purposes. In the generic PAL guideline, however, some outcome indicators for monitoring of asthma and tuberculosis management are mentioned.  
37%

**Rigour of development**  
(Process used to gather and synthesise the evidence, the methods to formulate recommendations and to update them)  
The guideline is based on ‘expert consultation’ and literature research; it is unclear which criteria were applied for the selection of the underlying evidence. The design for the literature search is unclear. The process of decision-making after the expert consultation is not described. Recommendations are not explicitly linked to the evidence. Differences between recommendations in generic guideline and the PAL-Nepal guideline are not explained. Strong focus on health benefits. Side effects and risks are not made explicit. External reviewers were involved in the process of adapting the generic guideline into the country specific guideline. From the content, status and multitude of the different documents it is obvious that the guideline package is still under construction.  
29%

**Editorial independence**  
(Independence of the recommendations and acknowledgement of possible conflict of interest from the guideline development group)  
It is not stated whether the guideline is independent from the funding body. Conflicts of interest of guideline development members (both generic and PAL-Nepal) have not been recorded in any of the studied documents.  
6%

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1 Jefferson [28] validated the treatment recommendations of the generic PAL package on the basis of available evidence. This report was not available to the PAL-Nepal adaptation working group nor to the AGREE assessors and is therefore not included in their analysis.
agree’ or ‘disagree’ and ‘strongly disagree’. The multi-rater Kappa for clustered answering categories ‘agree’ and ‘disagree’ was 0.65, confirming our hypothesis.

**Implementation strategy**

Generic PAL training materials for a five-day training course were developed at international level (by the WHO) and adapted at national level (by the WHO and the National Tuberculosis Association). The National Tuberculosis Centre coordinated the adaptation and translation of all materials and would conduct the training. The implementation strategy in PAL-Nepal consisted mainly of training of primary care health workers using a mix of classroom teaching and interactive elements including clinical practice and exercises. Participants received training documents, a copy of the algorithms—including an A4-sized decision-support tool — as well as a large, poster-sized copy of the algorithms. A critical analysis of the intervention, using the format proposed by Hulscher [21], is presented in Box 3.

Table 2. shows the comparison of the planned implementation strategy with four characteristics of strategies that are likely to enhance effective implementation.

**The social system**

*Stakeholder analysis at national level*

The stakeholder analysis revealed that the initiators of PAL-Nepal, the National Tuberculosis Centre and the WHO, were the most supportive and powerful stakeholders in the pilot implementation in Nawalparasi. The complete list of potential stakeholders included 15 other international and national health care organisations and groups of professionals in the field of health care in Nepal. The respondents considered all these to be supportive of the implementation of PAL-Nepal but without having an interest or power to influence its implementation.

*Characteristics at district and local level*

Table 3. summarizes the potential barriers at district and local levels that were identified by the adaptation working group and by the WHO and PAL-Nepal partners as well as suggestions made to address these barriers.

**Discussion**

Our three-pronged assessment of the PAL-Nepal guideline enabled a systematic study of those factors that are seen as important for a successful implementation. In the pilot stage of programme development it identified potential complementary improvements in the guideline formulation, a broader and more multifactorial implementation strategy, and some obstacles at district and facility levels.
## Relevant Elements of the Intervention:

### Type of implementation strategy: Professional intervention
- Health workers at Primary Health Care Centres, Health Posts and Sub Health Posts are trained in using new clinical practice guidelines during a 5-day classroom course.
- Training materials can be used as decision tools (visual aids) in daily practice.
- Supervision visits by district health management are recommended.
- First year of intervention is combined with an effect evaluation study, involving daily –non-participatory presence of research assistants in health facilities.

1. **Flexibility** means the accepted variation (or standardisation) in delivering the intervention (site to site/time to time).
   - The intervention appears not to be flexible and therefore may not be able to address variation in needs of learners.

2. **Timing** includes the time interval between delivering the intervention and clinical decision-making (proximity) as well as the number and the duration of intervention events and time interval(s) between these events (frequency).
   - The intervention is a one-off 5-day course. Given turnovers in staff and uncertainty about how easy it will be for participants to take what they learn and apply it in their own settings without structural support this is likely to be an important limitation.

3. The content of the information consist of the message(s) (e.g. general or specific information on guidelines and/or performance, descriptive or graphical information), and its comparability (the possibility of comparing the received data on performance with those from others, or with standards).
   - Extensive written clinical practice guidelines for the management of respiratory diseases (TB, COPD, Asthma, Pneumonia) containing specific recommendations in approx. 50 decision flowcharts, algorithms and text boxes. The volume and complexity of the algorithms are likely to be a limitation for swift uptake by the target users.

4. The medium for delivering the message(s) can be for example oral, written, electronic, or a combination of these.
   - The course contains a mix of didactic and interactive training activities: Oral presentations to explain the guideline and interactive sessions on clinical practice and exercises. One practical session is planned in a secondary care hospital, demonstrating assessment and classification of symptoms in admitted patients.

5. The sender (deliverer) of the message has various characteristics, including his or her profession (also in relation to the clinical problem) and perceived authority (credibility, attractiveness, power).
   - The initiative for the intervention is taken by the World Health Organisation;
   - The responsibility for implementing PAL-NEPAL rests with the Ministry of Health, Department of Health Services and is delegated to the National Tuberculosis Centre;
   - The training of health workers is organised and facilitated by National Tuberculosis Centre;
   - Trainers are qualified doctors and chest physicians from regional hospitals and are likely to be perceived as respected opinion leaders.

6. The receiver of (or participant in) the intervention can equally be described by profession (also in relation to the clinical problem). The number (targeted and actual) of receivers and their motivation to participate (voluntary, compulsory, financial support) needs description. State also if the intervention was delivered to individuals or groups, including group size, and whether the receivers can learn from each other (social interaction).
   - Receivers are groups of primary care health workers with a mix of training levels: Health Assistants, Auxiliary Nurse Midwives and Auxiliary Health Workers, all employed by the Ministry of Health in Primary Health Care Centres, Health Posts or Sub Health Posts. The differences in qualifications were not reflected in the training.
   - Receivers attend compulsory and receive financial benefits (per diem) during the course. Selection of the first batch of trainees is determined by the random selection of a first group of intervention facilities for the purpose of the effect evaluation research study.
   - Receivers interact during the course in a question and answer format. It is unclear from the training documents to what extent extend the interaction opportunities are likely to facilitate learning.
The innovation

The AGREE instrument indicated areas to improve the present version of the guideline. Firstly, although the scope and purpose of the guideline was clearly stated, it could be more specific about demographic and geographic characteristics of the target population. Secondly, clarity and presentation were generally of high quality. In addition, the reviewers recommended an easily accessible, comprehensive guideline document that integrates the generic and Nepal-specific recommendations. Thirdly, in the area of stakeholder involvement, it was recognized that the target users — health workers at health posts and sub health posts — had not been involved in the development of the generic guideline, nor in the adaptation process for PAL-Nepal. Likewise, patient views had not been incorporated in the development process of the guideline. Their perspectives — for example, the views on the accessibility and use of facilities, or the cultural acceptability of treatments — could have identified potential barriers. Fourthly, the low scores for applicability can partly be explained by the fact that the cost implications and organizational barriers have yet to be evaluated for the pilot implementation of PAL-Nepal. That does not explain, however, why PAL-Nepal lacked recommendations for monitoring and auditing. Some indicators for monitoring and audit of tuberculosis and

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**Table 2.** Comparison of strategies that enhance effective implementation and PAL-Nepal’s implementation strategy

<table>
<thead>
<tr>
<th>Multifaceted strategies seem more effective than single strategies [6;22].</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>PAL-Nepal:</strong></td>
</tr>
<tr>
<td>- Core strategy: 5-day training course</td>
</tr>
<tr>
<td>- Decision support tools are provided</td>
</tr>
<tr>
<td>- Supervision is recommended, implementation not yet planned</td>
</tr>
</tbody>
</table>

| **Multi-event strategies over a longer time period seem more effective than single event strategies [6].** |
| **PAL-Nepal:** |
| - Single, one-off training course |
| - Supervision schedule yet unknown |
| - Visual aids distributed once might have continuous effect |

| **Active strategies like workshops and in-practice training are more effective than passive strategies like classroom teaching [6].** |
| **PAL-Nepal:** |
| - Combination of classroom teaching and interactive training techniques involving clinical practice and exercises. |
| - One practical session (patient demonstration) in secondary hospital |

| **Analysis of barriers and facilitators, strengthening of facilitators and selecting effective measures for crucial barriers [23;29].** |
| **PAL-Nepal:** |
| - Potential barriers related to drug treatment were discussed and documented in adaptation phase by working group [17;18]. |
| - Occasional discussion of human resource barriers (personal observation) |

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asthma management were mentioned in the background document, but not for chronic obstructive pulmonary disease and pneumonia. Including such recommendations in the PAL-Nepal guideline is helpful for health workers, supervisors and mid-level managers. Fifthly, the score for rigour of development will increase when the link between the evidence base and the recommendations is clearly shown. The generic PAL guideline referred to different national guidelines and international scientific papers. Most of these documents were made available to the Adaptation Working Group for PAL-Nepal. It is not mentioned to which rec-

Table 3. Barriers in social system at district and local level and the suggestions made of how to address them

<table>
<thead>
<tr>
<th>Barriers</th>
<th>Suggestions that were made to address the barriers</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Availability of drugs (identified by AWG)</strong></td>
<td>Drug supplies of listed essential drugs are supposed to last 6 months but part of the drugs usually ran out before that.</td>
</tr>
<tr>
<td></td>
<td>Some recommended drugs (e.g. salbutamol inhaler) are not listed in the Nepalese essential drug list. Consequently, these are not supplied by the government to the health facilities.</td>
</tr>
<tr>
<td></td>
<td>The essential drug list would be upgraded for the Nawalparasi situation [17;18]. Pending this matter the AWG was confident that the drugs would be available at local retail shops.</td>
</tr>
<tr>
<td><strong>Applicability of drug treatment (identified by AWG)</strong></td>
<td>Several specific recommendations in the generic PAL guideline were considered inapplicable in the Nepalese context. For example, the dosage or form of drugs was not always available and possibilities to apply drugs intravenously were absent.</td>
</tr>
<tr>
<td></td>
<td>It was stated that ‘there is definitely a resistance of health workers to use intramuscular drugs’ [18]. Explanations included the occurrence of complications and the fact the most health workers are not authorized to give intramuscular injections.</td>
</tr>
<tr>
<td></td>
<td>Drug treatments were altered according to applicability in the context of SHP and HP.</td>
</tr>
<tr>
<td><strong>Human Resources (identified by WHO, DHS and NTC)</strong></td>
<td>High staff turnover would be problematic, since there were no plans for training of newly appointed health workers.</td>
</tr>
<tr>
<td></td>
<td>During the pilot implementation staff transfers were minimized. No solutions were discussed for the situation beyond the first year.</td>
</tr>
<tr>
<td></td>
<td>Absenteeism. At SHP the AHW is the only health worker qualified to attend to adults with respiratory complaints, but is also frequently absent (e.g. for training or meetings). In their absence, the MCH attends to patients although she is formally unqualified.</td>
</tr>
<tr>
<td></td>
<td>Should MCHW be trained as well? No, the educational level of MCHWs was judged to be insufficient to attend the PAL training effectively. It was anticipated by WHO and partners that absenteeism of PAL-trained AHWs will be compensated by the dissemination of training materials and visual aids among non-trained staff in the health facilities.</td>
</tr>
</tbody>
</table>

Abbreviations: AWG=Adaptation Working Group, AHW=Auxiliary Health Worker, MCHW=Maternal and Child Health Worker, SHP=Sub Health Post, HP=Health Post, DHS=Department of Health Services, NTC=National Tuberculosis Centre
ommendations the evidence was linked and what the evidence was for the adaptations made in Nepal. The recommendations made in the generic guideline were validated a posteriori [28]. Unfortunately, the validation document was not available during the adaptation process in Nepal, or to the AGREE reviewers. There is substantial evidence in support of the generic PAL recommendations. Most evidence is from contexts in developed countries; hence, it is still informative to confirm this evidence in the context of developing countries. Evaluation of costs, organizational effects and health outcomes of PAL-Nepal — currently being carried out by an international consortium of researchers — is expected to reveal the health benefits and related cost of the PAL-Nepal guideline.

Finally, background documents and the PAL-Nepal guideline lacked a statement of editorial independence. It was not clearly stated whether the guideline developers might receive personal or institutional benefits from the recommendations.

The validity of the AGREE instrument in the Nepal context needs to be dealt with. The low inter-rater agreement might be related to characteristics of the AGREE instrument itself, PAL-Nepal or the Nepal context. AGREE is an appropriate instrument in the European context, where guidelines target health workers with a high level of education and address a limited area of clinical practice. In addition, algorithms can be used as an additional decision support tool that summarizes the main actions and recommendations. In PAL-Nepal a set of algorithms is the core element and replaces a textual guideline document. In the case of less skilled health workers and the absence of specific reference manuals on respiratory diseases, the logical choice of the PAL developers has been the introduction of algorithms based on a pragmatic symptomatic approach. The generic background document [16] is an extensive book containing a great deal of information that, according to one reviewer, ‘one expects in a medical textbook rather than in a guideline’. The size of this document is not only an important obstacle in the implementation of PAL-Nepal but it also might challenge the validity of AGREE.

The instrument does not assess the medical quality of the recommendations in the guideline, nor does it assess whether basic conditions are met for introducing the guideline, such as educational level of the health workers, drug availability and limited complexity of the algorithm. Also, the relative importance of the domains in AGREE might be unequal. For example, at sub health post level a notification that the authors were not paid by pharmaceutical companies (editorial independence domain) seems a lesser priority than, say, a visually well-depicted algorithm with clearly stated recommendations (clarity and presentation domain). To better understand the applicability of the AGREE instrument in the context of developing countries, more case studies are needed.

The implementation strategy

The discussion about effective implementations is ongoing and lively [24;29-31]. We used this discussion to assess the effectiveness of an implementation strategy prospectively in a developing country. Our findings give some support to the hypothesis that the PAL-Nepal implementation strategy needs upgrading to become effective. In our critical analysis of the
PAL-Nepal implementation strategy as well as its comparison with the international literature, we identified potential areas for improvement. Firstly, there is a body of evidence that training alone is not effective in changing clinical practice [32]. The PAL mono-event strategy therefore needs expansion. It is likely that a multi-facettted and multi-events strategy will be more effective, for example, organizing multiple opportunities to learn and practice working with the guideline. Recent studies, however, show that it might be more complicated than just adding more and different approaches [29-31]. Studies done in Nepal on effective strategies for improvement of primary health care will also provide useful suggestions [33;34].

Secondly, analysis of implementation barriers was only documented as part of the guideline adaptation process and more specifically the treatment recommendations in the generic guideline. The Adaptation Working Group members studied the applicability of these recommendations in the Nepalese context before including these in the PAL-Nepal guideline. They identified several barriers related to drug treatment and human resources, although these have not been addressed systematically. The adaptation process can benefit from using checklists for barriers and facilitators as used for example by Flottorp and Oxman [35]. These tools can also be integrated in the generic PAL package, like it has been in the adaptation of PAL in South Africa.

The social system: stakeholder analysis at national level
The assessment of stakeholders at national level has been informative in that it has shown the real status of the policy process: as long as PAL-Nepal is dealt with as a pilot implementation project of the WHO and the Ministry of Health — and by other potential future stakeholders — it will not mobilize opposition or support from others. At this stage, the receptiveness for PAL-Nepal was high. Respondents added that if PAL-Nepal is to be implemented nationwide, less tolerant stakeholders might be identified due to the financial implications. So far, the financial input has been relatively low and also has been covered by an international donor. The respondents commented that positive results from the effect evaluation were necessary to mobilize political will and more financial resources. These critical factors have not yet been addressed explicitly.

The social system at district and local levels
During the development of PAL-Nepal, several aspects of the social and organizational context at district and local levels were identified that potentially obstruct its effective implementation. Some were addressed effectively (inapplicable treatments), others were only temporarily solved (staff transfers) or not at all (drug availability, absenteeism of PAL-trained staff in SHPs). In the literature, other obstructing factors are reported. General factors affecting health service delivery in Nepal are poor human resources, difficult geography and poor general infrastructure [10;36-38]. Others address factors that more specifically influence the district and local services’ ability to take central guidance. Campbell et al. [39] mention a lack of human and financial resources and motivation. These two factors hamper the utilization
of clinical protocols and operational guidelines, although these tools are considered to be important and widespread in Nepal [39]. In addition, high absenteeism and communication gaps between villagers and rural health workers on the one hand and the higher health authorities on the other play an important role [40-42]. Aitken [43] presents an explanatory model for the functioning of district health services that shows ‘two value systems or theories with entirely different aims and expectations. (…) Officially, the organization’s value system emphasizes the services delivered: their quality and the number delivered.’ The implicit theory ‘is that the organization exists in order to distribute and account for funds and to provide the staff (…) with income. The duty of staff is therefore the provision of reports showing how these funds have been distributed and justifying their expenditure in terms of “showing progress” towards government targets. (…) The actual services provided are not seen to be very important. The staff are aware of both theories, often translating the demands of one into the language of the other’ [43]. It is crucial to understand these local circumstances in which changes are meant to take place.

Our observations and experiences confirm the comments made by Campbell, Justice and Aitken. We observed a multitude of separate activities in the district capital to support and improve the quality of health services, sponsored by national and international organisations, including the PAL-activities. These activities generated needed additional staff income, i.e. daily allowances and travel allowances. Also, it gave health workers a welcome change from work in remote stations and an opportunity to meet up with colleagues. This resulted in significant absenteeism of health workers from clinical work. Additional earnings through drug retail or private practice were the rule rather than the exception. Drugs that were not available at government facilities could often be purchased from the private businesses of health workers. Health workers spend their working hours combining private and public jobs, favouring the more profitable private jobs, while making sure that reports were presented on time. The centrally run staff appointment system also contributed to absenteeism, as health workers were appointed to unfamiliar localities. Health workers may spend a lot of time in administrative offices negotiating transfers to a more suitable location. Health workers and senior supervisors reported that supervision of peripheral staff in their own duty stations was rare. Supervising tasks had to compete with providing clinical health services at district level, facilitating training courses and workshops, and travelling to regional and national administrative offices. Although the current mechanism for introduction of PAL-Nepal seems appropriate in this stage of PAL development (i.e. through the additional financial incentives provided by a donor in a small pilot), we anticipate that in future, alternatives may be required linking implementation to adaptation of existing systems, for example through the integration of PAL-guidelines into existing training curricula.

We observed that despite these difficult circumstances, health staff were very positive and willing to contribute to the implementation of PAL-Nepal, for example, by facilitating meetings or participating in content discussions. PAL-Nepal is endorsed by the Department of Health Services and facilitated and promoted by the National Tuberculosis Centre, which established operational success through charismatic leadership, staff motivation and strong
communication lines [44]. This programme setting ensured attention and cooperation from the health workers.

**Conclusion**

Our ex-ante assessment of the adaptive local implementation of international WHO guidelines showed that the feasibility to implement PAL-Nepal could be improved, given the characteristics of the guideline package, the implementation strategy and the social system (in July 2002). The innovation has potential for improvement, the implementation strategy is limited and the social system has several cultural and manpower problems at district and local levels. Besides the technical and organizational challenges posed by the assessment of the innovation and the implementation strategy, we argue that the social and organizational reality of the district health services in Nepal need a more prominent role in the efforts to change clinical practice. Borrowing the approach — and the reputation as well — from the successful tuberculosis control programme might prove to be an important step. It can be explored further whether the organizational structure (including reporting, supervision and public relations) can be copied to the PAL-Nepal network of involved primary care health workers in rural Nepal.

We emphasize that both the context-specific PAL-Nepal and the global, generic PAL package are in the development stage. Our findings contribute to the maturation of the PAL programme, as do the findings from colleagues who evaluate PAL elsewhere in the world. This paper is one in a series of assessment reports supporting its further implementation. Developing good generic innovative interventions globally is just the first step in a long process towards successful implementation in local, specific contexts. Assessment of the feasibility of implementation can contribute to efficient allocation of scarce resources. The selected instruments allow for specific recommendations for further development of both the generic as well as the adapted guideline package in Nepal. For successfully transferring global knowledge into local practice, we need to support the development of additional sustained interventions that tackle other, culture- and health system-specific barriers as well. Health workers are key informants for these barriers.

**Acknowledgements**

We are grateful to the Nepalese respondents for providing detailed information for the stakeholders’ analysis at national level and the social and organizational context of health workers in Nepal. We thank P ten Have, J de Koning and P Rosier of The Dutch Institute for Healthcare Improvement CBO for assessment of PAL-Nepal guidelines with the AGREE instrument.
This assessment would not have been possible without the kind cooperation of the Department of Health Services, Ministry of Health of His Majesty’s Government of Nepal; the District Health Office and the health workers in Nawalparasi; the Department of Community Medicine and Family Health of Tribhuvan University, Kathmandu; and the National Tuberculosis Centre.

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We are grateful to the two anonymous reviewers for their valuable comments on earlier drafts of this article.

Reference List


Global knowledge in local practice


Chapter 7

How did you get here? Twenty-six journeys on the road to tuberculosis treatment in rural Nepal


Submitted for publication
Abstract

The fact that tuberculosis can effectively be treated with DOTS (Directly Observed Treatment, Short-course) is not enough to control the disease. Patients have to find their way to DOTS first. To better understand the route to DOTS in rural Nepal we interviewed twenty-six patients enrolled in DOTS. In semi-structured interviews DOTS patients shared their disease history and health seeking behaviour. Patient routes often started in the medical shop and led via intricate routes with multiple providers to facilities with higher qualified and more competent staff where tuberculosis was diagnosed. Analysis showed several factors that influenced the route to DOTS. Besides known patients factors (such as severity of complaints, the ability to pay for services, availability of services and peer support for choosing a provider) we also identified specific health services factors. These included the perceived quality, costs and service level of a provider, and not being adequately referred by one provider to another. Self referral because of waned trust in the provider was very common. In contrast, once tuberculosis was considered a possible diagnosis, referral to diagnostic testing and DOTS treatment was prompt. Patient routes are likely to become shorter if providers of care are better capable of recognising tuberculosis and if providers are willing to refer patients in case of unsatisfactorily response to treatment, and if service level in facilities is improved. Whereas international initiatives rightfully address the disease management skills of providers, additional strategies are needed too. These should address the referral between providers in different levels of health care and the quality of services in primary care facilities.
Background

Tuberculosis causes a high burden of disease world wide especially in low and middle income countries, despite the availability of efficacious treatment [1]. Tuberculosis treatment revolves around the DOTS strategy which has been proven to be effective [2]. An enduring challenge is how to ensure that tuberculosis patients find their way to DOTS. Lack of community awareness and patient knowledge about tuberculosis and its management have been mentioned by other researchers as obstructing factors [3;4]. Besides these patient factors, health services factors may be obstructing the road to tuberculosis treatment [5]. In this respect, insufficient human resources, lack of diagnostic facilities are some of the known barriers. As a consequence, patients may spend an unnecessary length of time before reaching DOTS, resulting in worse outcomes and an increased risk of disease transmission. Internationally, efforts have been made to improve the health services for patients with lung diseases, including tuberculosis. As patients ordinarily seek health care from multiple sources for different reasons, both in western as non-western societies [6-8] it is important to understand their routes through the system. Health care policymakers and program-designers need information about how patients use the health system and whether this confirms implicit assumptions about its functioning. With this in mind we assessed the patient’s narrative of their journey that eventually resulted in tuberculosis treatment. We aim to answer the following questions: “What is the route that these patients took through the health care system, from the moment of their first complaints until the start of DOTS treatment?”, “What were their reasons for taking this specific route?”.

Methods

Setting

We conducted the study in the rural lowland district of Nawalparasi, in the Terrai area of Nepal, bordering India. The Nepalese health care system faces multiple challenges such as lack of financial and human resources, difficult geographical and poor general infrastructure [9;10]. The health care system can be characterized as a national health system based on the principles of primary health care. In addition, there is a large private health care sector [11]. Nawalparasi has 601,000 inhabitants, for whom the following governmental health care facilities are available: 1 district hospital in its capitol Parasi, 5 primary health care centres, 8 health posts, 63 sub health posts, and a multitude of private health care facilities: hospitals, clinics, consulting medical officers, health workers and drug retail shops [12]. Much more and larger facilities can be found in the nearest big city, Butwal, approximately one hour by bus from Parasi. The sub-health posts are the smallest governmental health care facilities and provide basic health care. This includes mother and child health care, by a mother and child health worker, and general health care, by an auxiliary health worker who is also
health posts have more, and higher educated staff. Primary health care centres are yet another, higher, level of facilities. The one district hospital has one medical doctor who is responsible for clinical care and the management of the hospital and who is also the head of the district health care as a whole. The regional hospital is the referral hospital for several districts and offers outpatient and inpatient services by many specialists. The tuberculosis control program in Nawalparasi is part of the national tuberculosis program. This previously ‘vertical’ program has been integrated at service provision level over the past decades. In Nawalparasi the facilities for tuberculosis management include 4 DOTS centres for diagnosis and treatment as well as 10 DOTS sub-centres where sputum smears can be prepared and patients receive their medication after a diagnosis at a DOTS centre [13]. Patients presenting in primary care health facilities, who are suspect of having tuberculosis, need to be referred to a DOTS centre for re-assessment, diagnosis and treatment. In Nawalparasi DOTS sub-centres are located in all health posts [14].

**Study design, data collection, and study period**

In semi-structured interviews, respondents were invited to tell their story of how they first perceived their complaints, sought treatment, and how they eventually ended up in the DOTS treatment program. We asked respondents about their route through the health care system and the reasons for this route. Respondent’s characteristics were collected with a questionnaire. Interviewers (MB, AtA) used one interpreter to communicate with respondents in local languages (Nepali and Bojphuri). Interviews were recorded on tape and summaries of the interviews were written in English by the interpreter and reviewed by the interviewers. To assess the validity of the summaries we transcribed the recordings of five interviews. Independent translators translated these transcription from local languages into English. We compared these transcriptions to the written summaries. We found the summaries to correctly reflect the fully transcribed interviews with respect to the routes and reasons for these routes. Details about locations of providers and types of treatment were omitted in the summaries, as were courtesy introductions.

The quotes presented in the results section are phrased in third person, reflecting the translations by the interpreter. Text in [rectangular brackets] indicates additional information by the researchers about facilities or providers.

Data for this study were collected between November 2002 and April 2003. During the time of the interviews the exchange rate for Nepalese Rupees was 78 to 1 US Dollar, 81 to 1 Euro [15]. The estimated gross domestic product per capita (purchasing power parity basis) was $1400 [16].

**Measures**

A topic list was used to semi-structure the interviews. After brief introductions the respondent was asked to share the story of his/her illness: *What were the initial complaints* when he/she first fell ill? *Did he/she get any help or advice? From whom/where did he/she get help or advice? Why did he/she choose this person/provider? What happened next? These
questions were repeated until the story of how he/she got enrolled in DOTS was completed. The interview ended with registering respondents’ characteristics: age, gender, profession, position in household, marital status, town or village of residence.

Study population and recruitment of respondents
Eligible patients were all tuberculosis patients who collected their daily or weekly doses of tuberculosis treatment at one of five selected DOTS (sub-)centres in Nawalparasi. The centres were selected on geographical accessibility and facility level (DOTS sub-centres and DOTS centres). Each facility was visited on several occasions. There were 12 interview days. Patients were invited to participate in the study upon arrival at the DOTS facility. To avoid interference with and from health workers, the consenting patients were interviewed in a separate room, or outside the DOTS facility building but mostly on the facilities’ premises.

Ethical considerations
We conducted this study within the framework of the Practical Approach to Lung Health initiative, which was implemented and evaluated under supervision of the Nepal Health Research Council. Respondents gave informed consent. Anonymity was assured and we explained respondents that participation would not have effect on their treatment. None of the invited DOTS patients declined participation.

Analysis
The analysis of the summaries of the interviews identified the different providers that were visited by patients, and in which order, and the arguments stated for the patients’ specific choices. For the analysis of these routes we categorised the mentioned providers and facilities into three groups, according to the anticipated level of competence of the attending staff. The first group (A) consists of the medical shop, where drugs can be purchased and where no formal consultation is necessary. The second group (B) consists of private health workers (not known to be medical doctors), sub health posts, health posts and primary health care centres where trained health workers provide disease management. The last group (C) consists of medical doctors in private practices, and hospitals where medical doctors are available for patient care. In all groups we indicate the number of private and governmental providers and facilities. The general description of the routes also includes the providers that diagnosed tuberculosis or ordered the test that led to diagnosis of tuberculosis.

We grouped the reasons for their route in the following order: Firstly, what were the initial complaints and which provider was visited to address these complaints first. Secondly, if patients changed providers what were the grounds for this change and how did they chose a new provider. In our analysis we also looked at the importance of referrals as these could provide a clue whether the system is able to guide patients to higher levels of care when needed. This is particularly relevant for programs as the PAL initiative that aim to improve the adequacy of the referrals of suspected tuberculosis cases. Arguments for not going to a certain provider were also included in the analysis.
Results

We interviewed 26 tuberculosis patients, 12 women aged between 7 and 48 years (mean 29) and 14 men aged between 15 and 74 years of age (average 42). The 7 year old girl was accompanied by her father who responded to the questions by the interviewer. Respondents’ characteristics are shown in table 1.

Table 1  Respondents characteristics

<table>
<thead>
<tr>
<th>Age in years</th>
<th>Women (n=12)</th>
<th>Men (n=14)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Minimum – Maximum</td>
<td>7 – 48</td>
<td>15 – 74</td>
</tr>
<tr>
<td>Mean (SD)</td>
<td>28.7 (11.7)</td>
<td>41.6 (16.0)</td>
</tr>
<tr>
<td>Occupation (n)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Housewife (7)</td>
<td></td>
<td>Farmer (3)</td>
</tr>
<tr>
<td>Housewife and laborer (4)</td>
<td></td>
<td>Farmer and laborer (3)</td>
</tr>
<tr>
<td>Student (1)</td>
<td></td>
<td>Driver (2)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Laborer (1)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Carpenter (1)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Mechanic (1)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Retired from army (1)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Student (1)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Hotel employee (1)</td>
</tr>
</tbody>
</table>

Years of schooling per age group:

- Age 7-18
  - 2 years schooling: Women 1, Men 0
  - 7 years schooling: Women 0, Men 1
  - Missing: Women 1, Men 0

- Age >18
  - 0 years schooling: Women 6, Men 6
  - 4 years schooling: Women 0, Men 1
  - 5 years schooling: Women 1, Men 2
  - 8 years schooling: Women 1, Men 4
  - Missing: Women 2, Men 0

Number of providers approached before treatment start

<table>
<thead>
<tr>
<th>Number of providers</th>
<th>Women (n=12)</th>
<th>Men (n=14)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>2</td>
<td>3</td>
<td>4</td>
</tr>
<tr>
<td>3</td>
<td>1</td>
<td>5</td>
</tr>
<tr>
<td>4</td>
<td>3</td>
<td>1</td>
</tr>
<tr>
<td>5</td>
<td>-</td>
<td>3</td>
</tr>
<tr>
<td>6</td>
<td>1</td>
<td>2</td>
</tr>
<tr>
<td>7</td>
<td>2</td>
<td>-</td>
</tr>
<tr>
<td>8</td>
<td>1</td>
<td>-</td>
</tr>
</tbody>
</table>

Kind of provider first approached

<table>
<thead>
<tr>
<th>Kind of provider</th>
<th>Women (n=12)</th>
<th>Men (n=14)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Private providers</td>
<td>10</td>
<td>11</td>
</tr>
<tr>
<td>Public providers</td>
<td>2</td>
<td>3</td>
</tr>
</tbody>
</table>
**General description of routes**

The most common starting point for the patient’s route was the medical shop, not the governmental sub health post or health post. (Table 2) At medical shops drugs were purchased, with or without consultation from a health worker. As complaints persisted or even worsened, the routes of our DOTS patients led to health workers or medical doctors who held formal consultations. Drug retail was no longer the foremost important characteristic. Subsequently, facilities with a good reputation for competence and or diagnostic facilities were consulted at increased financial costs. Eventually the patients arrived higher up in the organizational hierarchy of the health care system.

When explaining their road to lung health, 21 patients reported that they first went to a private health facility, 5 consulted a governmental primary health care facility at the start of their pathway. After a minimum of one (1) and a maximum of eight (8) different providers in all possible combinations and orders (data not shown) the patients had reached a provider (11 of category B and 15 of category C) who diagnosed tuberculosis and were subsequently enrolled in DOTS. The last health care facility before enrolment was for 15 patients a government health care facility (i.e. hospital, primary health care facility, or Health Post), and for 11 patient the last stop was at a private health care facility. (Table 2)

<table>
<thead>
<tr>
<th>First providers visited after initial complaints (n=26)</th>
<th>Last providers visited before enrollment in DOTS (n=26)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>(A)</strong></td>
<td></td>
</tr>
<tr>
<td>(10) Medical Shop (p)</td>
<td>(0) Medical Shop (p)</td>
</tr>
<tr>
<td><strong>(B)</strong></td>
<td></td>
</tr>
<tr>
<td>(3) Health Post (g)</td>
<td>(2) Health Post (g)</td>
</tr>
<tr>
<td>(0) PHC centre (g)</td>
<td>(5) PHC centre (g)</td>
</tr>
<tr>
<td>(8) Priv. Practitioner (p)</td>
<td>(3) Priv. Practitioner (p)</td>
</tr>
<tr>
<td><strong>(C)</strong></td>
<td></td>
</tr>
<tr>
<td>(2) Hospital (g)</td>
<td>(8) Hospital (g)</td>
</tr>
<tr>
<td>(2) Medical doctor (p)</td>
<td>(3) Medical doctor (p)</td>
</tr>
<tr>
<td>(1) Hospital (p)</td>
<td>(5) Hospital (p)</td>
</tr>
</tbody>
</table>

(A) = medical shop, drugs can be purchased without prescription and formal consultation is necessary (B) = private health workers (not known to be qualified medical doctors), sub health posts, health posts and primary health care centers with trained health workers (C) = qualified medical doctors in private practices, and hospitals where medical doctors are available for patient care  
(p) = private health care facility (g) = governmental health care facility

Overall, patients visited facilities with higher qualified staff at the end of their routes. The routes differed considerably both in length as in providers visited, with an occasional U-turn to a lower level of care:

“you are suffering from fever for a long time, so it has changed to typhoid”, the doctor said and gave medication for typhoid. She took this medicine from the district hospital for one week. Someone from her neighborhood had told her about video X-ray, she
knew that she could go to the regional hospital for this, but she had not enough money. Because she still thought it might be malaria she went to a health post to get a malaria test. At this health post the malaria test was performed and malaria medication was given, but they also advised her to do a sputum test”. (Interview summary 5).

Once a provider **suspected tuberculosis**, sputum tests were ordered.

“After a week of taking medication she went back to the government health post. This time she did tell about her husband’s tuberculosis. The health worker examined her, she was given plastic containers and asked to bring sputum to health post the next day. Slides were prepared and these confirmed that she had tuberculosis.” (Interview summary 25).

After the **diagnosis of tuberculosis** a referral to the DOTS programme followed:

“The first time he went to this health worker he told him he had loss of appetite. The Private health worker listened to his lungs and prescribed syrups and pills costing Rs 900. The second time the same thing happened. The third time he was advised to get a sputum test, he did not turn in enough sputum and tuberculosis was suspected, but not confirmed. The private health worker again prescribed medication. The fourth time his sputum was successfully tested and tuberculosis confirmed and he was prescribed medication and referred to the DOTS centre at the district hospital by this private health worker.” (Interview summary 6).

Providers also informed diagnosed patients **that tuberculosis treatment was available for free**:

“The medical shopkeepers told him that he could buy it from them, but that he could also get this tuberculosis medication for free at the district hospital” (Interview summary 9).

“It was confirmed that he had tuberculosis and the doctor told him so. He informed him that he should take medicine for 6 month and that he could buy medicine at medicine shops, or get it for free at a government health facility” (Interview summary 2).

Although an exception was also reported:

“She went for an X-ray at a private shop as advised by someone in her community who said that she could know exactly what was wrong. The X-ray cost her Rs 320. The person who did the X-ray told her that she had tuberculosis, but no other advise or treatment was given” (Interview summary 23).

**Reasons for the followed route**

**Initial complaints, care seeking actions and first providers visited**

Respondents’ stories started with a variety of complaints, such as fever, cough, headache, blood in sputum, abdominal pain, and loose stools. Most respondents mentioned the complaints to their family members or to others who were close to them. Also the health care
options were discussed with them. The first health care seeking actions, following the onset of the initial complaints, were related to the **perceived seriousness of illness** and **anticipated level of competence** needed from a provider:

When respondents thought that the complaints were due to a not-severe and not chronic illness they were hopeful that they could manage on their own and bought medication at a medical shop.

“He first thought he had the common cold and stayed at home for 2 to 3 days. After 2 to 3 days his nephew bought one tablet for the fever from a medical shop that he took.” (Interview summary 17).

“Because she thought she was suffering from a simple illness, she did not think it necessary to go to a health post, which in her opinion is for more severe diseases. She first went to a medical shop and got medication for her fever and her headaches” (Interview summary 3).

“At first the gland in the neck was very small …… I did not know what this could be and bought pain medication from medical shops.” (Interview transcript 7).

One respondent said that her **peers recognized the complaints** and she sought help from a **specific provider known to be competent** in treating such complaints:

“Someone from her neighbourhood, who had dropped in on the family to watch television, told her mother that her daughter might have tuberculosis because she had been coughing for a long time, and advised to take her to Semari [the nearest Primary Health Care Centre]. He had taken his wife there when she had been coughing, and she had been diagnosed with tuberculosis.” (Interview summary 24).

Also **economic factors** influenced the choice of a provider:

“He did not consult any other medical practitioners in these four years because he did not have enough money. He had blood in his sputum during this time.”(Interview summary 1).

“They could not afford to send him to Parasi hospital until six or seven months later” (Interview summary 4).

“Someone from her neighbourhood had told her about video X-ray, she knew that she could go to Butwal for this, but she had not enough money. Because she still thought it might be malaria she went to the nearest health post to get a malaria test” (Interview summary 5).

The **reputation of a provider** was mentioned by respondents. Either a reputation known by the patient before the complaints started, or a reputation that the patient came to know while discussing the complaints with others.
“he started a fever, with headache and whole body ache. During the first ten to twelve days of his illness he stayed at home without any medication. After this he went to a certain private clinic because his friends suggested he (should) go there and he had heard about this place before.” (Interview summary 8).

The perceived quality and service level at sub health posts, seem to be an obstacle for patients to use these facilities. None of the respondents had visited the sub-health posts during their route to the DOTS program. Patients reported aspects of these facilities such as drug availability, competence of staff, waiting times, and minimal opening hours, to prevent them from going there.

“You have to queue at sub health posts and health posts”, “and you have to pay five rupees to make a ticket and you can not buy on credit”, “he had to buy medication anyway, he could just as well go to a private health worker immediately”, and “these sub-health posts do not have all the medicines”, are arguments that made respondents reluctant to go there. (Interview summaries 7, 15, 9).

Social dependence was mentioned by yet another respondent. He could not go to the provider of his preference because

“At the time there was nobody willing to accompany him there” (Interview summary I).

Changing providers, referral.

All respondents reported to have consulted more than one provider. The main reason was that after the initial treatment the complaints persisted or returned after initial relief. The trust in the provider had waned.

“The first and second time he was prescribed medicine for simple cough and fever. He took these for seven days both times. It gave him some relief for some days but it did not cure his cough and after the second time he became angry and said to the medical doctor: if you can cure me then do, if not please tell me so. (…) The third time he was told it was typhoid and was given ayurvedic medicine. This also did not help. The fourth time the same doctor prescribed two medicines for typhoid. He took one that cost Rs 45 per day, it made his muscles shrink and his condition worse. After this the doctor wanted to check his urine and stool but he decided to go to a private nursing home in Butwal” (Interview summary 2).

To consult a different provider was mostly an initiative from the patient or his/her peers, not from the current provider.

“He did not get better and his family members and people from his neighbourhood advised him to go to …” (Interview summary 17).

The actual choice for another provider was more complex when the complaints became more serious and needed a more competent provider, that often lived further away.
“It seemed in the beginning that her problem of diarrhoea was controlled when she took medicines. Afterwards, when she got seriously ill, then everybody suggested to take her to Butwal.” and “there – in the medical shop – was also a doctor who was a quack. They treated but nothing improved. When the baby got weaker, than I hastened to Butwal” (Interview transcript nr 9. father answering for the 7 year old patient).

“. when blood started to appear in his sputum he became afraid (…) and someone in his neighborhood suggested that he was not getting better and advised him to go to …” (Interview summary 1).

Such consultations would therefore cost more money, both for consultation and travelling:

“She first contacted a private health worker at Tadi [small village] near her home. He told her she had typhoid and prescribed medication. While taking this medication she felt better for 8 days but after it was the same as before. Sometimes the medicine would be undigested, it would appear in her faeces. After this health worker she went to another private health worker who also diagnosed typhoid and gave medicine twice. Many people advised her to go to a good doctor in Parasi or in Butwal or to go to Harnata [location of a famous doctor in India]. The transportation by train and accommodation costs to India were Rs 400. Because she had no money she did not follow up on this advice.” (Interview summary 5).

One respondent mentioned that it was actually the provider that advised her to go to a different provider who had higher qualifications:

“… blood in her sputum for four times came. Initially she took medicine from the medical shop near her home. However, she did not get better and her condition became more severe. Then the medical shopkeeper told her that “I cannot control it” and advised her to go to AMDA [a certain private not-for-profit clinic] in Butwal” (Interview summary 26).

Discussion

Our analysis of twenty-six interviews with diagnosed tuberculosis patients under DOTS treatment in rural Nepal shows a route that led via multiple providers, in private and public health care facilities, to tuberculosis treatment. The reasons for seeking health care and for their choice of a specific provider were a heterogeneous mix of arguments. These arguments included both patient specific and provider specific arguments. The former included perceived seriousness of the disease by the patient or his peers, the ability to pay for services, and peer support for choosing and visiting a provider. These findings confirm findings of other studies in different contexts in developing as well as developed countries [17-20]. Provider specific aspects were also explicitly mentioned by the respondents. These included
the perceived quality, costs and service of a provider and being adequately referred by one provider to another.

A potential problem in our analysis was misclassification of the provider and health care facilities. Whether or not a shop keeper was also a health worker was often unknown to the patient. Likewise, a private health worker was sometimes known to be a health worker or a medical doctor but not always. Although much attention was given to verify the level of providers this might have led to misclassification. As both health workers and medical doctors are mentioned in all phases of the journey we believe that mis-classification has not led to distortion of our overall findings. Misclassification of health care facilities is much less likely as this could be verified on the basis of the reported location.

One of the limitations of our study is of course that we only interviewed patients that actually found their way to the DOTS programme. We have no information therefore about the extent to which tuberculosis patients do not find their way into the programme. Our findings would benefit from a comparison with data from patients that were actually followed during their journey towards treatment.

Self-referral because of lack of treatment results and waned trust was very important in the patients’ routes. Patients reported that the decision to change provider was usually taken by the patient themselves, supported by suggestions of their peers. Referral by providers was not based on the provider’s perception of his own competence, the symptoms presented by the patient, or the lack of treatment results. In contrast, however, provider initiated referral after a tuberculosis diagnosis seemed to be unproblematic. Once a provider had thought of tuberculosis, and sputum tests had confirmed the presence of \textit{M. tuberculosis}, referral to the tuberculosis programme seemed a logical and prompt next step. Providers did inform the patient about the fact that tuberculosis drugs are free and that treatment is available near home. Referral to the DOTS programme seems to be perceived by the provider as a successful and acceptable end of the consultation and diagnostic process.

Probably the lack of medical knowledge is one of the reasons for what seems to be a trial and error case-management. There might be other interpretations too. Firstly, managing a case in spite of inadequate knowledge may indicate that it is difficult for providers to acknowledge that someone else might be more competent to deal with the health problem presented. Secondly, once a provider refers a patient he/she is no longer the provider’s customer. This is on short term a direct and negative economical consequence of an in potential professionally sound decision. This economical argument (for non-referral) seems obvious for health workers in private facilities but it could also be applicable to health workers in public facilities. Health professionals in the public facilities commonly also have a private practice from where they sell drugs that are not available at the government facility or where they conduct follow-up consultations outside clinic hours [21]. Their public activities could be seen as an entry point for clientele into their private enterprises. As a consequence, patients might undergo inappropriate treatment and waste valuable time and money with an incompetent provider. These negative effects became more evident as the symptoms worsened and the choice for yet another provider became more complex.
Initiatives that aim to strengthen the capacity of primary care facilities, such as WHO’s Practical Approach to Lung Health (PAL) [21-23] can benefit from these findings. If, for example, sub health posts are expected to become a major tool in passive case-finding of tuberculosis then the perceived low service quality of sub health posts needs to be addressed. To improve health care in general—and passive case finding for tuberculosis in particular—the issue of referral needs explicit attention. Socio-economical factors as mentioned above seem to be important for not referring the patient. To improve referral in general, a reward for adequate referrals may be considered. As financial benefits might turn out to be perverse incentives such measures need careful consideration. Another suggestion – albeit a challenging one - could be to build social networks of lung health care providers (public and private) at different levels of care who share resources and knowledge, allowing for referral of patients to known colleagues. As members of the same social network a referral might not be considered as failure but as a joint effort for the better of the patients’ health.

Conclusion

Tuberculosis patients in rural Nepal have followed intricate routes before reaching DOTS. Self-referral, because of lack of treatment results and waned trust, was the most common reason for changing providers. This not only indicates that providers often fail to treat the patient satisfactorily, but also do not guide the patient on his/her route to better treatment. To address these health system factors we need to develop complementary strategies that strengthen the service performance of health facilities and improve the collaboration between providers at different levels of care. This can contribute to strengthening passive case-finding initiatives and eventually smoothen the road to tuberculosis treatment.

Acknowledgements

We are indebted to the respondents, the staff of the regional health office, and the staff of the tuberculosis treatment centres for their kind cooperation. We thank J Schuster, JF Wendte, NS Klazinga and C Gunneberg for valuable comments on earlier drafts of this paper.

Reference List


A rational multi-criteria approach to priority setting: Should a lung health programme be implemented in Nepal?

R. Baltussen, A.H.A. ten Asbroek, X. Koolman, N. Shrestha, P. Bhattarai, L.W. Niessen

Submitted for publication
Abstract

Objectives
To identify and weigh the various criteria for priority setting, and to assess whether a recently evaluated lung health programme in Nepal should be considered a priority in that country.

Methods
Through a discrete choice experiment with 66 respondents in Nepal, the relative importance of several criteria for priority setting was determined. Subsequently, a set of interventions, including the lung health programme, was rank ordered on the basis of their overall performance on those criteria.

Results
Priority interventions are those that target severe diseases, many beneficiaries, and people of middle-age, have large individual health benefits, lead to poverty reduction, and are very cost-effective. Certain interventions in TB control rank highest. The lung health programme ranks 13th out of 34 interventions.

Conclusion
This explorative analysis suggests that the lung health programme is among the priorities in Nepal when taking into account a range of relevant criteria for priority setting. The multi-criteria approach can be an important step forward to rational priority setting in developing countries.
Introduction

With the advent of many new initiatives to increase funding for health care in developing countries, such as Global Fund on HIV/AIDS, TB and Malaria [1], the need for rational priority setting at country level is becoming more and more apparent. Countries have always been asking whether the interventions they are doing are the best use of resources, but now also need to plan strategies if the initiatives to raise additional funds for health are successful.

However, priority setting has often been implicit, based on decisions made in the past, or resulting from ‘unintended outcomes of the various pressures on providers, government agencies, purchasers and patients’ [2-4]. It is argued that only by using an explicit approach to priority setting that resources can be directed to maximize the achievement of societal objectives in relation to health [4]. The use of cost-effectiveness analysis has often been proposed as such an explicit approach, but many other criteria for priority setting have also been put forward, including medical (such as burden of disease) and non-medical criteria (such as age of target population) [5-8]. Recently, the development of a multi-criteria approach to priority setting – or more generally labelled multi-criteria decision analysis (MCDA) - has been identified as one of the important issues in health system research [9].

In stark contrast with the near-absence of applications of MCDA to allocation decisions in health, is the widespread acceptance and routine use of MCDA in other disciplines, e.g. to structure remedial decisions at contaminated sites in environmental sciences [10]. MCDA has also been applied in agricultural [11], energy [12], and marketing [13] sciences. In those disciplines, MCDA has evolved as a response to the observed inability of people to effectively analyze multiple streams of dissimilar information. The analysis establishes preferences between intervention options by reference to an explicit set of criteria that the decision making body has identified [14].

Following the experience of MCDA in those disciplines, we aim to develop a similar approach to prioritize intervention options in health. It has been argued that successful MCDA in health should i) involve quantitative rather than qualitative analysis since the priority setting process involves many criteria and many interventions and intuitive processing of this data can lead to unjustified conclusions; ii) cover a comprehensive set of criteria relevant for decision-making; iii) consider criteria simultaneously to allow trade-offs between various criteria; iv) establish the relative importance of criteria in a way that allows a rank ordering of a comprehensive set of interventions; and v) be strongly embedded in the organizational context [8]. This paper explores the potential of discrete choice experiments (DCE) in this process. DCE provide one way of analysing and communicating preferences for different interventions to policy makers [15;16].
In an explorative analysis, the approach is used to support current policy making in Nepal on the implementation of the Practical Approach to Lung health (PAL) program. The program, initiated by the World Health Organization, introduces a set of guidelines on integrated case management of pneumonia, TB, COPD, and asthma [17-20] and is intended to promote better lung care for school aged children, youths and adults at first level health facilities. In a pilot implementation in Nepal, PAL was found to be more costly and more effective than current treatment guidelines for lung disease in Nepal, and thereby relatively cost-effective [21]. Topic of discussion in Nepal is now whether PAL should be scaled up to other districts and eventually to the whole country. The nation-wide implementation of the intervention will require significant resources [22], and thereby obviously competes with other interventions in e.g. HIV/AIDS, maternal and child health for scarce health care resources. These and related issues were discussed in a workshop on the future plans of scaling up PAL in Nepal, and the present study was conducted alongside this workshop. It thus provides a unique opportunity to compare a rationale approach to decision making to those discussions that are actually taking place.

Table 1. Definition of attributes and levels in DCE

<table>
<thead>
<tr>
<th>Attribute</th>
<th>Level (regression variables)</th>
<th>Definition</th>
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<tbody>
<tr>
<td>Severity of disease</td>
<td>Not severe (NotSev)</td>
<td>Remaining healthy life expectancy more than two years in absence of intervention, when acquiring / having disease</td>
</tr>
<tr>
<td></td>
<td>Severe (Sev)</td>
<td>Otherwise</td>
</tr>
<tr>
<td>Number of potential beneficiaries</td>
<td>Few (FewBen) a</td>
<td>Less than 100,000 (those who could potentially benefit from intervention)</td>
</tr>
<tr>
<td></td>
<td>Many (ManyBen)</td>
<td>Otherwise</td>
</tr>
<tr>
<td>Age of target group</td>
<td>Young (YoungAge) a</td>
<td>0-14 years</td>
</tr>
<tr>
<td></td>
<td>Middle-age (MidAge)</td>
<td>15-59 years</td>
</tr>
<tr>
<td></td>
<td>Elderly (OldAge)</td>
<td>60 years and older</td>
</tr>
<tr>
<td>Individual health benefits</td>
<td>Small (SmallBen) a</td>
<td>Less than five healthy life years, on average for whole target group</td>
</tr>
<tr>
<td></td>
<td>Large (LargeBen)</td>
<td>Otherwise</td>
</tr>
<tr>
<td>Poverty reduction</td>
<td>Neutral (NeutPov) a</td>
<td>Disease is not more prevalent among poor</td>
</tr>
<tr>
<td></td>
<td>Positive (PosPov)</td>
<td>Disease is more prevalent among poor</td>
</tr>
<tr>
<td>Cost-effectiveness</td>
<td>Not Cost-effective (NotCE) a</td>
<td>Cost per DALY &gt; 3* GDP/capita*</td>
</tr>
<tr>
<td></td>
<td>Moderately cost-effective (ModCE)</td>
<td>Cost per DALY between 1 and 3* GDP/capita</td>
</tr>
<tr>
<td></td>
<td>Cost-effective (VeryCE)</td>
<td>Cost per DALY &lt; 1* GDP/capita*</td>
</tr>
</tbody>
</table>

a=reference
Methods

Discrete choice experiment
In a DCE, respondents choose their preferred option from sets of hypothetical options, each consisting of a bundle of attributes or criteria that describe the option in question, with each criterion varying over a range of levels. The criteria are constant in each scenario, but the levels that describe each criterion may vary across options. Analysis of the options chosen by respondents in each set reveals the extent to which each criterion is important to the decision at hand [15;16]. Running a DCE involves a number of steps, and these are discussed in turn.

Definition of criteria and levels
We organized two group discussions to identify the relevant criteria and related levels to be included in the DCE, which included a total of seven policy makers and people otherwise involved in regional health care programmes. A wide range of criteria were mentioned, and summarized in a number of categories. Some criteria put forward in these discussions were related to common aspects of all interventions, such as the need to improve access to health

<table>
<thead>
<tr>
<th>Attribute</th>
<th>Level (regression variables)</th>
<th>Definition</th>
<th>Comments</th>
</tr>
</thead>
<tbody>
<tr>
<td>Severity of disease</td>
<td>Not severe (NotSev)</td>
<td>Remaining healthy life expectancy more than two years</td>
<td>Societies may want to give preference to severely ill patients on the basis of their greater need for health care, and the diminishing marginal utility of health: an improvement in health from a severe health condition is then valued more highly by individuals than the same size improvement in health for a less severe condition [29].</td>
</tr>
<tr>
<td></td>
<td>Severe (Sev)</td>
<td>Otherwise</td>
<td></td>
</tr>
<tr>
<td>Number of potential</td>
<td>Few (FewBen)</td>
<td>Less than 100,000</td>
<td>Societies may favour interventions that target many people because these interventions may have a larger impact for society at large</td>
</tr>
<tr>
<td></td>
<td>Many (ManyBen)</td>
<td>Otherwise</td>
<td></td>
</tr>
<tr>
<td>Age of target group</td>
<td>Young (YoungAge)</td>
<td>0-14 years</td>
<td>Societies may have age-preferences because of ethical considerations (&quot;disadvantage the old because they have had their fair innings&quot;) or economic considerations (&quot;advantage the adults because they are more productive&quot;) [30].</td>
</tr>
<tr>
<td></td>
<td>Middle-age (MidAge)</td>
<td>15-59 years</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Elderly (OldAge)</td>
<td>60 years and older</td>
<td></td>
</tr>
<tr>
<td>Individual health benefits</td>
<td>Small (SmallBen)</td>
<td>Less than five healthy life years, on average for whole target group</td>
<td>Societies may wish to favour interventions with a large health impact on a few individuals compared to those with a small health impact on many individuals since the former has a greater capacity to reduce health inequalities [31].</td>
</tr>
<tr>
<td></td>
<td>Large (LargeBen)</td>
<td>Otherwise</td>
<td></td>
</tr>
<tr>
<td>Poverty reduction</td>
<td>Neutral (NeutPov)</td>
<td>Disease is not more prevalent among poor</td>
<td>Societies may want to give preferential treatment to disadvantaged populations because they are in some moral sense more deserving of health resources than others [32]. In general, it is argued that the poor have a greater need for support than less poor sections of the community, due to their lower income and typically lower 'stock' of health [33], and that investments in the health of the poor could lead to poverty reduction [34].</td>
</tr>
<tr>
<td></td>
<td>Positive (PosPov)</td>
<td>Disease is more prevalent among poor</td>
<td></td>
</tr>
<tr>
<td>Cost-effectiveness</td>
<td>Not Cost-effective (NotCE)</td>
<td>Cost per DALY &gt; 3* GDP/capita*</td>
<td>Societies may wish to prioritise on the basis of the cost-effectiveness criteria, as this would generate the largest health gains at population level for the available budget. Classification of cost-effectiveness results is according to WHO-CHOICE methodology [35].</td>
</tr>
<tr>
<td></td>
<td>Moderately cost-effective (ModCE)</td>
<td>Cost per DALY between 1 and 3* GDP/capita</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Cost-effective (VeryCE)</td>
<td>Cost per DALY &lt; 1* GDP/capita</td>
<td></td>
</tr>
</tbody>
</table>
In care. In as far these criteria related equally to all interventions, they were not retained in the subsequent research. Furthermore, economic impact of a disease was discussed as a priority criterion and was later summarized into including a specific age category for the income generating population (considered as those between 15 and 59 years). The group discussion resulted in identification of six criteria with associated levels. These are shown in Table 1 (together with their coding for the regression analysis).

### Experimental design, and data collection

On the basis of four criteria measured at two levels, and two criteria at three levels, 144 unique scenarios can be defined for inclusion in a full factorial experimental design in DCE [15;16]. However, to avoid informational overload, it is often suggested to use a fractional factorial design including a limited number of scenarios [15]. Our fractional factorial design included a subset of 18 scenarios (representing an orthogonal array), to allow for estimation of all main effects [23]. Each of these 18 scenarios was paired to its mirror image so as to retrieve the maximum information from each choice. An example of a pair of scenarios is given in Figure 1. The DCE survey was administered during three sessions with policy makers in health, and health professionals involved in mid-level health care management and public health provision. All respondents had at least two years professional experience in the health sector. The sessions were organised around a workshop on the future plans of scaling up PAL in Nepal. Respondents were familiarized with the conceptual framework, and worked through a number of examples before they embarked on the DCE exercise. In total 66 respondents (43 men and 23 women, mean age 38.6 years) made choices between 18 sets of scenarios.

### Data analysis

All levels for all criteria were qualitative and were dummy coded. Dummy coding involves that a criterion with L qualitative levels is transformed into L-1 dummy variables in which each dummy is set equal to 1 when the qualitative level is present and set equal to 0 if it is not.
Binary logistic regression models were used to analyse the response data, with the following equation being estimated:

\[
\text{Logit}(P) = \ln\left(\frac{P}{1-P}\right) = \beta_0 + \beta_1\text{Sev} + \beta_2\text{ManBen} + \beta_3\text{Midage} + \beta_4\text{Oldage} + \beta_5\text{LargBen} + \beta_6\text{PosPov} + \beta_7\text{ModCE} + \beta_8\text{VeryCE} + \beta_9\text{Scen2} + \beta_{10}\text{Scen3} + (\ldots) + \beta_{26}\text{Scen18} + \varepsilon
\]

(Equation 1)

where \( P \) is the probability of an intervention being chosen by the respondents, \( \beta_0 \) the intercept term, \( \beta_i \) (\( i=0-26 \)) the parameters of the model to be estimated, \( \varepsilon \) the unobservable error term and all other variables are as defined in table 1. To control for differences in attractiveness of scenarios we added dummies for scenarios to the equation. We assume a main-effects additive probability model, which derives a linear combination of the weights of each level of all criteria. It is standard practice in a DCE to assume such a linear function. It has been observed that, given the inclusion of dummy variables, this model does not impose an interval scale or ordinality on the relationship between the criteria and predicted probability scores [16;24].

The results are presented as regression coefficients, average marginal effects and relative contributions. Regression coefficients indicate the sign of the effect of a variable on the probability of selection of an intervention, but have no direct quantitative interpretation here. Average marginal effects can be quantitatively interpreted and reflect the change in probability of selection of an intervention following a change in a single variable. The average marginal effects are computed by taking the difference in estimated probability of \( P \) with and without the variable, while holding the distribution of the other variables at their sample value, and then taking the sample mean of these differences. The relative contributions indicate the contribution of one criterion to the variation in preferences explained by the model (Efron’s \( R^2 \)). These contributions are calculated by computing Efron’s \( R^2 \) with the model that excludes the criterion of interest while holding the coefficients of the other criteria constant. This procedure allows us to evaluate the contribution of criteria irrespective of the number of levels they have.

**Composite league table**

Next, we considered a large set of 33 interventions as comparators for the PAL program. These interventions are related to child and maternal health, tuberculosis, HIV-AIDS, and address an important part of the burden of disease in Nepal. They cover the disease areas as put forward in the health related Millennium Development Goals as adopted by the UN including Nepal [25], and we therefore regard them as relative priorities in health in Nepal. The cost-effectiveness of interventions was based on work by the WHO-CHOICE project [26]. Information on poverty reduction was retrieved from the World Health Report 2002 ‘Reducing risks, promoting healthy life’ [27], whereas information on severity of disease, number of potential beneficiaries, and individual health benefits were based on consultation of disease models employed in WHO-CHOICE. In addition, the information on PAL was
Table 2. Composite league table for Nepal, taking into account multiple criteria for priority setting

<table>
<thead>
<tr>
<th>Interventions</th>
<th>Dummy variables and qualitative levels</th>
<th>Probability of selection</th>
<th>Rank</th>
</tr>
</thead>
<tbody>
<tr>
<td>TB: Standardized second-line drug re-treatment of TB</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>TB: Treatment of smear-negative TB cases under DOTS</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>TB: Treatment of new smear-positive TB cases only under DOTS</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Child health: Oral rehydration therapy for diarrhoea</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Child health: Case management of pneumonia</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>HIV/AIDS: Antiretroviral therapy: no intensive monitoring, first-line drugs only</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>HIV/AIDS: Antiretroviral therapy: intensive monitoring, first- and second-line drugs</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>HIV/AIDS: Peer education and treatment of sexually transmitted infections for sex workers</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>HIV/AIDS: Treatment of sexually transmitted infections</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>HIV/AIDS: Screening &amp; treatment of syphilis</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>HIV/AIDS: Voluntary counseling and testing</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>HIV/AIDS: Mass media campaign to promote safer sex</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Practical Approach to Lung health (PAL)**

Maternal health: Referral care for severe post-partum haemorrhage
Maternal health: Neonatal resuscitation / Treatment of severe pre-eclampsia/eclampsia
Maternal health: Management of maternal sepsis
Maternal health: Management of obstructed labour, breech and fetal distress
Maternal health: Antibiotics for pre-term premature rupture of membranes
HIV/AIDS: Prevention of mother-to-child transmission
Child health: Zinc supplementation
HIV/AIDS: School-based education
HIV/AIDS: Antiretroviral drugs, intensive monitoring, first- and second-line drugs
Maternal health: Tetanus toxoid
Maternal health: Community-based case management for neonatal pneumonia
Maternal health: Facility-based care of very low birth weight babies, severe neonatal infections, severe neonatal asphyxia and neonatal jaundice
Child health: Measles vaccination
Maternal health: Normal delivery by skilled attendant. Active management of third stage & initial treatment of post-partum haemorrhage
Maternal health: Screening & treatment of pregnancy induced hypertension. Screening & treatment of asymptomatic bacteruria
Child health: Zinc fortification of staple food
Child health: Vitamin A supplementation
Child health: Vitamin A fortification of staple food
Maternal health: Community-based support for low birth weight babies
Child health: Improved complementary feeding, growth monitoring and promotion
Maternal health: Antenatal steroids for pre-term births

* '0' denotes the absence and '1' the presence of a level

Based on the results from an economic evaluation [21] (Shrestha et al. 2005), and associated population-level disease model (KC et al. 2005), whereas information on poverty reduction related to PAL was retrieved from the World Health Report 2002. Table 2 shows the list of interventions and whether a qualitative level of a variable is present (where ‘0’ denotes the
Table 2. Composite league table for Nepal, taking into account multiple criteria for priority setting

| Maternal health: Facility-based care of very low birth weight babies, severe neonatal infections, maternal health: Normal delivery by skilled attendant. Active management of third stage & |
| HIV/AIDS: Peer education and treatment of sexually transmitted infections for sex workers |
| Maternal health: Screening & treatment of pregnancy induced hypertension. Screening & |
| HIV/AIDS: Antiretroviral therapy: intensive monitoring, first- and second-line drugs |
| Maternal health: Neonatal resucitation / Treatment of severe pre-eclampsia/eclampsia |
| Child health: Improved complementary feeding, growth monitoring and promotion |
| HIV/AIDS: Antiretroviral drugs, intensive monitoring, first- and second-line drugs |
| Maternal health: Community-based case management for neonatal pneumonia |
| HIV/AIDS: Antiretroviral therapy: no intensive monitoring, first-line drugs only |
| Maternal health: Management of obstructed labour, breech and fetal distress |
| HIV/AIDS: Antiretroviral therapy: intensive monitoring, first- and second-line drugs |
| Maternal health: Antibiotics for pre-term premature rupture of membranes |
| Child health: Vitamin A supplementation |
| HIV/AIDS: Prevention of mother-to-child transmission |
| Pracical Approach to Lung health (PAL) |
| Child health: Measles vaccination |
| Maternal health: Tetanus toxoid |
| HIV/AIDS: Treatment of sexually transmitted infections |
| HIV/AIDS: Mass media campaign to promote safer sex |
| TB: Treatment of new smear-positive TB cases only under DOTS |
| HIV/AIDS: School-based education |
| Child health: Vitamin A fortification of staple food |
| Child health: Zinc fortification of staple food |
| HIV/AIDS: Screening & treatment of syphilis |
| HIV/AIDS: Voluntary counseling and testing |
| TB: Standardized second-line drug re-treatment of TB |
| Child health: Zinc supplementation |
| Child health: Oral rehydration therapy for diarrhoea |
| Child health: Case management of pneumonia |
| Child health: Initial treatment of post-partum haemorrhage |
| ManyBen | MidAge | OldAge | LargeBen | PosPov | ModCE | VeryCE |
| 1 | 1 | 1 | 0 | 1 | 1 | 0 | 1 | 0.91 | 1 |
| 1 | 1 | 1 | 0 | 1 | 1 | 0 | 1 | 0.91 | 1 |
| 1 | 1 | 1 | 0 | 1 | 1 | 0 | 1 | 0.91 | 1 |
| 1 | 1 | 0 | 0 | 1 | 1 | 0 | 1 | 0.84 | 4 |
| 1 | 1 | 0 | 0 | 1 | 1 | 0 | 1 | 0.84 | 4 |
| 1 | 0 | 1 | 0 | 1 | 1 | 0 | 1 | 0.80 | 6 |
| 1 | 0 | 1 | 0 | 1 | 1 | 0 | 1 | 0.80 | 6 |
| 1 | 0 | 1 | 0 | 1 | 1 | 0 | 1 | 0.80 | 6 |
| 1 | 1 | 1 | 0 | 0 | 1 | 0 | 1 | 0.77 | 11 |
| 1 | 1 | 1 | 0 | 0 | 1 | 0 | 1 | 0.77 | 11 |

A rational multi-criteria approach to priority setting

<table>
<thead>
<tr>
<th>Dummy variables and qualitative levels*</th>
<th>Probability of selection</th>
<th>Rank</th>
</tr>
</thead>
<tbody>
<tr>
<td>Sev</td>
<td>ManyBen</td>
<td>MidAge</td>
</tr>
<tr>
<td>1</td>
<td>0</td>
<td>1</td>
</tr>
<tr>
<td>0</td>
<td>0</td>
<td>1</td>
</tr>
<tr>
<td>1</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>1</td>
<td>0</td>
<td>0</td>
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<tr>
<td>0</td>
<td>1</td>
<td>0</td>
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<tr>
<td>0</td>
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<td>1</td>
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<td>0</td>
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<tr>
<td>1</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>1</td>
<td>0</td>
<td>0</td>
</tr>
</tbody>
</table>

absence and ‘1’ the presence of a level). Next, the ‘probability of selection’ was estimated for each intervention using equation (1). Subsequently, all interventions were rank ordered on the basis of this ‘probability of selection’, on the assumption it relates in a positive way to the attractiveness of that intervention. The results can then be interpreted as a composite league
table, with the most attractive interventions on top and the least attractive interventions at the bottom.

## Results

The results of the discrete choice experiments are shown in Table 3. All coefficients were significant and their signs had the expected direction. The marginal effects show e.g. that interventions that target severe diseases have a 19.7% higher probability of being selected than interventions that target non-severe diseases, other things being equal. Also, interventions that target people of the mid-age group have a 12.7% higher probability of being selected than interventions that target the young, but those that target the elderly have a 30.5% lower probability of being selected. Overall, interventions that target severe diseases, many beneficiaries, people of the mid-age group, have large individual health benefits, lead to poverty reduction, and are very cost-effective have a higher probability of being selected than interventions without (one of) those characteristics.

The relative contributions show that age of target group is the most important criterion, followed by individual health benefits, severity of disease, cost-effectiveness and number of potential beneficiaries. The least important criterion is poverty reduction. The model explained 31% of all observed variance in preference.

### Table 3. Results from binary logistic model

<table>
<thead>
<tr>
<th>Criteria</th>
<th>Coefficient</th>
<th>P-value</th>
<th>Marginal effect</th>
<th>Contribution R²</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Severity of disease</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Severe disease (Sev)</td>
<td>1.08</td>
<td>&lt;0.001</td>
<td>0.197</td>
<td>0.038</td>
</tr>
<tr>
<td><strong>Number of potential beneficiaries</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Many beneficiaries (ManBen)</td>
<td>0.97</td>
<td>&lt;0.001</td>
<td>0.176</td>
<td>0.034</td>
</tr>
<tr>
<td><strong>Age of target group</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Middle-age (MidAge)</td>
<td>0.71</td>
<td>&lt;0.001</td>
<td>0.129</td>
<td></td>
</tr>
<tr>
<td>Elderly (OldAge)</td>
<td>-1.65</td>
<td>&lt;0.001</td>
<td>-0.305</td>
<td>0.166</td>
</tr>
<tr>
<td><strong>Individual health benefits</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Large individual health benefits (LargeBen)</td>
<td>1.15</td>
<td>&lt;0.001</td>
<td>0.214</td>
<td>0.048</td>
</tr>
<tr>
<td><strong>Poverty reduction</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Positive poverty reduction (PosPov)</td>
<td>0.46</td>
<td>&lt;0.001</td>
<td>0.08</td>
<td>0.007</td>
</tr>
<tr>
<td><strong>Cost-effectiveness</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Moderately cost-effective (ModCE)</td>
<td>0.93</td>
<td>&lt;0.001</td>
<td>0.156</td>
<td>0.034</td>
</tr>
<tr>
<td>Very cost-effective (VeryCE)</td>
<td>1.14</td>
<td>&lt;0.001</td>
<td>0.194</td>
<td></td>
</tr>
<tr>
<td>Intercept</td>
<td>-3.166</td>
<td>&lt;0.001</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

R² (Efron) = 0.307
The composite league table shows that interventions with the highest probability of selection are in TB control, followed by oral rehydration therapy for diarrhoea and case management of pneumonia in child health, and several interventions in HIV/AIDS control including the provision of antiretroviral therapy (Table 2). PAL ranks 13th. On the basis of cost-effectiveness information alone, the rank ordering would be less differentiated, and PAL would be ranked 27th only (not in Table).

**Discussion**

This paper has shown the feasibility of simultaneously accounting for efficiency, equity and societal concerns in prioritization decisions and its potentially large impact on priority setting. For example, whilst PAL would be given low priority under pure efficiency considerations, it is ranked much higher if the policy maker would also be concerned about severity of disease, number of potential beneficiaries, age, individual health benefits, and poverty reduction. Second, by showing the relative importance of the different criteria, policy makers can clearly see the implications of tradeoffs between different concerns on prioritization decisions.

The evaluation of PAL in the period 2002-2004 was lead by the National Tuberculosis Centre (NTC), and was seen as one if its potential priority programs to combat the increase of tuberculosis [20]. An important operational issue in the implementation is the budget impact of PAL incurred by its training costs of PAL [22]. Overall, PAL leads to significant cost savings but these fall on the patient and not on the budget of the government. If no additional resources would become available, it is not sure whether PAL will be scaled up to the country as a whole. This situation seems to contradict with our findings that indicate PAL as a priority programme.

One methodological explanation for this apparent contradiction is the absence of high-level decision-makers in the working groups, to identify criteria for priority setting. As a consequence, the views of those who take the final programme decisions were not included to the optimal extent, and lead, for example, to the omission of criteria like budget-impact in the analysis. PAL might not have been a priority program in our findings as budget-impact would have been included as a criterion. Another – merely political – explanation could be the rigidity of the resource (re)allocation process [28]. If PAL would indeed be a priority in Nepal, vested interests and influence of political groups may make the reallocation of resources from unattractive programs to more attractive, priority, programs, such as PAL, difficult. This would reiterate the importance of rational priority setting processes as proposed in this paper.
The composite league table should be considered as illustrative only. First, the interventions that were included as comparators for the PAL program do not reflect the variety and nature of all interventions that are currently implemented in Nepal. For example, many of the child interventions are presented here in isolation, but are carried out in Nepal in the Integrated Management of Childhood Illnesses (IMCI) program. Second, not all information on criteria and levels was available for all interventions to construct the composite league table. This also applies to the PAL program: the economic evaluation only included the short-term gains in quality of life and not in long-term mortality reduction [22], and this may have underestimated its health effects and cost-effectiveness, and consequently its ranking in the composite league table. On the other hand, if we would have classified lung health diseases as non-severe, PAL would be rank ordered 26th in the present list.

This paper has introduced the concept of a composite league table to guide priority setting in health. It thereby responds to the recent call for the development of MCDA to priority setting [9]. This can be an important step forward to a rational approach to priority setting in developing countries. The approach meets the conditions for successful MCDA in health as recently put forward by Baltussen and Niessen 2006 [8]: in a quantitative manner, a comprehensive set of criteria (as identified through group discussions) is considered simultaneously allowing trade-offs (in the DCE) and rank ordered (on the basis of their probability of selection). However, the priority setting process was not embedded in the organizational context (e.g. Ministry of Health in Nepal) and its results have not been discussed with a range of stakeholders as e.g. organized in an advisory panel. This has indeed limited the relevance of results for actual policy making, and follow-up research should aim to embed the approach in that context.

The overall methodological approach is generalizable to other settings. Its application to e.g. another country would require the identification of priority setting criteria as relevant to that country, including marginal effects to derive ‘probabilities of selection’ for the interventions, to arrive at a country-specific composite league table.

**Conclusion**

This explorative analysis suggests that the lung health programme is among the priorities in Nepal when taking into account a range of relevant criteria for priority setting. The multi-criteria approach can be an important step forward to rational priority setting in developing countries.
Acknowledgements
We thank the participants in the DCE for their kind cooperation. We are grateful to Dr K.B. Shrestha, Dr P. Malla en Dr C. Gunneberg of National Tuberculosis Centre of Nepal, and Prof R.P. Gartoulla of Nepal Institute of Health Sciences for facilitating the DCE presented in this study.

Reference List


Chapter 9

Health services research at work in Case II
The second case in this thesis concerned the implementation of respiratory care guidelines in Nepal. With the aim of improving the quality of care for children (over 5 years of age), youths and adults with respiratory symptoms, the World Health Organization (WHO) developed the Practical Approach to Lung Health (PAL)-initiative. In Nepal, we studied the cost-effectiveness of the development and implementation of PAL as a contribution to its wider, global development and to inform the national government about the initiative’s implementation costs and effects. The results can guide the decision-making process on implementing PAL nationwide. In addition, we developed three research questions that focused on the context and processes in which the PAL package is implemented in Nepal.

The answers to these questions were presented in the case study. First, in Chapter 6 we presented the implementability of PAL [1]. In Chapter 7 we described the context in which PAL is implemented: the health care system in rural Nepal as experienced by patients who were treated for tuberculosis [2]. In Chapter 8 we explored a method for priority-setting in health policy and applied to the situation in Nepal. We assessed how much of a priority the implementation of PAL would be if our method was the gold standard for priority-setting in Nepal [3]. The cost-effectiveness of PAL in Nepal, addressed elsewhere by our health services research team [4;5], showed that implementing PAL costs more for the government than the current practice, but that PAL care is cheaper for patients. Also, patients treated in facilities where PAL had been implemented gave higher quality-of-life scores than those treated in facilities where current practice is used.

The research group that evaluated PAL consisted of health economists, public health specialists, medical doctors, a health scientist and epidemiologists of different nationalities. We worked together with the designers of PAL at the WHO and with the implementers of PAL in Nepal, the National Tuberculosis Center (NTC). We formed a consortium of involved institutions, which was chaired by the Nepal Health Research Council. The director-general for health services of the Ministry of Health participated in this consortium. Furthermore, a steering committee was formed to supervise the implementation and evaluation of PAL. Representatives of academic institutions, professional organizations, the Nepalese Ministry of Health and health care providers participated in this committee. The evaluation research proposal was developed in close collaboration with the PAL designers and implementers at the WHO and the NTC. The research was commissioned by the Netherlands Organisation for Scientific Research’s Foundation for the Advancement of Tropical Research (WOTRO) as part of its subsidy programme ‘Knowledge Enriches’ [6].

Inclusion of additional research questions
The selected research questions were: 1) What is the implementability of PAL in Nepal? 2) What route did tuberculosis patients take through the health care system? and 3) What is the relative importance of PAL in Nepal compared to other programmes?

We used different strategies to be able to pursue these additional research questions. Answering the first question was a precondition negotiated with the principal investigator of the cost-effectiveness evaluation for the involvement of the author of this thesis. As project
manager stationed in Kathmandu, Nepal, the author was in a position to collect the data for the *implementability* study. As long as it did not interfere with the project management tasks and as long as it could be carried out on a ‘budget-neutral basis’ there were no objections to ‘piggybacking’ because it was potentially a win-win situation. The implementability study was presented to other stakeholders in Nepal as an integral part of the initial research proposal, not as a separate research activity. Additional financial support for this study was obtained from the Department of Social Medicine at the Amsterdam Medical Center (computer facilities and two months of research time) under the condition that one research paper would be produced on this topic.

The second study was conducted by a team of Nepalese research staff and a Dutch fourth-year medical student conducting his obligatory research internship. The student provided his own finances for this study [7].

The third study was the result of successfully combining a workshop held to present the outcomes of the PAL-Nepal evaluation study with a discussion on the future of PAL in Nepal. The initiative for the workshop and for exploring rational priority-setting was taken in response to questions from our counterparts in Nepal. The initiators were senior researchers involved in the cost-effectiveness evaluation of PAL and developing tools for rational priority-setting [8]. The workshop received financial support from the WHO and WOTRO.

**Interactions with policymakers and other actors**

In general, our experiences of the interactions with policymakers and other actors in achieving these additional research goals were very positive. Although the initial evaluation proposal strictly focused on the costs and effects of implementing PAL, our counterparts in Nepal and at the WHO certainly welcomed additional information. The assessment of the quality of the guideline and the implementation strategy was ‘desk research’ and interaction with other actors was limited to discussions with the designers of PAL at the WHO to define what was actually in the PAL package. Assessing the receptiveness of the social system at national level involved Nepalese health officials, who ranked stakeholders by their power to influence PAL’s uptake. These officials were apprehensive about the consequences of this exercise, even after the promise of anonymous participation. At international level, we had intense discussions with our colleagues at the WHO on the phrasing of the paper, which later developed into Chapter 6 of this thesis. Because we as health services researchers wanted to contribute to PAL’s development, we did not want to upset potential support for the PAL programme. Presenting our study results, which were critical about several aspects of the PAL package, was therefore complicated. The good cooperation between us and our colleagues at the WHO and the NTC was an important facilitator to jointly write a critical message about PAL that could be useful in PAL’s further development.

The assessment of patients’ routes in seeking health care was welcomed by senior staff at the NTC because it provided information about how the health care system functioned and could produce views and suggestions for improving passive case-finding of tuberculosis patients in Nepal. Cooperation was both positive and constructive, both from management
level in the tuberculosis programme as well as in the health care facilities where the interviews were conducted.

For assessing the relative importance of the PAL programme in Nepal, organizing a workshop was a logical approach. The two funding organizations (WOTRO and the WHO) supported the idea of having a workshop with two goals: presenting results of the evaluation and discussing the future of PAL. Also, the NTC had provided information about feedback of study results on several occasions. During the workshop the question of ‘What do we do with the results of the evaluation studies?’ was linked with the topic of prioritization in health care [9;10] The discrete choice experiment [3] that was conducted during the workshop was presented as an integrated part of the discussion on the future of PAL in Nepal. The intention is to discuss the results of this exercise with the participants and other stakeholders in the near future.

**Follow-up of events and developments**

We can conclude that we had good and constructive cooperation with other stakeholders, both in Nepal and in the WHO. This facilitated our contribution to the discussion in Nepal about respiratory care and the functioning of the primary health care system and we explored a method for priority-setting. In June 2005, during the workshop mentioned in Chapter 8, we were able to present some of the results of the evaluation study. Most of the audience were enthusiastic about this opportunity to discuss the results of the evaluations and the future of PAL in Nepal with staff members from the NTC and the Ministry of Health. For example, the results of the study on patients’ routes through the health care system raised the interest of the staff at the NTC to involve the private health care sector in the implementation of PAL [11]. Unfortunately, the WHO staff were not able to attend this meeting and an opportunity to share ideas with them was lost. At international level, we contributed to PAL research and review meetings together with representatives from Nepal [12;13]. As a result, supervision of the health workers trained in using PAL guidelines was included as a recommended implementation strategy.

PAL has gained momentum worldwide. By 2002, PAL had been introduced in four countries: Chile, Morocco, Nepal and South Africa. By the end of 2005, this number had increased to 16 countries [14] and more evaluation results have become available [15]. However, it is difficult to estimate the impact of our studies on the development of PAL in general and more specifically on the decision-making process regarding nationwide implementation in Nepal. Several developments in Nepal need to be mentioned in this regard. Firstly, after the fieldwork for the evaluation study was completed in September 2003, no PAL-related activities were undertaken, either in the district of Nawalparasi or at national level at the WHO offices or the NTC. The fact that the evaluation results were not available till June 2005 could well be linked to this situation. As the director of the NTC told us during the assessment of the social system at national level, a good start for mobilizing support for implementing PAL was to prove its cost-effectiveness [16]. Now that it has been shown that implementing PAL costs the government money, it is likely that further implementation will not be easy.
In that sense, our findings negatively influenced the implementation process in Nepal. The WHO had already indicated that funding the pilot implementation was a one-off. Funding the nationwide implementation was the responsibility of the government. No funds from the WHO would be available for this purpose. Other political developments influenced PAL as well. For example, during the period of the PAL evaluation, the NTC’s charismatic director had been a strong supporter of PAL. In 2004 this director was removed from office and the new director was reluctant to support PAL, for reasons unknown to us. Also, in addition to this changing political climate at the NTC, the general political and security situation in the country has deteriorated, both during and since the time of the PAL evaluation. Democracy has been seriously challenged by continued violent actions between army and Maoists and by the dismissal of the house of representatives by His Majesty the King of Nepal in 2002. After long and violent demonstrations, the house of representatives was reinstated in April 2005. Since then, the King has lost much of his ruling power. These unstable circumstances are not favourable for attracting potential donors who can support successful implementation of new initiatives such as PAL.

In conclusion, the studies presented in this case pointed out the room for improvement in the PAL package, the characteristics of the health care context in which PAL is implemented, and the level of priority that mid-level health care managers assign to PAL. These findings are useful for the further development of PAL as expressed by stakeholders during the workshop in Nepal. However, such developments seem to have come to a standstill. This could be because of the lack of funds to implement PAL nationwide, but could also be because of the negative influence of political developments. At international level, our studies contributed to improving the PAL package and informed the international community about PAL’s cost-effectiveness in a low-income country.

Reference List


An instrument for measuring national health system performance?
Chapter 10

Developing a national performance indicator framework for the Dutch health system

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Abstract

Objective:
To report on the first phase of the development of a national performance indicator framework for the Dutch health system.

Methods:
In January 2002, we initiated an informed interactive process with the intended users—policy makers at the Ministry of Health, Welfare and Sport—and academics to develop both the conceptual framework and its content. Decisions were based on consensus after discussing strategic goals of the health system, and information needs of policy makers at the Ministry of Health, Welfare and Sport, and after studying existing theory and international experiences with national performance indicator frameworks. We identified objectives and criteria for a framework at the national level, constructed a conceptual framework, and selected indicator areas.

Results:
As a starting point we chose a balanced scorecard reflecting four perspectives towards healthcare management information at the national level. These perspectives are consumer orientation, finances, delivery of high quality care, and the ability to learn and grow. We then linked the Lalonde model for population health to a balanced scorecard model. The constructed framework makes the relationship between population health and health system management apparent, and facilitates the presentation of performance information from various perspectives. The model reflects the strategic goals of the Dutch health system in contributing to the production of health by providing necessary healthcare of good quality that is accessible for all Dutch citizens while simultaneously informing policy makers about the performance of the entire health system in all sectors (care, cure, prevention and social services). The selected indicator areas for health system management information reflect the policy and management functions of the government and the defined public goals of the health system. The framework was formally adopted by the Ministry of Health, Welfare and Sport in February 2003, and since then individual indicator areas are being operationalized by 30 representatives of various departments at the Ministry with continuous external research support.

Conclusion:
The merit of linking the balanced scorecard inspired model to public health data is that it facilitates the visualization of the contribution of the health system to the improvement of population health. The method of an intensive interactive indicator development process between policy makers and researchers has so far proven successful.
Introduction

The political and scientific agenda for states to measure and improve the performance of their health systems is encouraged by international organizations [1,2]. As a member state of the Organization for Economic Co-operation and Development (O.E.C.D.), the European Union, and the World Health Organization (W.H.O.), the Netherlands participates in the initiatives of these organizations to measure and manage the performance of health systems.

On a national level, the changing accountability mechanisms, burgeoning budgets of national health systems, ageing populations, lapses in quality of care, safety issues, low consumer satisfaction levels, and attendant market mechanisms have contributed to the growing movement towards performance measurement, quality improvement and even re-engineering of healthcare delivery systems. All these developments contribute to the health system dynamics in the Netherlands (as illustrated in Table 1) and increase the demand for a national performance indicator framework. The Dutch government, in particular the Ministry of Health, Welfare and Sport (MoH), wants to use such a framework to monitor the health system performance at the national level and link it to the existing policy and accountability processes.

In this paper we report on the first phase of the development of the Dutch framework. In this first phase we distinguish two stages: (1) construction of the conceptual framework; and (2) selection of different indicator areas for the management information part of the framework. Both stages of the first phase were realized in the period January 2002 to March 2003. The second phase will include selecting indicator areas for the population health information part of the framework, identifying causal relationships between indicator areas, constructing and operationalizing indicators, and integrating the framework into the policy and accountability mechanisms of the MoH. The second phase is currently in progress.

Methods

In the first phase of the development of a national performance indicator framework we distinguish two stages. The core activity in the first stage was the construction of a conceptual framework, and in the second stage the selection of indicator areas. To conduct these activities we initiated an informed, intensive interactive process involving multiple stakeholders.

The information used in this process was retrieved by (1) detailed exploratory analysis of performance indicator frameworks from other countries and international organizations [3]; (2) focused analysis of the policy and management roles of the Dutch MoH in relation to national stakeholders including professional and institutional providers, financiers (insurers), healthcare consumers/patients, and regulators such as the inspectorate of health; (3) analysis of the existing information infrastructure for public health and healthcare. To analyze the envisioned governmental policy and management roles we studied publicly available policy documents [4,5]. We analyzed the existing infrastructure for public health and healthcare...
<table>
<thead>
<tr>
<th>Feature</th>
<th>Description of current situation</th>
<th>Government proposal</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Actors</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Patient/ consumer</td>
<td>Limited patient choice partly due to (1) shortage of manpower and healthcare facilities; and (2) lack of comparative consumer information.</td>
<td>Informed and empowered patient, choosing rationally between insurers and providers.</td>
</tr>
<tr>
<td>Provider institutions</td>
<td>Privately owned, not-for-profit with fixed budgets, non-competing. Government determines size of budget.</td>
<td>Privately owned, operating within a regulated market: competing with other providers.</td>
</tr>
<tr>
<td>Insurers</td>
<td>Public insurers are obliged to contract with every provider of in-patient care. Legally, there is freedom of contracting in ambulatory care. In reality, public insurers contract with every provider of ambulatory care too, partly as a result of shortage of manpower and healthcare facilities. Private insurers do not engage in contract because of the restitution system (see below), but act as ‘free riders’ with respect to the contracts that are negotiated by public insurers.</td>
<td>In the comprehensive single mandatory insurance scheme (see below) all insurers are obliged to act as strategic purchasers of care, entering in to contracts with those providers that offer the best deal in terms of quality and efficiency.</td>
</tr>
<tr>
<td>Government</td>
<td>Cost containment through provider budgets and manpower planning.  (Limited) performance management through its inspectorates. Detailed control over the healthcare system through budgeting, manpower planning, and regulation</td>
<td>Encourage market mechanisms to determine supply and demand, while securing public goals. To monitor performance via the use of indicators in a unifying framework More at distance overseeing the functioning of the healthcare system and its regulated market.</td>
</tr>
<tr>
<td><strong>System Financing</strong></td>
<td></td>
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<tr>
<td>Public</td>
<td>AWBZ: social insurance for long-term care and exceptional medical expenses; covers 100% of the population; operates under a system of direct payment; insurance carriers are the social insurance funds under the ZFW (see below). ZFW: social insurance for acute care and prescription drugs; covering mandatory for 62,9% of the population earning an income below a cut-off value of €31,750 in 2003; operates under a system of direct payment; insurance carriers are 25-30 sickness funds. Taxes (general taxation mainly for prevention activities).</td>
<td>AWBZ remains unchanged in the short run One single mandatory insurance scheme, offering a limited benefits package for the whole population regardless of income</td>
</tr>
<tr>
<td>Private</td>
<td>Private insurance: for acute care and prescription drugs, for people with an income above the cut-off value Out of pocket payments</td>
<td>Private insurance for services outside the limited benefits package</td>
</tr>
<tr>
<td><strong>Other Characteristics</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Governance of the system</td>
<td>Supply driven, with government regulating budgets, and numbers of providers and institutions.</td>
<td>Demand driven regulated market, where government is responsible only for securing public goals.</td>
</tr>
</tbody>
</table>
information using publications from the MoH, and from governmental health data institutes and research institutes [6]. In addition, we discussed the results from these analyses with key informants from the involved organization.

The structure for the interactive process was formed by a multidisciplinary academic research group (n = 6) and, at the MoH, a strategic co-ordination group (n = 2) and an intra-departmental project group (n = 30) chaired by a director general of the MoH. In addition, the research group was strengthened by members from the MoH and from the National Institute for Public Health and the Environment (RIVM; a governmental organization which collects, analyses and reports population health information for the Netherlands). The intensive interaction was facilitated in weekly meetings of the research team, biweekly meetings of the research team with representatives of the strategic co-ordination group, and by 10 monthly meetings between the research team, the strategic co-ordination team, and the intra-departmental project group.

The choices for the framework and the indicator areas—made in the meetings between the research team, the strategic co-ordination team, and the intra-departmental project group—were the result of decision making through a consensus approach after discussing strategic goals of the health system, information needs of policy makers at the MoH, and studying existing theory and international experiences with national performance indicator frameworks.

Results

Construction of the conceptual framework

The literature study revealed three main conditions for the Dutch national framework. Firstly, a framework which is to be chosen must be coherently balanced, covering various performance dimensions such as effectiveness, efficiency, and equity. Secondly, such a framework needs to be comprehensive; that is, it must involve information originating from all sectors in the healthcare system (namely prevention, cure, care, and social services). Thirdly, a suitable framework needs to link performance of health services to population health using public health data [7].

We selected a balanced scorecard as a starting point for the development of a performance framework [8], because it provides managers with a comprehensive, balanced, yet minimized amount of management information that combines strategies with policies. There were several reasons to select a balanced scorecard model. Firstly, a balanced scorecard would satisfy the information need of a government that has changed its main steering philosophy from a budget-driven mechanism to a regulated market mechanism. Secondly, a balanced scorecard is a dynamic model that accommodates changing strategies and thus optimizes flexibility in an altering political and economic context. Thirdly, this model has previously been adopted in several other healthcare performance frameworks, which enables us
to build on the experiences of others [9-11]. Fourthly, in the Netherlands, this model has also increasingly been applied in healthcare institutions and is therefore known to a large group of healthcare stakeholders. Fifthly, the model can be linked to population health information by using the consumer perspective as the interface between health management and population health information. Subsequently, we selected Lalonde’s determinants of health model to present the population health information [12,13]. The Lalonde model conceptualizes health determinants in four main quadrants, namely lifestyle, the environment (physical, work, social, political, and economic), the genetic or host’s constitution, and healthcare. This model has been used frequently in the presentation of public health and health determinants information in the Netherlands [6]. In the healthcare quadrant of the Lalonde model, the link with the consumer perspective in the balanced scorecard is found. The result of this construction is a performance framework which visualizes the relationship between health system management information and population health determinants (Figure 1).

Selection of indicator areas
In the second stage, we focused our activities on the selection of the indicator areas of the management information part of the constructed model. We discussed which core questions needed answering in each of the four perspectives in order to meet the information needs of the MoH.

Consumer perspective: What effect does the health system have on the (experienced) health of consumers?
Financial perspective: What are the financial consequences of the health system?
**Internal business processes perspective:** Are the preconditions for a regulated market mechanism met, and do these result in efficient healthcare delivery?

**Innovation perspective:** Does the health system have the ability to learn and grow? Does it learn and grow?

To answer these broadly formulated core questions, we identified sub-questions and corresponding indicator areas for each of the perspectives. The choice for indicator areas was guided by two criteria. Firstly, indicator areas need to be relevant for policy and management decisions in the specific Dutch context. Secondly, the complete set of indicator areas (Figure 2) must be applicable to the entire Dutch health system.

**Consumer perspective**

Through the consumer perspective, information is presented concerning the link between the health system and the health status of the population. We identified the following outcome-focused sub-questions:

- Are citizens prevented from getting ill?
- Do patients get better?
- Are disabled persons properly taken care of?
- Does the healthcare system help patients to attain good health?
- Are the risks to which patients are exposed acceptable?
Is the Dutch population in general, specifically its patients, satisfied with the functioning of the healthcare system?

These sub-questions are answered in three indicator areas:
- Effectiveness of healthcare (in all sectors, cure, care, social services, and prevention).
- Patient safety.
- Patient-centeredness.

Financial perspective

We need to answer the following sub-questions:
- What are the costs of healthcare?
- Are the available resources used optimally?
- Is the financial burden for consumers equally distributed (and who is paying)?
- Can all citizens carry this burden?
- Can financiers and providers guarantee continuity of care? Are they financially viable?

These questions are represented by such indicator areas as:
- Health system costs
- Allocative efficiency
- Vertical equity
- Financial accessibility
- Financial viability of financiers and care providers

Internal business processes perspective

Identified sub-questions include the following:
- How do the financiers, that is, private and social insurers (for acute care), care agencies (for exceptional medical expenses) and municipalities (for preventive care and population screening) perform?
- How do they cope with their responsibilities?
- Is the provided care of good quality?
- Do patients still have a choice of insurer and providers?
- Are there enough human resources to provide the necessary care currently required?
- Does the market mechanism enforce the creation of new professions or concentrations of care provision?
- What are the effects for the patient? Does the care delivery setting change?

The corresponding indicator areas are:
- Performance of care financiers
- Quality of the healthcare delivery process
- Availability of choice of insurers and providers
- Concentration of care provision
- Human resource management (1): (a) availability of staff; (b) vacancies; (c) staff satisfaction
- Substitution of care between professions and between care delivery settings

**Innovation perspective**
We identified sub-questions that focus on the conditions for developing innovative potential and actual innovations:

What financial means are made available for the learning and growth function of the healthcare system?
Are new technologies being promptly implemented?
Does the information infrastructure accommodate innovation?
Do employees work in conditions that are supportive for innovations?
Are professionals motivated to change old habits and procedures for new ones?
Does the healthcare system anticipate the need for new professionals for future healthcare delivery (especially in professions with long training pathways)?
Are organizational innovations such as new service arrangements developed and implemented that enable the system to respond to changing contexts, opportunities, needs, and demands?
Do the market mechanisms increase an influx in learning and growth potential through intensified co-operation between industry and health sectors?

Corresponding indicator areas are:
- Allocation of funds for learning and growth
- Diffusion of new technologies
- Information infrastructure
- Human resource management (2): (a) innovative working environment; (b) training of new staffs in professions with long training pathways
- Development and diffusion of organizational innovations
- Industry-initiated research and development activities in healthcare

For each of the four perspectives, discussion reports were produced in which boundaries and definitions were set for the perspective and guidance for the indicator areas.

**Discussion**
We have described the first phase of development of a performance indicator framework for the Dutch health system. We argue that the approach described in this paper presents an appropriate platform for ‘contextual’ development of a performance measurement and management framework, one that focuses flexibly on needs assessment, public goals,
users, owners, and regulators. This framework, like efforts elsewhere [14], is a step towards modernization, coherence, capacity building and engagement of all health actors. The first phase of the Dutch MoH project entailed the contextual, conceptual, content, and process development of the national indicator framework. The second phase, already underway, will specifically define, scientifically assess, and field test all chosen and potentially useful indicators to arrive at a critical operational core set.

We built the conceptual framework based on the notion that health improvement is the main objective of any health system. Subsequently, it is only sensible to design a performance framework around the consumer. In this era of consumerism and reawakening of public health values underscored by globalization, focusing on the consumer is a rational move towards qualifying and quantifying healthcare contributions to population health. In doing so, we capture clinical care as part of the broad-based public health efforts towards population health [15]. Population health is a function of healthcare, lifestyle, genetic makeup, and the environment, all being factors that are increasingly within the influence of the health system, thus necessitating a public health approach to performance measurement [16]. The Netherlands has rich sources of public health data, which can be used for research and policy issues. An effort is underway to link existing databases as much as possible and to create new cost-effective sources as the need arises. Regular population surveys will satisfy some of the data requirements.

We realize that the balanced scorecard has been developed as a tool for the strategic management of corporate businesses [8]. Although it has been applied in the non-profit and public sector, it has not been applied before at health system level, in a context where the hierarchical and steering responsibilities and relations can be quite different from a corporate business [9]. The Dutch health system is not a corporate business, nor can it be run like one. The MoH has kept responsibilities for the health system but has given financiers (e.g. insurers) and providers more responsibilities in organizing and managing the delivery of healthcare. We adapted the balanced scorecard approach to fit this specific setting. As a consequence, the content of the four perspectives has been tailored to the needs of the MoH. In addition, its application in the strategic steering mechanisms of the MoH is still underway. A first ‘dummy’ of the model is expected soon and will replace the existing annual budget plan and policy report of the MoH. Furthermore, the Council for Health and Social Service (RVZ) is preparing a report on how to embed the framework in the existing policy, budget, and accountability documents of the MoH. Also, the next Public Health Forecast report of the RIVM in 2006 will be set up around performance indicators, in relation to the framework [6].

Selecting the indicator areas as outlined in this paper should be seen in the light of the necessity to link the drivers of performance to the output and outcomes of the health system. The ambition is to acquire a better insight into the impact of health system determinants on the health of Dutch men, women, children, minorities, and other characterized populations.

The interactive development of the framework plays an important role in the broader information policy at the MoH. The framework interacts directly with two other policy tracks. The first track is creating transparency within the roles and responsibilities of the Dutch MoH in
relation to that of (public) health agencies. The second track is connected to an overall MoH inter-departmental operation, ‘From budget plan to policy accountability (VBTB)’, aimed at developing more result-oriented policy processes. These two tracks constitute two essential pre-conditions to develop and implement the performance framework. Both policy tracks will be implemented simultaneously with the framework. The intra-departmental project group at the MoH will be the central decision-making platform. The informed, intensive, and interactive collaborative process of building the framework serves as a practical example of how research originates from policy needs and reforms, and goes back to inform the policy-making process [17]. This developmental process represents one of the important ways of enshrining evidenced-based policy making and serves as a guide to the creation of a performance management system for public health. Here, we can see health services research as an important tool for public health, policy analysis, social research, and system redesign.

Conclusions

The linking of the balanced scorecard with the Lalonde health determinants model highlights the relationship between population health and health system management information. The selection of indicator areas has resulted in a set that is recognizable, relevant, and appropriate for policymakers. The ‘informed’ interactive process was able to bring scientific knowledge into the policy development discussions at the MoH. The ‘intensive’ interactive process has pushed the agenda for the effective development of the framework within the first year of the project. Finally, the ‘interaction’ with the MoH has resulted in the ownership of the framework by its users, policy makers at the MoH.

Acknowledgements

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The performance indicator framework of the Dutch health system: a progress report

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Abstract

This chapter discusses the development, implementation, and findings of a health system performance indicator framework in the Netherlands. This framework was principally designed to satisfy the Ministry of Health’s information needs in its far-reaching reform of the governance of the country’s health system. A ministerial advisory task force, line managers, a multi-disciplinary academic public health research group, and population health information institutes comprise the main actors in this ongoing process.

The Dutch framework consists of a balanced scorecard approach (for health care performance information) linked with the Lalonde model (for population health information). Twenty-six indicator areas exist for each of the balanced scorecard’s four perspectives (i.e., the system’s consumer orientation, financial outlook, care delivery quality, and ability to learn and grow). These indicator areas reflect the government’s monitoring and steering functions as well as the health system’s stated public goals.

Making these indicators operational entails applying the following measurement principles:
  - indicator areas must be relevant for policy and management decisions,
  - the complete set must be applicable to the entire Dutch health system, and
  - data used in the indicators must be available, actionable, reliable, and valid.

These indicators are primarily based on existing data collection systems and the National Institute for Public Health and the Environment supervises their implementation and coordinates data collection and evaluation.

Throughout the framework’s development process an integrated communication strategy has informed policy makers representing insurers, providers, and consumers. When the framework is ready to be fully implemented, the results will be presented annually to the government and parliament.

To date, the combination of interactive indicator development and a tailored communications strategy has proven successful and has strengthened ownership among all stakeholders.
Introduction

The growing international interest in measuring the performance of health systems has also been felt in the Netherlands. As a member of the European Union (EU) and the Organisation for Economic Co-operation and Development (OECD), the Netherlands has participated in several collective initiatives that address health system performance measurement [1-3]. At the end of 2001, the Dutch Ministry of Health, Welfare, and Sports (hereafter known as “the Ministry”) initiated the development of a national health system performance indicator framework. The framework aims to satisfy the Ministry’s information needs and support its redefined governance role. Unlike countries with a national health system, the Dutch government is limited in its ability to monitor and steer the system. In view of the recent redesign of the health system, the Ministry needed a new model for its information infrastructure (i.e., the health system performance indicator framework). The development and implementation of the framework challenges the stakeholders’ policy development skills in the complex managerial and organizational context of the Dutch health care system. This chapter describes the specific Dutch context, the chosen framework development strategy, the conceptual model of the framework and its content, the policy processes that influenced its development, and the planned dissemination strategy. We then evaluate the impact of its development and implementation and present the lessons learned thus far by the different stakeholders.

History and Context

Like many other Western health care systems, the Dutch system originated in the second half of the nineteenth century. Industrialization and the emergence of nation states created the conditions in which public health issues could be addressed collectively. Although the first regulations for trained medical professionals and hospitals date back to this period, the role of the national government with respect to health care was limited until the 1970s of the twentieth century when a growing tension was perceived between economic development and the rising costs of health care.

In the Netherlands, health care provision is organized mainly on a private, not-for-profit basis. The organization and management of the health care system is divided into four sectors: prevention, curative care, chronic care, and social services. Private enterprise is highly valued in Dutch society and “self-regulation” has always been a dominant management philosophy, used by the government and providers alike. Since 1865, a number of laws and the Health Care Inspectorate have been the tools used by the Dutch government to monitor and assess the performance of the health sector. Accountability has not been a standard entry in the providers’ vocabulary.
A common national insurance scheme for health care costs did not exist until 1941, when the Sickness Fund Act (in Dutch: “Ziekenfondswet”) was introduced during the German occupation. This law still forms the basis for the present social health insurance, in which everyone with an income below a certain threshold (including dependants) has compulsory insurance; the premium for this is fixed and collected through an income deduction. Those with incomes above the threshold can apply for private health insurance.

In 1968, social health insurance was expanded with the introduction of the Exceptional Medical Expenses Act (AWBZ). The AWBZ covers the cost of chronic care and nursing homes. Payment of AWBZ premiums are compulsory for everyone with an income and are fixed. Although the AWBZ was based on the changing care needs of an aging population, the economic recession in the 1970s and the need for cost containment were the main reasons the government became more involved in health care policy making. After a series of planning regulations that included health care manpower planning, reduction of bed capacity, and a budgetary regime, it was concluded that these policies were stifling the health care system. These regulations certainly did conflict with the philosophy of self-regulation.

Over the past 20 years, the government – led by a number of different political coalitions–has made efforts to redesign the health care system. The increase in health care costs and the limited effect of previous planning-based approaches has led to the idea that the health care system is best served by introducing a self-regulated market as the guiding principle for the interaction between all actors in health care. Several measures were taken to support this change. The non-profit sickness funds were privatized and for-profit health insurers were also allowed to execute the Sickness Fund Act. A reimbursement system for providers based on Diagnosis and Treatment Combinations (DTCs) was introduced to make competition between providers and insurers more transparent. Also, a new Health Insurance Act (in Dutch: Zorgverzekeringswet) will be introduced in 2006. Participation will be compulsory for all citizens, regardless of income. This Act will cover a basic package of acute and curative care. The contribution to be paid by the insured person will be partly nominal and partly income-related. Employers will contribute by making compulsory payments towards the income-related insurance contributions of their employees [4].

These measures show that although government rhetoric is that of being less directly involved in the health care market, it does want to set out the playing field boundaries for providers, insurers, and patients/consumers. “Steering from a distance” is the phrase used to indicate that although the government is taking on responsibilities for the health system, individual actors have their own roles to play as well. Furthermore, the provision-driven system has been reformed into a demand-driven system in which the government wants the health care policy to be guided by consumer preferences and care outcomes [5]. These changes in health system governance call for an information tool that supports the Ministry in its new governance role [6] (see for a detailed description of the Dutch health care system Exter et al. [7] and Van Ewijk et al. [8]). Characteristics of the Dutch population and some of the health system indicators in 2002 are presented in the table below along with data from Canada, New Zealand, United Kingdom and United States.
The health care system is complex, with multiple actors who have different goals and perspectives. These actors are often the main stakeholders involved in health care reforms such as designing, developing, and implementing a performance indicator framework. In the development process described here, the primary stakeholders were the Ministry, an academic research group, health care insurers, health care providers, and patients and consumers of health care.

The Ministry can be regarded as the principal stakeholder in the development process. Inspired by the aforementioned national developments as well as by international initiatives, the Ministry defined the need for a framework for health system performance indicators at national level. The Ministry sought research support from an academic research group for the development of such a framework. It initiated and funded the development of the performance indicator framework of which the Ministry will also be the primary user. As mentioned previously, the organization of Dutch health care is divided into care sectors. Both the financing and monitoring structure and the policy development structures are defined along the lines of the following care sectors: prevention, curative care, chronic care, and...
The level of involvement by government varies per sector. The division of the health care system into sectors is in part reflected in the organizational structure of the Ministry. Since this has an impact on whether interventions that address the system at large are incorporated, the subdivisions at the Ministry will be introduced briefly.

There are three subdivisions in the Ministry’s organizational chart: public health, health care, and social services. Public health is concerned with improving the population’s overall state of health, preventing diseases, and supporting the public health policies of municipalities and other tiers of government. Health care deals with institutional and extramural care, the health care insurance system, and pharmacy and medical technology. The social services subdivision handles care for the disabled and older persons as well as youth policy, nursing, and mental health care. These subdivisions are set out in the organizational chart (Figure 1.).

In addition to these subdivisions, a new “Strategic Unit for Information Policy in Health Care” (In Dutch: Strategische Eenheid Informatiebeleid Zorg, SEIZ) was formed to facilitate the

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1 The subdivision for social services is called “Maatschappelijke Zorg” in Dutch. In the Ministry’s English-language documents, the name of this subdivision is translated as “Social Support”. However, since “social services” is most commonly used in international literature, we will use social services throughout this chapter to indicate the “Maatschappelijke Zorg” subdivision.
development of information policy within the Ministry. This unit had the position of an executive office with advisory and coordinating tasks and responsibilities, and is not presented in the organizational chart. In November 2004, the SEIZ was integrated into a new policy directorate called the Market and Consumer Directorate (MC).

An academic research group was invited to support the Ministry in developing the framework. This group had published on performance indicators and the need to integrate public health thinking into health care policy making. They envisioned a system-wide approach to the framework, and realized this was a change from the sectoral approach currently dominant in Dutch health care policy making. The role chosen by the researchers was one of an advisory group, crafting the rough outline of the framework and suggesting the mechanism by which it would operate. As a result, the input from this group was significant at the start of the development and less apparent towards the end, when the blueprint was accepted and chosen as the basic structure for monitoring the performance of the Dutch health system. The group liaised with knowledge institutes to create an academic basis and sounding board for the conceptual framework. The National Institute for Public Health and the Environment (RIVM) and the Netherlands Institute for Health Services Research (NIVEL) were their main collaborative partners. During the development process, though, contacts were also established with all other Dutch national knowledge institutes such as the Netherlands Institute for Health Promotion and Disease Prevention (NIGZ), Prismant, Statistics Netherlands CBS, and the Dutch Institute for Healthcare Improvement (CBO).

The RIVM is a government organization that collects, analyses, and publishes reports on population health information about the Netherlands. It is also the secretariat for the European Community Health Indicators project, ECHI, [9] and coordinated the first pilot data collection for the Netherlands in the OECD health care quality indicators project. At the end of 2004, the RIVM was asked by the Ministry to take the lead in implementing the indicators of the performance framework.

The government has increasingly promoted the insurers’ coordinating role in health care. Just as in other European social health insurance systems (like those in Germany and Switzerland), managed competition has been introduced in the Netherlands [10]. For the Dutch social insurance system this implies that sickness funds (insurers) should attract clients by contracting efficient and good-quality health care services. From the government’s point of view, the insurers need to be transparent about their performance so consumers can choose between insurers and so the government can hold them accountable for that performance. Because of this, insurers have a keen interest in the development of the framework so they know which indicators will be used to measure their performance.

Care providers face yet another change in government influence in their primarily private, not-for-profit organizations. The current budget-based involvement will be complemented with indirect involvement by measuring performance using indicators that have yet to be defined. Their position as stakeholder can be defined as “reluctantly interested”. On the one hand, this is based on their compulsory participation, and on the other on the uncertainty of the subsequent increase in workload and the consequences of a potential average or poor
performance. Also, hospitals, ambulant care, and home care organizations as well as mental health care providers are all involved in their individual care sector’s own quality performance improvement initiatives. The development of the Ministry’s performance indicator framework is regarded by some providers as potentially distracting from and competitive with their own initiatives. Others see opportunities for strengthening the case for their own initiatives by aligning these with the ministerial framework development.

The development of the regulated market for health care has also put performance measurement on the public agenda. Patients/consumers have important roles to play in the redesigned health system. Once the regulated market has come into effect, patients/consumers have to be able to choose between different services and products, including insurance. Also, insurers need to be able to purchase care and choose between providers. Transparency regarding products and performance is a prerequisite. Information to guide the choices of both patients/consumers and insurers is essential if the regulated market is to work. Recent initiatives undertaken jointly by health care insurers, researchers, and patient/consumer organizations such as the Dutch Consumer Association (Consumentenbond) and the Federation of Patients and Consumer Organizations in the Netherlands (NPCF) — and observed with keen interest by the government — have started to develop consumer assessment surveys for the Dutch population. These surveys are based on CAHPS® [11], developed in the United States, and the Quote questionnaires [12], developed in the Netherlands. Results from these newly developed questionnaires are based on patients’ experiences with health care, and can inform both consumers and insurers [13;14]. This initiative is of great relevance for the Ministry of Health, because this information can potentially be aggregated to the macro-level where it will provide the Ministry with information about patients’/consumers’ experiences with health care [15].

Conceptual Model

In 2002, the research group was invited to head up the development of the framework. As a starting point, the concepts of other national and international frameworks for health system performance were studied [16]. The Dutch government finds itself in a complicated position: the mix of public and private partners, non-profit, not-for-profit and for-profit organizations, and the limited legislative tools for managing the health care system are all components of the complex environment in which the Ministry needs to gather comprehensive performance information that fits its new governance role of steering from a distance. In addition, the Dutch health system is struggling to merge market mechanisms with solidarity principles.

We identified the health of the Dutch population and the health care available to them as two major components of the health system. Based on the health care system’s goal of helping people attain the best level of health, we concluded that both population health and health care were the two perspectives that had to be part of monitoring the performance of
the entire health system. Also, we realized that if we wanted to monitor health and health care within one framework, the sector-based approach to data collection and reporting would be inappropriate. However, since the division into sectors in health care management and governance is deeply rooted in all levels of policy making, we realized that any attempt to monitor the system as an integrated whole (and not based on its separate sectors) would mean a significant change in the thinking of policy makers and other stakeholders. Our interactive approach was a direct result of our awareness that we were part of a major change process.

We first conceptualized the performance of the components of the health system, population health, and health care. The performance in terms of population health can be described by Lalonde’s model for determinants of health [17]. Lalonde described health as being determined by four types of factors: the environment, genetic constellation, life-style, and health care. An extended version of this model has for many years been the model on which the Dutch national Public Health Status and Forecasts reports have been based [18], thereby providing a framework Dutch policy makers are familiar with.

For the conceptualization of the performance of the health care system, we expanded the “care” determinant in Lalonde’s model into a “balanced scorecard” as presented by Kaplan and Norton [19]. To make steering from a distance possible, the Dutch Ministry needs a limited amount of information on the performance of the entire health care system. The balanced scorecard was developed for managing corporate businesses. In Kaplan and Norton’s design, the performance information is presented from four perspectives on the organization: the financial perspective, the consumer perspective, internal business processes perspective, and the innovation perspective. The first two are considered the “outcomes” of business strategy and the last two are the “drivers”. For corporate businesses, the outcomes in financial terms are at the core of the overall performance. This model has been successfully implemented in for-profit, not-for-profit, and non-profit organizations in both public and private contexts and at different levels of steering and monitoring. For application in our performance indicators framework for the Dutch health system, the consumer perspective is the focal point of overall performance in health care. Patient-centeredness is illustrated in the conceptual model by the change in focal point towards the consumer perspective, which subsequently makes this the interface between the Lalonde model and the balanced scorecard.

The conceptual model was further developed by exploring the indicator areas that would come under the four perspectives. Here, the input for the discussions was given by policy documents and strategy plans developed by the government. Consequently, we identified the indicator areas reflected by the Ministry’s policies and steering roles. Much of the debate here focused on whether the Ministry had to measure the performance of the health system at large, or whether it needed to measure the performance of those aspects of the system for which the Ministry was directly responsible. For example, there was debate about whether or not to include “waiting lists” in the framework. Strictly speaking, the health care providers and insurers are directly responsible for health care provision, not the government. However, some health care issues become political priorities, regardless of who is formally respon-
sible. Therefore, such issues (like waiting lists) are relevant for including in the framework. In the interactive approach, we identified all indicator areas. The collaboration between the research group and the stakeholders at the Ministry resulted in the conceptual framework (shown in Figure 2) presented in December 2002.

**Selection of indicator areas**

In the second stage, we focused our activities on selecting the indicator areas of the management information portion of the constructed model. We discussed which core questions needed answering in each of the four perspectives in order to meet the Ministry’s information needs.

*Consumer perspective*: What effect does the health system have on the health or experienced health of consumers?

*Financial perspective*: What are the financial consequences of the health system?

*Internal business processes perspective*: Are the preconditions for a regulated market mechanism met and does this result in efficient health care delivery?

*Innovation perspective*: Does the health system have the ability to learn and grow? Does it learn and grow?

To answer these broadly formulated core questions, we identified sub-questions and corresponding indicator areas for each of the perspectives. The choice of indicator areas was guided by two criteria. First, indicator areas needed to be relevant for policy and manage-

\[\text{Figure 2: Performance indicator framework for the Dutch health system}\]

\[\text{Lalonde model}\]

\[\begin{align*}
\text{Environmental factors} \\
\text{Genetic layout} \\
\text{Health} \\
\text{Health Care} \\
\text{Lifestyle}
\end{align*}\]

\[\begin{align*}
\text{Balanced scorecard} \\
\text{Financial Perspective} \\
\text{Consumer Perspective} \\
\text{Internal Processes Perspective} \\
\text{Innovation Perspective}
\end{align*}\]

\[\text{Population Health Information} \quad \text{Management Information}\]

\(^1\text{Health care includes all sectors: prevention, cure, care and welfare (ten Asbroek et al., 2004 [20])}\]
ment decisions in the specific Dutch context. Second, the complete set of indicator areas (Table 1) had to be applicable to the entire Dutch health system.

**Consumer perspective**
The consumer perspective presents information concerning the link between the health system and the health status of the population. We identified the following outcome-focused sub-questions: Are people prevented from becoming ill? Do patients get better? Are disabled persons taken care of properly? Does the health care system help patients attain good health? Are the risks to which patients are exposed acceptable? Do patients have a choice of insurers and providers? Is the Dutch population in general – and its patients in particular – satisfied with the way the health care system functions? These sub-questions are answered in four indicator areas:
- Effectiveness of health care (in all sectors: prevention, cure, care, and social services)
- Patient safety
- Availability of choice of insurer and provider
- Patient-centeredness

**Financial perspective**
We needed to answer the following sub-questions: What are the costs of health care? Do the market mechanisms (regulated or otherwise) function? Is the financial burden for consumers equally distributed (who pays for it?)? Are all individuals able to carry this burden? Can financiers and providers guarantee continuity of care and are they financially viable? These questions are represented by the following indicator areas:
- Health system costs
- Functioning of the market
- Vertical equity
- Financial accessibility
- Financial viability of care financiers and care providers

**Internal business processes perspective.**
The sub-questions we identified included:
How do the financiers – i.e., private and social insurers (for acute care), care agencies (for exceptional medical expenses) and municipalities (for preventive care and population screening) perform? How do they cope with their responsibilities? Do they purchase the right volume and types of care? Is the provided care of good quality? Are there enough human resources to provide the necessary care currently required? Does the market mechanism enforce the creation of new professions or concentrations of care provision? What are the effects for the patient: does the care delivery setting change? The corresponding indicator areas are:
- Performance of care financiers (a. purchasing of care, b. productivity, and c. waiting lists)
- Quality of the health care delivery process
- Concentration of care provision
- Human resource management (1): a. availability of staff, b. vacancies, and c. staff satisfaction
- Substitution of care between professions and between care delivery settings

**Innovation perspective.**

We identified sub-questions that focused on the conditions for developing innovative potential and actual innovations: What financial means are made available for the learning and growth function of the health system? Are new technologies being promptly implemented? Does the information infrastructure accommodate innovation? Are employees’ working conditions supportive of innovations? Are professionals motivated to exchange old habits and procedures for new ones? Does the health system anticipate the need for new professionals for the health care delivery of tomorrow (especially in professions with long training pathways)? Are organizational innovations (like new service arrangements) developed and implemented to enable the system to respond to changing contexts, opportunities, needs, and demands? Do the market mechanisms stimulate an increase in learning and growth potential through intensified cooperation between industry and health sectors?

**Table 2** Overview of perspectives and indicator areas

<table>
<thead>
<tr>
<th>Consumer perspective</th>
<th>Financial perspective</th>
<th>Internal business perspective</th>
<th>Innovation perspective</th>
</tr>
</thead>
<tbody>
<tr>
<td>Effectiveness of care:</td>
<td>Health system costs</td>
<td>Performance of care financiers:</td>
<td>Allocation and volume of R&amp;D funds</td>
</tr>
<tr>
<td>prevention</td>
<td>Functioning of the market</td>
<td>purchasing of care productivity</td>
<td>Diffusion of innovations</td>
</tr>
<tr>
<td>curative care</td>
<td>Vertical equity</td>
<td>waiting times and lists</td>
<td>Information infrastructure</td>
</tr>
<tr>
<td>chronic care</td>
<td>Financial accessibility</td>
<td>Quality of health care delivery process</td>
<td></td>
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<tr>
<td>social services mental health</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Patient safety</td>
<td>Financial viability of care financiers and providers</td>
<td>Concentration of care provision</td>
<td>innovative working environment education &amp; training</td>
</tr>
<tr>
<td>Availability of choice of provider and insurer</td>
<td>Human resource management (1): availability of staff vacancies</td>
<td>Organizational innovation</td>
<td></td>
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<tr>
<td>Patient-centerdness</td>
<td></td>
<td></td>
<td>Link with the industry</td>
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<td></td>
<td></td>
<td></td>
<td>Substitution of care between professions and settings</td>
</tr>
</tbody>
</table>
The corresponding indicator areas are:
- Allocation of funds for learning and growth
- Diffusion of new technologies
- Information infrastructure
- Human resource management (2): a. innovative working environment and b. training of new staff in professions with long training pathways
- Development and diffusion of organizational innovations
- Industry-initiated research and development activities in health care

For each of the four perspectives, background papers were written setting out boundaries and definitions for the perspective and the indicator areas. These gave the first proposals for concrete indicators that could possibly be used.

**Measurement principles**

In the next step of development, the indicator areas in the framework needed to be fleshed out with actual indicators. The Ministry choose a pragmatic approach: the first assessment for the proposed indicator areas was “What does already exist” and secondly “Can this be aggregated to national level?”

Some indicator information already existed, such as the number of accredited health care institutes as a measure for quality of providers. Information for this indicator can be drawn directly from the national registry of accreditation for health care institutions. Other indicators would need aggregation of information into one health system measure. For example, the number of professionals necessary to deliver health care is now calculated for the different sectors in separate registries. Aggregation of these data seems feasible. For others indicators, like the “patient and consumer experience and satisfaction”, new initiatives are necessary to generate suitable information. Although measurement tools for patient satisfaction exist in different forms and shapes for different care settings, standardized methods are needed to measure patient and consumer experiences that can be aggregated to health system level. For this example the CAHPS® [11] instruments are now being introduced in the Netherlands by a consortium of different stakeholders.

For the Ministry it has always been clear that the implementation of the framework would need to build on existing data and data collecting systems. In the Netherlands, data collection in health care is fragmented. There are separate registries for institutions like hospitals, nursing homes, mental health care institutes, and obstetric care. Statistics Netherlands CBS (the national bureau of statistics) also collects data on health care and health care consumption. Some of the health care institutes have regional data collection systems, like the cancer registry. Indicators that could be built on these existing data sources were given priority. This meant that instead of building new systems, the coordination and cooperation between existing registries needed to be strengthened. For example, in a pilot project, hospital admis-
Dissemination strategies

Interactive research input
The dissemination of the framework was facilitated by an intensive interactive development process involving multiple stakeholders.

All the subdivisions at the Ministry were represented in the working group “Knowing Better” (in Dutch: Beter Weten), an intra-departmental project group of approximately 30 participants, chaired by a director-general of the Ministry and coordinated by members of the SEIZ. In this working group, the information policy issues within the Ministry were the subject of debate, and new developments were disseminated in the Ministry through this network of sub-departmental representatives (see Figure 1).

Frequent meetings
Researchers met with representatives of the strategic coordination group on a bi-weekly basis, and on a monthly basis with the intra-departmental project group and the strategic coordination.

The choices for the model and the indicator areas — made in the meetings between the research team, strategic coordination team and intra-departmental project group — were the result of decision-making by consensus after discussing the strategic goals of the health...
system, the information needs of policy makers at the Ministry, and studying existing theory and international experiences with national performance indicator frameworks. In addition, the research group was reinforced with members from the Ministry, the NIVEL, and from the RIVM.

The interactive approach for the development of the framework allowed the framework to be tailored to the Ministry’s information and policy needs. The research group was most active during the period January 2002 through June 2003, when the conceptual framework was developed and the indicator areas were defined in the four perspectives of the balanced scorecard. This also marked the beginning of the period when the Ministry took clearer ownership of the framework. The researchers’ input became less intensive and was given on an “as needed” basis.

**Communication plan to moderate societal debate**

As the conceptual basis for the framework became more definite and accepted by both the project group and ministerial staff, a communication plan was developed to inform the stakeholders outside the Ministry. The Ministry presented the conceptual framework at two invitational conferences. The first was held on 29 January 2003. Those invited to attend included managers and policy makers of health care insurers, provider organizations, patient and consumer organizations, home care organizations, and the national mental health umbrella organization (GGZ Nederland). During this conference, the discussion with those attending focused on the policy of measuring performance at macro level. In the second conference, held on 12 March 2003, the participants were data managers (and those involved with reporting on performance) from the same institutions and organizations invited to the January conference. This conference focussed on the practical implications of measuring performance with a limited set of indicators.

The conferences were supported by two publications: “Setting a New Course” (in Dutch: *Bakens Zetten*) [21], which presented the conceptual framework, and a discussion paper that presented the framework and its proposed indicator areas [22].

With both conferences, the Ministry wanted to initiate a broad societal debate on performance measurement. This debate continued in national health care journals [23;24] and at health care conferences, such as “Sharing Knowledge Better” (in Dutch: *Kennis beter delen*) and “Calculating Policy” (in Dutch: *Cijferen met Beleid*). The government also opened a website for discussions with the public about the changes in the health care financing and insurance structure as well as for measurement of the performance of the system as a whole [25].

**International cooperation**

At international level, the Netherlands actively participated in the OECD Health Project and in the European Community Health Project. The Secretary-General of the Dutch Ministry of Health co-chaired this OECD project, which made clear the Netherlands’ motivation to align its health policy to international developments. In the OECD’s Health Care Quality Indica-
tor Project, the Netherlands was represented by the head of the research team supporting the Ministry in its development of the national framework. This created a strong network between the OECD and the project group developing the Dutch framework. When choosing the indicator areas, particularly with regard to the consumer perspective of the balanced scorecard, information was provided by the newly selected indicators in the OECD project. In the same way, the discussions at the Ministry provided information for the OECD project, especially for the indicators on prevention and primary health care. This relationship of jointly developing performance indicators substantially strengthened the support at the Ministry.

A similar interaction occurred in the European Community Health Indicator project. The RIVM has been the secretariat for the project since it started in 1998. Although the project originally focused on health status data, as of 2003 it now also includes health system information, taking its example from the OECD project. The involved researchers at RIVM took part in the research team for developing the performance indicator framework in the Netherlands, and were also invited to participate as experts in the OECD plenary sessions and working groups, which optimized the alignment between the three indicator initiatives.

**Consulting knowledge institutes**

By actively participating in the international projects, the project group at the Ministry was encouraged to give priority to developing the indicator framework. The indicators sets developed at the OECD and EU served as good examples for the Dutch indicators. However, the OECD indicator set was strongly focused on curative care, and the EU set on health status. These indicator developments initiated a sense of urgency at some policy directorates in the Dutch Ministry (including those for mental health and social services) to start developing specific indicators for their own sectors. Several knowledge institutes and consultancy bureaus were approached to develop discussion papers and advisory statements to support sector-specific indicator sets. As a result, these institutes and bureaus had to gather information about the performance indicator framework and its underlying motives, and this contributed further to the dissemination of the framework.

**Scientific spread**

To further increase the awareness about the performance indicator framework in the Netherlands, an additional communication strategy addressed the academic world. We presented the conceptual framework at scientific meetings and conferences and started a meeting series with the RIVM. The RIVM produces the Dutch Public Health Status and Forecasts reports for the government. We made it a priority to hold discussions with them on the health system changes, the potential implications for the health status forecasts, and the consequences for information tools such as the performance indicator framework. Together with RIVM, the possibilities were explored for developing clinical logics [26] described at population level in order to make the link between health status and health system performance more explicit. These clinical logics will be included in the next Health Status Forecast.
This integration of public health information with health care performance information was essential to the construction of the conceptual framework.

Ministerial decision-making

The interactive work of the SEIZ working group and the research support group has resulted in identifying the conceptual model and identifying the indicator areas in the four perspectives in the balanced scorecard. The Ministry decided on the conceptual model in January 2003. This enabled further development of the framework and small working groups were formed for each of the four perspectives to further flesh out the indicators. The name of the working group was changed from “Knowing Better” to “Working Better” (in Dutch: Beter Werken).

In 2003, a steering group was formed within the Ministry’s hierarchical line structure to enable concrete decision-making and further dissemination at more levels in the Ministry. Participating in this steering group were the heads of subdivisions and the heads of the financial and macro-economic directorates (FEZ and MEVA).

By the end of 2003, the working groups at the Ministry that focused on indicator development had come up with a set of plans of action, each proposing an approach to further develop the actual indicators. The organizational structure was adjusted to enable optimal alignment with other current policy themes. Together with the Unit for Patient and Consumer Policy among others, the executive office SEIZ was integrated into a new policy directorate known as the Market and Consumer Directorate (MC). The development of the framework became an important task of this directorate – in this new organizational structure, this development was now clearly positioned in the administrative “line” hierarchy.

Political environment

The opinion was widely shared that the performance indicator framework would only be useful if it would provide information for the Ministry’s policy, steering, and monitoring functions. The governance model had received a great deal of attention within the Ministry, but with the frequent political changes it hadn’t been developed enough to be implemented. Three governments were in power while the framework was being developed, and each were made up of different coalitions: in 2002 by liberals, social democrats, and democrats; in May 2003 by liberals, Christian democrats, and liberal populists; and at the end of 2003 by liberal democrats and Christian democrats. During this period, the system redesign and redefinition of the roles of the actors in the system was not further developed. With the start of the second cabinet under the leadership of prime minister Balkenende in November 2003, a relatively stable environment came into being in which decisions regarding health system policy could be taken. This period made a stronger positioning of the framework possible, as well as further development of the governance model and the resulting tasks and responsibilities of the involved organizations.
Policy advice by the National Council for Public Health

In September 2003, the State Secretary for Health asked the National Council for Public Health (RVZ) to formulate a recommendation on the potential functions of the framework and how it could best be applied in the current Dutch health care context. The RVZ’s recommendation focused on the managerial applications of the framework and the need for clearer communication strategies. It also advised using the framework to monitor the consequences of the planned new social health insurance [27].

Pilot testing

Throughout the entire development process (including during the informational conferences) questions arose about the framework’s practical implications. A pilot exercise was undertaken to explore what existing information could be used to flesh out the indicators in the identified areas. Prismant, the leading organization for data collection and data management in the curative care sector, carried out this exercise in 2004. Expert consultations were held for each of the four perspectives. Health care data collection and data managing organizations were involved in the pilot test on an invitational basis. As a result, many organizations were informed about the framework development and the potential information needs of the Ministry in the near future. This project also boosted the development of the action plans that had been developed by the indicator working groups at the ministry. The pilot test showed that the availability of ready to use indicators is still very limited and, if existing, often not covering the whole system, or not covering all sectors. In its final presentation Prismant stated that only 14 out of 26 indicator areas could be filled with currently available information. This confirms our impression that not many “ready to use” indicators – for the proposed indicator areas - do exist.

Activities foreseen in the near future

By the end of 2004, the strategic position of the framework had been formulated. The performance indicator framework will be the basis on which the Ministry will report the results of health care policy to parliament and the public at large. This new document, called The Balance of Care, will be published bi-annually. Additional plans are being developed to build a web-based information system that presents the entire framework.

The RIVM was asked to develop and implement the individual indicators and to complete the framework and provide the content for the first edition of The Balance of Care, planned for publication in May 2006.

Chronological overview of activities and strategies, and stakeholders involved

2001: On invitation of the OECD, Klazinga presented the situation on health system performance measurement in the Netherlands as a case study at the OECD conference “Measuring UP” in Ottawa (autumn 2001). Top-level ministerial staff participated in the OECD Health Project working group (among them the Secretary General). During this period,
the Dutch ministry identified the need to be able to measure performance in health care at system level.

January – June 2002. Developing the conceptual framework. The ministry of health involved the academic research group to support the development of a framework for health system performance indicators. In this period most activities were carried out by the research team. Progress was presented in monthly “Knowing Better” working group meetings. At national level, the research group consulted institutes that are involved in health statistics and public health. RIVM, NiGZ, Nivel. Members of Nivel and RiVM became permanent members of the research team. At international level, the principal investigator represented The Netherlands in the OECD indicator project and participated in other networks focussing on indicator development. At the MoH, departments that had performance measurement on their agenda gave and got feedback to align other policies with the developing PIF. This occurred already in the first months. Researchers and the coordinating Strategic Unit for Information Policy in health care (SEIZ) tried to make all efforts convergent.

July 2002 – January 2003. When the first sketchy contours of the framework became apparent, the discussions in PBW focussed on the question “What does it mean for “my” department”. This was discussed at different levels. Within the ministry the topic of the framework became known to more people. In January 2003 the framework—with indicator areas, not indicators— was officially endorsed by “Knowing Better” and decided upon by a top level steering group for information policy (SIZ). The framework became a tool (under construction) owned by the ministry.

February – June 2003. Activities focussed on the development of the individual indicator areas, 26 in total. For each of four indicator groups a group manager was assigned, for each indicator area a project leader. He/she lead the development of a specific indicator. Most group and project leaders attended PBW frequently. Researchers participated in indicator group meetings and met separately with project leaders on request. In two invitational conferences the PIF was communicated with other parties in the health care field. 75 Institutes and organisations were invited and 180 participants were welcomed. In this period the ministry adopted the proposed framework. The activities focussed on the development of the 26 individual indicator areas. For each of four indicator area groups a group manager was assigned, for each indicator area a project leader.

July 2003 – December 2003. Project leaders developed proposals for individual indicators. Completeness of the proposals varied largely. Some were very explicit and included data, others were very global still.

Integration of indicators into the existing budgetary planning tools was proposed to link the framework with the governmental budgetary planning and control cycle. This initiative of the financial department was informative about the non-budgetary character of the framework and demonstrated the different goals of, on the one hand, the framework and the budget plans and reports on the other.
January – June 2004. To strengthen the development of the framework a project started to fill as many indicators as possible. This exercise, carried out by a national health care data registry institute (Prismant), was guided by the 26 proposals. The Council for Public Health and Health Care (RvZ) advised the MoH on the strategic positioning of the framework in the existing organisational structure of the MoH and in the overall picture of roles, responsibilities and steering mechanisms.

To build a stronger influence on the development process of the PIF and other information policies the ministry created a new department within the hierarchical line structure that addresses the ministry’s information policy agenda. The new department is built on the existing frame of SEIZ but with a stronger position within the ministry and linked to the health system reforms.

July 2004 – January 2005. The newly formed “Market and Consumer Department“ was given the responsibility for the co-ordination of the policy processes necessary for the implementation of the framework. The advices of RvZ and Prismant will be integrated in the policy processes.

The national institute of Public Health and the Environment (RIVM) will develop the technical-infrastructure that is needed to fill the indicators with data. The involvement of the RIVM stresses the importance of public health data in the framework. RIVM coordinates the network of institutes that will contribute information for the individual indicators. The technical infrastructure will provide longitudinal information to monitor the system’s performance. This monitoring information is expected to be published bi-annually in the “Balance of Care”. Table 3. summarizes the activities.

Evaluation

During the period of health system redesign the development of the framework has put health system performance measurement on the agenda of the Ministry’s subdivisions and directorates and on the agendas of its counterparts. Also, the integration of public health information and health care management information was discussed at length. Although the currently dominant sectoral approach has not been replaced, the integration of sectors is receiving more attention.

The need for the performance framework was expressed at a time when international initiatives and national needs formed fertile ground for the prosperous growth of the health system performance indicator framework. But more than just fertile ground is needed for a good harvest – the weather conditions also have to be favourable. The conditions under which the framework had to be developed can be summarized as the policy context and, more specifically, the dominant policy developments that occurred parallel to the development of the performance indicator framework.
### Table 3. Summary of activities

<table>
<thead>
<tr>
<th>Activities</th>
<th>Stakeholders</th>
<th>2002</th>
<th>2003</th>
<th>2004</th>
</tr>
</thead>
<tbody>
<tr>
<td>Development of conceptual framework</td>
<td>AMC/SEIZ/PBW</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Identifying indicator areas</td>
<td>AMC/SEIZ/PBW</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Endorsement of Conceptual model and its indicator areas,</td>
<td>SGiP/PBW/SEIZ</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Communication Plan: Involvement of organizations and institution, Conferences</td>
<td>SEIZ</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Development of action plans for individual indicators</td>
<td>AMC/SEIZ/PBW</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Pilot Implementation Exercise : Which Indicators can already be filled?</td>
<td>Prismant/STATDAT/AMC/WGI</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Assessment of Strategic Positioning of the framework in the existing organisational structure of the Ministry</td>
<td>RVZ</td>
<td></td>
<td></td>
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</tr>
<tr>
<td>Organisational changes, repositioning of Framework activities</td>
<td>SGiP,</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Summarizing Framework Developments 2002-2004</td>
<td>AMC</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Developing concrete indicators, preparing for Balance of Care (due 2006)</td>
<td>RIVM</td>
<td></td>
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</tr>
</tbody>
</table>

1 the involved participants of invitational conference were: HCI, UMBR, NPCF, STATDAT, INSUR, INSP
2 the involved resource organisations for pilot implementation by Prismant were: STATDAT, Nivel, INSP, UMBR, PolDep,
3 the involved organisations of a meeting to inform RVZ for their advise to the Ministry were: UMBR, STATDAT, HCI, PolDep, SGiP, SEIZ, AMC, NPCF, INSUR, INSP

### List of abbreviations for stakeholders

<table>
<thead>
<tr>
<th>Stakeholders</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>SEIZ</td>
<td>Strategic Unit Information Policy (MoH)</td>
</tr>
<tr>
<td>PBW</td>
<td>Ministerial Project Group (MoH)</td>
</tr>
<tr>
<td>SGiP</td>
<td>Steering Group Information Policy (MoH)</td>
</tr>
<tr>
<td>WGI</td>
<td>Working Groups for Indicator development (MoH)</td>
</tr>
<tr>
<td>MCD</td>
<td>Market and Consumer Department (New since Oct 2004)</td>
</tr>
<tr>
<td>PolDep</td>
<td>Policy departments (MoH)</td>
</tr>
<tr>
<td>AMC</td>
<td>Academic Research Team Academic Medical Centre University of Amsterdam</td>
</tr>
<tr>
<td>RIVM</td>
<td>Researchers from RIVM</td>
</tr>
<tr>
<td>Nivel</td>
<td>Researcher from Nivel</td>
</tr>
<tr>
<td>NiGZ</td>
<td>Researchers from NiGZ</td>
</tr>
<tr>
<td>INSP</td>
<td>Inspectorate for Health Care (MoH)</td>
</tr>
<tr>
<td>Prismant</td>
<td>Prismant</td>
</tr>
<tr>
<td>RVZ</td>
<td>Council for Public Health and Health Care</td>
</tr>
<tr>
<td>HCl</td>
<td>Health Care Providers institutions (n=40)</td>
</tr>
<tr>
<td>UMBR</td>
<td>INSUR</td>
</tr>
<tr>
<td>NPCF</td>
<td>National Patients and Clients Federation</td>
</tr>
<tr>
<td>STATDAT</td>
<td>Statistics and Registry Managing Organisations</td>
</tr>
</tbody>
</table>
At the Ministry, six parallel policy developments can be identified that influenced the development process of the performance indicator framework [28]:

**From budget accountability to policy accountability**
The first policy mentioned here is one that has contributed significantly to the idea of developing a health system performance measurement system. The inter-ministerial policy “From budget accountability to policy accountability” (VBTB, an initiative of the Ministry of Finance) was initiated to increase outcome-oriented policy making and accountability. It was implemented in all ministries, and the previously dominant budgetary orientation had to be abandoned.
The performance indicator framework could serve perfectly as a tool to achieve and reflect an outcome-oriented vision on policy making in health care. Also, it would provide a framework for reporting overall performance in a transparent way. The VBTB strategy had – and still has – a strong link with the financial policy making and accountability structure. The development of the framework therefore enjoyed the keen interest of the Ministry of Health’s directorate of Financial and Economic Affairs (FEZ).
In summary, the VBTB strategy had a strong and positive influence on the development of the performance indicator framework.

**Redesigning the budgetary planning and control reports**
The FEZ sub-department at the Ministry of Health, which was especially interested in implementing the VBTB, explored the possibilities of integrating the indicator areas of the performance framework into the existing budgetary planning tools. In doing so, the framework would be linked with the governmental budgetary planning and control cycle, while at the same time facilitating the needs of the VBTB strategy. This investigation by FEZ provided information on the non-budgetary character of the framework and demonstrated the different goals of the framework on the one hand and the budgetary plans and reports on the other. Because the FEZ was aware that the framework content and budgetary planning and control reports were not a perfect match, this prompted it to prioritize redesigning its own reporting structure separately from that of developing the framework. Subsequently, the two emerging information structures were felt to be potentially competitive. At coordination meetings it was explicitly expressed that it was important to align both developmental processes and perceive them mutually as synergetic and congruent rather than competitive. A positive effect of the exploration by FEZ to integrate the indicators in their accounts and budgetary documents is that it showed the framework has a different nature and serves different goals than those shown in the existing FEZ reports from 2003. At this point it is unclear how the new financial planning and control reports will be structured and how this relates to the perspectives, indicator areas, and indicators of the performance indicator framework. The alignment of both developments will need ongoing attention.
Reducing the administrative burden

During the 2003 parliamentary elections, the government had targeted the decrease of administrative burden in the health care system as a priority. A policy was formulated that explicitly stated that the administrative burden should be reduced by 25%. This reduction was to be achieved by decreasing the number and length of the questionnaires sent to care providers by the Ministry. Providers feel that the government requests the same information over and over again, although sometimes with a slightly different focus. Different sub-departments at the Ministry sometimes even ask for the same information. It emerged during the working group meetings that participants felt more efficient information-gathering (i.e., omitting duplicate requests) would reduce the administrative burden by 25%, if not more.

In the working group “Knowing Better” it was expressed time and again that because of the administrative burden, the indicators should be built on data from existing registries and other information-collecting systems currently in use. Also, the expectation was that if the Ministry wants to have a comprehensive yet limited set of indicators (as proposed in the framework), less information will be needed rather than more. In such a situation, the framework would benefit from the policy of administrative burden reduction. It became clear, however, that information was not readily available for some of the proposed indicator areas. Because of this, new and additional data collection mechanisms would be necessary. These were viewed with reservation, as they could lead to an increase rather than a decrease in the administrative burden.

It is as yet unclear to what extent the framework contributes (or fails to contribute) to a decrease in the administrative burden.

Promoting the public interests: Affordability, Quality, and Accessibility

In the current political vocabulary, the government communicates messages relating to the performance of the health system in terms of three public interests: affordability (can health be paid for?), quality (does it meet a minimum standard of quality of health?), and accessibility (can all Dutch people make use of a basic package of care?). The government indirectly controls health care institutions, health care insurers, and health care consumers by using aggregated market information collected and interpreted by governmental regulatory and standardizing or supervisory agencies. This information will be compared to macro information, such as indicated in the performance indicator framework. Subsequently, a confrontation between market information and macro information should lead to the assessment of the level of attainment of public interests in health care. This information guides the steering from a distance the government employs on the health care market. This vocabulary of public interests emphasizes health care performance from the consumers’ perspective. Although the performance indicator framework follows different ordering principles, the communication in terms of public interests does not necessarily conflict with the chosen framework. The framework will be used as a tool to form a database for analysis, informa-
tion development, and decision-making in health care. The communication regarding these decisions can be formulated in terminology of public interests. In such a way the framework provides a knowledge base that does not dictate the political rhetoric and is potentially less vulnerable to political change.

**Redesign of governmental audit and control functions**

Accessibility, affordability, and good quality will set the preconditions for decisions in the primary process of care delivery. Supervisors guarantee and manage these public interests based on a public testing framework, and keep the primary processes in balance with public interests. In the present situation, a number of agencies regulate the access to the primary process of health care institutions, and these agencies regulate the implementation of the insurance laws and regulate the market behaviour in some market segments. The Ministry tries to control the primary process through supervisors (Health Care Inspectorate, the Health Care Authority (in Dutch: “Zorgautoriteit”), and the Health Care Insurance Supervisory Board), who will identify failures to achieve efficient coordination of decisions in the health care market. In addition, the Ministry formulates norms related to public interests and also determines the testing framework of the supervisory body. The roles and responsibilities of the supervisory bodies are currently in flux, and the place and position of the present supervisory bodies and regulating institutions will be reconsidered. Legislation to this effect is currently being drawn up. The redesign of governmental audit and control functions has the potential to positively effect the development and implementation of the national framework. Further definition of the roles and responsibilities of supervisory bodies and regulating institutions will affect the choice and formulation of specific indicators that are directly related to these supervisory and regulating roles (i.e., the functioning of the markets, patient safety, and performance of care financiers).

**Reorientation of health information infrastructure**

The reorientation of health information infrastructure at the Dutch Ministry of Health is a three-pronged policy that includes the development of a performance indicator framework. The first aspect is the policy for arriving at a minimal data set (MDS), which is the minimum data the concerned parties in health care need from each other (i.e., data on production, quality, and capacity) in order to function optimally. These data will be analysed at central level, in this way forming a minimal data set for every sector or market in health care. Because the different parties in the health care market need information focused on their new roles (including new information), a data management function (data warehousing) will be formed.

The second aspect of information policy is the construction of one or more Trusted Third Parties (TTPs). In these constructions, an independent third party becomes the manager of the data collected in the MDS. The concerned parties (consumers, health institutions, health insurers, and government) will be given a key that determines what information is accessible
to individual parties. Agreements must be put down on paper and evaluated periodically to optimize efficiency in data collection and data use.

The third aspect is the development of performance indicators. Based on the available information from the MDS, performance indicators can be developed that are derived from the actual health care processes. At its core, the Dutch performance indicator framework for the health system includes information from the MDS. In principle, the policy of reorientation of the health information structure is set up in such a way that the three aspects should be developed synchronically. If successful, the MDS and a functional TTP can prove to be important sources of information for the framework and as a result could boost its development and implementation.

At international level, the interest for performance measurement has remained undiminished since the start of the Dutch framework initiatives. The most relevant developments for the Dutch situation are those at the OECD and the EU. There are strong organizational links between the Netherlands and both the OECD project and the EU project. These have contributed to an international policy context that has had a positive influence on the development of the Dutch framework.

The continuation of the OECD Health Care Quality Indicator project beyond its initial project period is expected to have a long-term positive influence on the development and implementation of performance measurement frameworks in its member states, including the Netherlands.

In the EU, the indicator sets of the European Community Health Indicator project (ECHI) used to consist mainly of health status indicators [1]. Now, though, they have incorporated the OECD health care indicators and by doing so has strengthened the link between the OECD and European Union indicator projects. For the EU member states participating in the OECD, the overlap in the two indicator sets sends a signal to national policy makers on the importance of these indicators and of health system performance measurement in general.

In the Netherlands, many actors have indicator development on their agendas. For example, provider organizations and professional associations have developed their own performance indicator sets either as part of internal quality improvement policies or as external accountability instruments. These initiatives have contributed to a positive context for developing the national performance indicator framework. Initiatives were taken to align specific indicator sets with the national performance indicator framework in 2003. However, mis-communication and misunderstanding is a concern because of the different perspectives and goals of the different actors developing these indicators. Organizations have expressed a fear of duplication because of so many different initiatives seemingly focusing on the same information. Careful communication between actors at national level and those at meso and micro levels is important to ensure ongoing support from and for these actors. This support is essential if indicators have to use data aggregated from micro and meso levels. At national level, as of January 2005, the RIVM has coordinated the indicator-
building activities related to the national framework, and will seek cooperation from other institutes to ensure broad support for this task.

**Lessons Learned**

Since 2002, the Dutch Ministry of Health has been developing a health system performance indicator framework for the Dutch health system. This framework includes and links public health information and health care system information. The Lalonde model and a balanced scorecard form the two core components of the Dutch framework. An interactive approach was chosen that involved multiple stakeholders, including a research team and a ministerial working group. This approach proved to be useful. The research team was free to develop the conceptual model for the framework, which made it easier to incorporate existing international knowledge and experience about performance measurement frameworks in health care. The counterparts at the Ministry contributed to the conceptual framework, and coordinated it with the current policy context at the Ministry. Later on, the Ministry took full ownership of the framework and developed it further. This meant more participation by an even larger number of stakeholders, while at the same time a redesign of the organizational structure at the Ministry ensured more decision-making power of those working on the framework. A communication plan, advisory consultation, and a pilot test contributed to its further development.

Building the framework for performance measurement in a politically complex context has its specific challenges. Within this context, ambiguous terminology and implicit notions are part and parcel of day-to-day reality. To develop the performance indicator framework, it is necessary to be explicit about policy goals. Policy makers’ timing for choosing to be explicit about these goals did not always coincide with the timing preferred by the developers of the framework on the ground, including the researchers. This challenged the framework’s development while at the same time it stimulated the debate on the policy goals at the Ministry. Other researchers have been disappointed in the way their work was incorporated into the policy making process. Their research report was received with enthusiasm before indefinite storage in an anonymous drawer brought an end to its career. In contrast, the researchers involved in the work described here have been working side-by-side with policy makers who had expressed a need for the instrument they were building together. This close interaction optimized the applicability and acceptance of the performance indicator framework that was developed.

The development of the Dutch health system performance indicator framework proved to be a social and political process as well as a methodological exercise. It needed to be carefully managed as a research-based, policy-led change process. In this process, other policy developments were also relevant, and acknowledging this interaction rather than ignoring it strengthened the positioning of the framework.
The managerial and organizational contexts in which health system performance frameworks need to be developed are dynamic, difficult to predict, and cannot be steered as an isolated independent process. In the case of the Netherlands, linking the framework’s development to other developments and policy processes has proven successful. Both international and national developments could be made instrumental in dissemination of and communication about the Dutch framework.

Reference List


Chapter 11


Chapter 12

Health services research at work in Case III
The last case in this thesis concerned the development of an instrument for national health system performance measurement. We provided research assistance to the Dutch Ministry of Health (MoH) for developing a framework of performance indicators. The health services research group consisted of public health specialists, medical doctors, health scientists, epidemiologists and a political scientist. This research work was commissioned directly by the department at the Ministry of Health responsible for developing the instrument. A working group at the MoH regularly provided feedback on our work.

The initial content question (developing a performance indicator framework) is addressed in Chapter 10 [1]. Based on Lalonde’s model for determinants of health [2] and on Kaplan and Norton’s Balanced Scorecard [3], our proposal links the public health perspective with the management of health care perspective to measure the performance of the entire health system. We also described the interactive development process and some of the stakeholders in this process.

In addition to the question answered in Chapter 10, we developed a combination of four content-, context- and process-focused research questions: 1) What are the organizational and policy context and processes that influence, and potentially interact with, the development of the performance indicator framework? (presented in Chapter 11 [4]), 2) To what extent are decisions about reimbursement by the Dutch Sickness Fund based on cost-effectiveness information? [5], 3) What are useful and feasible performance indicators for mental health? [6] and 4) How can patient experiences with health care be measured as an indicator of the health care system’s responsiveness? [7]. The results of additional questions 2, 3 and 4 are not presented in this thesis. In Chapter 11, we identified several political and policy processes that influenced the development progress of the performance indicator framework [4]. While some processes contributed to its growth, others obstructed this.

**Inclusion of additional research questions**

As described in Chapters 10 and 11, the development of the performance indicator framework was a very interactive process involving frequent interactions with the policymakers at the Ministry of Health over a three-year period. This created the opportunity to observe the actual policymaking process at close proximity. Therefore, to address the context and process addressed in Chapters 10 and 11, no additional resources or data were used other than those already available to us. During the development process we felt that for certain potential indicators, an extra boost in terms of additional studies might help the MoH develop these indicators. As a result, we formulated three additional research questions. One regarded the efficiency of allocation of resources, a second addressed measuring the performance of mental health care and a third question studied how patient experiences could be measured. The first two studies were carried out by graduate students who were supported by our team as well as their own academic environment [5] [6]. Both research papers were brought to the attention of the relevant stakeholders at the ministry. The study of the measurement of patient experiences was formulated in a proposal by the Netherlands Institute for Health Services Research (NIVEL) and was separately commissioned by the MoH.
Interactions with policymakers and other actors

Because we took a highly interactive and collaborative approach to developing the framework, we were able to closely observe those policy dynamics at the ministry that influenced the progress of the framework. We became regular visitors to the ministry as guest members of the project group that dealt with all policies concerning information and information management within the Ministry of Health. This working group was called Beter Weten (in English, ‘Knowing Better’, and was later called Beter Werken or ‘Working Better’, when there was a push to put the discussions into practice) [8]. Good cooperation with the MoH at different levels allowed for assessing the larger context and processes we perceived to be relevant to the development of the framework. Also, our primary liaison at the MoH for developing the framework participated in our research meetings at the University of Amsterdam. The fact that the liaison was preparing a PhD thesis on policy aspects of the steering and control mechanisms of the government regarding hospitals [9] benefited our cooperation. Specific working groups were formed when the individual indicators in each of the four perspectives of the balanced scorecard had to be defined by MoH staff (see Chapter 10). We participated in these working groups to support the development of the framework. In the ‘financial perspective’ working group we presented the additional study on allocative efficiency. Both the studies on indicators for mental health and measurement of patient experiences were presented in the ‘consumer perspective’ working group.

Follow-up of events and developments

Since January 2005 (the time locus of the last chapter in this case), several developments have taken place regarding the performance indicator framework. Firstly, at national level, the framework has officially been chosen as the basis for a new document, the Zorgbalans 2006 (in English, the ‘2006 Balance of Health Care’) [10]. This Zorgbalans will be the MoH’s accountability document to the Dutch parliament and will provide a comprehensive picture of the performance of the entire Dutch health system [11]. Since then, the National Institute for Public Health and the Environment (RIVM) has proposed a format for presenting the performance indicators that is different from the suggestions we have presented in this thesis. The initial combination of Lalonde’s model and the Balanced Scorecard has been adapted into a set of 26 indicator areas grouped into three perspectives in concordance with three public goals of the Dutch health care system: ‘quality’, ‘accessibility’ and ‘affordability’, and does not include the Lalonde model for determinants of health in one framework [12]. The first Zorgbalans was presented by the minister of health in May 2006 and a website is now available, providing all of the information about the 26 indicator areas and the underlying 125 indicators [13]. Secondly, also at national level, the development of the indicator for consumer and patient satisfaction that followed after NIVEL’s study of the measurement of patient experiences [7] evolved in an impressive spin-off: the translation and adaptation of the United States’ Consumer Assessment of Health Plan Surveys® (CAHPS) [14] into the Dutch context. Combining these questionnaires with existing Quote questionnaires [15] in the Netherlands now
seems to have become the standard by which government, insurers, providers and patient organizations want to measure experiences with health care on a large scale [16;17].

Thirdly, at international level our work regarding the framework evolved as well. We had studied the conceptual basis of indicator frameworks that existed elsewhere as a first and necessary step in developing the performance indicator framework for the Netherlands [17]. Arah and colleagues [18;19] have continued to work on this topic encouraged by the Dutch participation in the Organisation for Economic Co-operation and Development’s (OECD) Health Care Quality Indicators project [20]. This has resulted in the OECD’s adoption of the framework developed for the Zorgbalans as the basis for its own performance indicator framework [21].

In conclusion, our work has directly contributed to the development of performance measurement of the entire health system in the Netherlands. The conceptual framework that we jointly developed with the MoH was used by the RIVM to develop the first Zorgbalans, which was sent to parliament by the minister of health in May 2006 [13]. At international level, this work has contributed to the development of the conceptual framework for the OECD Health Care Indicators Project. Less clear is the way the additional work on resource allocation and mental health indicators has influenced the policymaking process. Overall, we showed that the final product was developed within, despite and because of a dynamic political policy context and process.

Reference List


Chapter 12


Chapter 13

Discussion
This thesis presents three cases of health services research at work for national health policy. All three are research projects in which national health policymakers posed a primarily content-focused research question. These were: a cost-effectiveness evaluation of a genetic screening programme for familial hypercholesterolemia (FH) to support the decision-making process for national implementation (Case I: see Chapters 2, 3, 4 and 5); a cost-effectiveness evaluation of the development and implementation of respiratory care guidelines in Nepal to guide the decision-making process for nationwide implementation and international development (Case II: see Chapters 6, 7, 8 and 9); and lastly, the development of a performance measurement instrument for the entire Dutch health system to be used by the Dutch Ministry of Health, Welfare and Sport (MoH) as a monitoring and steering information tool (Case III: see Chapters 10, 11 and 12). This final chapter provides reflections on these three cases. (See Table 1 for an overview of this thesis with case study results)

We explored two research questions in this thesis to increase the usefulness of health services research for national health policy. Firstly, How can health services research include content-, context- and process-focused research questions? (in addition to the initial research question) and secondly, How can health services researchers participate in an interaction process with policymakers?

Case study results

Inclusion of additional research questions

The strategies to include additional research questions showed similarities in the three cases. Firstly, we tried to interpret our research assignments in such a way that we could answer the additional questions within the initial research contracts. This attempt was not always successful, as was shown by our efforts to study the analysis of the pedigree and completeness of the family tree in the screening programme for FH (see Chapter 5). Secondly, these cases illustrate that addressing the additional questions was partly based on feasibility and availability. Staff, time, costs and supervision possibilities were important ingredients for the decision on whether or not to proceed with a study that was not part of the initial contract. We had the opportunity to involve students to carry out part of the research activities. Within our academic setting, we were able to find means and staff to develop these questions and to answer them. In our cases, the students involved were from the fields of medicine (3), health sciences (1) and medical information sciences (1). All were in the final stages of their academic training. They shared high motivation and constructive criticism that allowed us to seek answers to specific questions within short time frames and at no additional cost. In other settings, such as in institutions that conduct contract research, this opportunity of combining research with academic training of graduate students does not exist and the means and staff to address research questions that were not part of the initial research contract will be increasingly difficult to find. In our three cases, we experienced that other stakeholders
were enthusiastic about additional research when it supported their cause and involved no additional cost.

**Interactions with policymakers and other actors**

Regarding the process of interaction with policymakers and other actors, it is important to point out the differences and similarities of the three cases. The first case – the evaluation of the screening programme for FH – was commissioned by the Netherlands Organisation for Health Research and Development (ZonMw) on behalf of the MoH [1]. In this case, the health services researchers did not interact with the policymakers or with the contractors at the beginning of the research period. After the agreement on the research proposal, the next interaction with policymakers and contractors was at the workshop, when we presented the results of all the studies we had conducted (including the studies on the psychosocial effects of the screening) [2]. Our interaction during the research was mainly with those involved in the programme that was evaluated: the staff and initiators of the Foundation for the Identification of Persons with Familial Hypercholesterolaemia (StOEH) (see Chapter 5).

The second case – the evaluation of the Practical Approach to Lung Health (PAL) in Nepal – was commissioned by the Foundation for the Advancement of Tropical Research (WOTRO), part of the Netherlands Organisation for Scientific Research (NWO) [3], but the request for the evaluation was initiated by the intended user of the research results, the World Health Organization (WHO). The interaction with WOTRO during the research period was limited to producing reports on research progress and financial project management. The main interaction during the research period was with the policymakers at the Nepalese National Tuberculosis Centre (NTC) and the WHO. The interaction consisted of discussions during the preparation phase of the research, feedback sessions during the fieldwork and discussions about the results of the research (see Chapter 9).

The third case – the development of the performance indicator framework – was contracted directly by the MoH [4]. The interaction with the policymakers was intensive throughout the research period because the contractor, co-developer and intended user were the same group of policymakers at the MoH (most of them were members of one department, see Chapters 11 and 12.).

In all three cases, part of the interactions between health services researchers on one hand and policymakers and other actors on the other took place in formalized meeting groups. These were a ‘scientific sounding board group’ (Case I), a steering group (Case II) and an MoH working group (Case III). These formalized settings provided a network for reflecting and discussing the research project, and as a result of these interactions, provided a basis for support for the respective studies. Alongside these similarities in how they functioned, the groups differed in constitution and roles. In Case I, the members of the scientific sounding board group included senior scientists from relevant academic fields and the involved health services researchers. The scientific soundness of the evaluation study was the core agenda point for this group. In Case II, the steering committee included senior staff members of the Nepalese MoH, senior officers of the NTC, senior medical specialists, academics from
the fields of clinical pharmacology and community medicine and family health. The steering committee had two formal roles: firstly, to inform the research and development process and secondly, to approve and support the proposed study. In Case III, the working group of staff members at the MoH had a clear objective for developing an instrument and the health services researchers provided assistance in reaching this objective.

In addition to the formal meeting groups in all three cases, the cases show different intensities of interaction between the health services researchers and the policymakers. Chronologically, the health services researchers had increasing levels of both formal and informal interaction. In the absence of a scale for measuring the interaction process in each of the cases in absolute terms, we can classify the interaction process in one case relative to the other cases. In Cases I, II and III, the interaction processes between health services researchers and policymakers (either chosen or proposed) can be classified as ‘non-interactive’, ‘interactive’ and ‘highly interactive’ respectively.

What lessons did we learn from these cases regarding the interaction process of researchers with policymakers and others? In the first case, during the health services research it became evident that policymakers would use the results as a basis for important policy decisions. The low level of interaction with policymakers at the beginning of the evaluation of the screening programme gave us, the health services researchers, a great deal of research freedom with regard to the policymakers. Towards the StOEH and its initiators, however, a higher level of interaction might have contributed to a better mutual understanding in these changing circumstances. But then again, the fact that the approach of the cost-effectiveness evaluation did not match the perspective of the StOEH probably had a much greater influence on our mutual interactions than can be attributed to our non-interactive mode of interaction. In contrast, the evaluation approach (and results) of the study of the psychosocial effects of the screening programme (not addressed in this thesis) were received positively and the interaction of the researchers in that study with the other actors was perceived as positive.

Similarly, in the second case, our criticism of the PAL package (Chapter 6) temporarily caused tension. Due to the positive interaction process at both formal and informal levels, we could eventually agree on a constructive but critical formulation of the study results and jointly published the evaluation to support the PAL’s development. The highly interactive cooperation between the researchers and the Ministry of Health was stimulating and productive, and our approach resembled much of what Lomas called ‘linkage and exchange’ [5]; in another study regarding the development of questionnaires to measure patient experiences (mentioned in Chapter 12.), though, a similar approach created tensions between researchers and other actors from time to time. Obviously, other factors along with mutual trust are also important for successful interaction, and influence the use of research by policymakers [6]. More generally, it is easier for researchers and other stakeholders to cooperate well if there is mutual trust and if there are positive results to report (i.e. confirming assumptions, answering expectations). The more interactive processes tend to be more dynamic (and are therefore preferred by the author), and can mobilize constructive and creative input. Although the interaction
with others can give rise to obstacles (like negative tensions between actors), these can also more easily be removed because of the interaction process.

**Usefulness of the produced knowledge by health services research**

Measuring the usefulness of health services research for national health policy is best done by looking at its aim: the actual use of the research results in the policy processes. This immediately raises the question whether research results that were not used were not useful. For example, the political instability in Nepal is an important contextual factor that prohibits the further development and implementation of PAL. These changing circumstances are not related to the content of our studies but effectively block their use. Therefore, to judge the usefulness of our PAL evaluation by its actual use in Nepal is harsh. Nevertheless, in the absence of major obstructing contextual factors (such as political instability), ‘use’ is probably the best indicator for ‘usefulness’. We will discuss the use of research results presented in this thesis, produced by health services research at work for national health policy, from two perspectives. The first focuses on different ways of interaction between health services researchers and policymakers [7] and the second focuses on the different ways in which the results were used by policymakers [8].

Several authors have described different ways in which policymakers use research (e.g. [7;9;10]). In their review, Hanney et al. [7] summarized several models for research utilization in policymaking that referred to the interaction process between research and policy. At first glance, the first case in this thesis – nationwide screening for familial hypercholesterolemia – resembles the classic/purist/knowledge-driven model: no interaction between researchers and policymakers during the research process, researchers produce knowledge and policymakers receive it when it has been finalized, and either use it in their policymaking process or not. However, this linear sequence of actions did not hold in the aftermath of our case: The development of the nationwide expansion of the screening programme by the Health Care Insurance Board (CVZ) was done in close collaboration with a group of stakeholders, including researchers, and thus followed the interactive/social interaction model. ‘This model is characterized by a set of interactions between researchers and users rather than a linear move from research to decisions. It ensures that they are exposed to each other’s worlds and needs’ [7]. With the addition of one content and one context question in this case (Chapters 2 and 3 respectively), we aimed to increase the usefulness of the produced knowledge for the policymaking process. Although the knowledge produced in these studies cannot be linked causally to developments since our publications, two aspects suggest a positive influence. Firstly, the Health Council [11] called for further investigation of the percentage of persons with a proven genetic mutation for FH but without high cholesterol levels. Secondly, there is increased attention for the role of GPs in managing hypercholesterolemia in persons with FH [12]. This influence fits with what is described as the enlightenment/percolation model according to which research is more likely to be used through gradual sedimentation of insights, theories, concepts and perspectives [7]. Whether these studies have also increased the usefulness of the cost-effectiveness analysis seems likely, as the issues discussed
in Chapters 2 and 3 addressed the assumptions underlying the programme and consequently influenced the results of the cost-effectiveness analysis. The fact that these issues have been put on the agendas of the coordinating institution (the CVZ) and its advisory expert committee that guides national implementation suggests that the cost-effectiveness analysis was meaningful beyond the result of the costs per life year gained.

In the second case, the interactive/social interaction model applies to some extent: Some of our findings presented in Chapter 6 could be addressed at an international forum before our final results were available because of our interaction with a wider network of PAL researchers and interested policymakers. This benefited PAL's international development. The use of the two additional studies related to the context of PAL in Nepal (Chapters 7 and 8) fit better within the enlightenment model. An example of this is the interest in exploring the involvement of the private health care sector based on our study presented in Chapter 7 [13]. It is unclear whether the exploration of the priority-setting method (Chapter 8 [14]) would support the development of PAL in Nepal in more peaceful and stable circumstances. As the results of the cost-effectiveness analysis have only recently been published, it is too early to judge the effect of the addition of the three studies presented here on the usefulness of the cost-effectiveness analysis. However, as these studies provide a broad and detailed perspective within which the cost-effectiveness analysis can be understood, it seems likely they increase its usefulness; additional study is necessary to explore this further.

The third case can also be described in terms of an interactive/social interaction model. The usefulness of our results can be derived from the fact that the conceptual framework was published by the MoH in a series of publications regarding the Dutch health system reform [15] and by the fact that the conceptual framework is currently being used to develop the Zorgbalans, the MoH's accountability document for the Dutch parliament (released in May 2006) [16]. The additional studies for individual indicators (mentioned in Chapter 12) brought the development of the performance indicator framework directly to the attention of a wider network of researchers and policymakers, both nationally and internationally. This increase in awareness and understanding of the framework has generated broader support and consequently contributed to its usefulness. Although these studies can be regarded as extensions of the initial content question, they can also be viewed as ingredients to increase the development process and implementation of the framework.

If we apply the categorization for research utilization outcomes as summarized in Lavis et al. [8] to indicate the utilization of our findings, we conclude that our findings on the screening programme’s cost-effectiveness study (Chapter 4 [17]), the study of the ‘implementability’ of PAL in Nepal (Chapter 6 [18]) and the development of the performance indicator framework (Chapter 10 [19]) have been used instrumentally, i.e. several research findings have been used in policy formulation. The findings of the other studies have been used conceptually, or at least have the potential for this. For example, developing the performance indicator framework has contributed to the shift of thinking for some of those at the MoH, which increasingly is executing its steering function based on indicators of performance rather than on budget figures. This is a major change: even more so because these indicators apply
Table 1. Thesis overview with case study results: research questions, interaction process within HSR at work for national health policy and utilization.

<table>
<thead>
<tr>
<th>Case</th>
<th>Chapter</th>
<th>Title</th>
<th>Content-, context- or process-focused research question</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Introduction</td>
<td></td>
<td></td>
</tr>
<tr>
<td>2</td>
<td>Results from a family- and DNA-based screening programme for FH</td>
<td>Content</td>
<td></td>
</tr>
<tr>
<td>3</td>
<td>Genetic screening for familial hypercholesterolaemia in 1992-1997: primarily younger patients in the care of general practitioners</td>
<td>Context</td>
<td></td>
</tr>
<tr>
<td>4</td>
<td>Cost-effectiveness of a family- and DNA-based screening programme on familial hypercholesterolaemia in the Netherlands</td>
<td>Content</td>
<td></td>
</tr>
<tr>
<td>5</td>
<td>HSR at work in Case I</td>
<td></td>
<td></td>
</tr>
<tr>
<td>6</td>
<td>Implementing global knowledge in local practice: a WHO lung health initiative in Nepal</td>
<td>Process</td>
<td></td>
</tr>
<tr>
<td>7</td>
<td>How did you get here? Twenty-six journeys on the road to tuberculosis treatment in rural Nepal</td>
<td>Context</td>
<td></td>
</tr>
<tr>
<td>8</td>
<td>A rational multi-criteria approach to priority setting: Should a lung health programme be implemented in Nepal?</td>
<td>Process</td>
<td></td>
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<tr>
<td>9</td>
<td>HSR at work in Case II</td>
<td></td>
<td></td>
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<tr>
<td>10</td>
<td>Developing a national performance indicator framework for the Dutch health system</td>
<td>Content</td>
<td></td>
</tr>
<tr>
<td>11</td>
<td>The Performance Indicator Framework of the Dutch health system, a progress report</td>
<td>Context and process</td>
<td></td>
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<tr>
<td>12</td>
<td>HSR at work in Case III</td>
<td></td>
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<tr>
<td>13</td>
<td>Discussion</td>
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</table>

1 HSR= health services research
### Health services research at work in Case I, II and III

<table>
<thead>
<tr>
<th>Inclusion of additional research questions</th>
<th>Interaction process within HSR at work for national health policy</th>
<th>Utilization type [8] and description of utilization</th>
</tr>
</thead>
<tbody>
<tr>
<td>- Testing the assumption that FH screening is 'probably nearly 100% coverage'</td>
<td>Non-interactive: There was contact with policymakers only at the end of the study when results were presented. Policymakers did not participate in 'scientific sounding board' meetings</td>
<td>Conceptual. With these findings we contributed to the debate on genotype-phenotype in FH.</td>
</tr>
<tr>
<td>- Funded as part of the initial proposal</td>
<td></td>
<td>Conceptual. With these findings we contributed to the discussion about the role of general practice in case management of FH.</td>
</tr>
<tr>
<td>- Assessing the proportion of newly identified FH patients who were already known in general practice to have hypercholesterolaemia</td>
<td></td>
<td>Instrumental. The study results were included in decision-making process of the MoH.</td>
</tr>
<tr>
<td>- Not separately funded, part of academic medical training</td>
<td></td>
<td></td>
</tr>
<tr>
<td>- Initial research question: Assessing the cost-effectiveness of the screening programme</td>
<td></td>
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</tbody>
</table>

| - Assessing the circumstances and processes in which PAL was developed and implemented | Interactive: Throughout the economic and developmental evaluation we communicated with the policymakers. Policymakers were participants in ‘steering committee’. | Instrumental. The implementation strategy for PAL has been adjusted on the basis of the study findings. |
| Funded as part of initial proposal, supplemented with department funds | | Conceptual. With these findings we contributed to the discussion about passive case-finding of tuberculosis in Nepal. |
| - Assessing the health care context in rural Nepal from the perspective of patient experiences | | Utilization is as yet unclear (potentially conceptual). With this study, we aim to contribute to the prioritization debate and methodology. |
| - Privately funded, part of academic medical training | | |
| - Assessing the relative priority of PAL in Nepal and exploring a method for priority-setting | | |
| - Funded as part of the initial proposal, supplemented with department funds | | |
| - Initial research question: developing a tool for measuring health system performance | Highly-interactive: Policymakers participated in the research process, and the health services researchers participated in the knowledge-generating process at the MoH. Policymakers participated in formal meeting groups. | Instrumental. Our conceptual framework was used as a starting point for the development of the national document Zorgbalans [16]. Utilization is as yet unclear (potentially conceptual). With this study we aim to contribute to knowledge about interactive research/policy processes. |
to the entire health system and not only to one of the four sectors, which is the way the steering functions of the ministry have been organized historically. Symbolic use – using research to justify a position or action that has already been taken for other reasons [8] – was not observed in the studies presented in the chapters in this thesis.

**Methodological considerations**

Several methodological considerations need explicit attention. Firstly, the cases in this thesis represent health services research as it occurred, i.e. not with a prior focus on an analytical model or with a prior plan to study different interactions and the translation of additional policy questions into researchable health services research questions. Because we applied Walt and Gilson’s [20] model retrospectively to our cases, the cases do not present an ideal type of balanced research whereby each case has studies on content, context and process, and with actors explicitly addressed in all of them. This is shown in Table 1 in Chapter 1. Furthermore, over the years of the author’s involvement in the cases described here, the author’s perspective towards the production of scientific knowledge changed from a content-oriented perspective towards a process-oriented perspective. Consequently, the cases developed towards context and process in chronological order. For example, in the first case (evaluating the screening programme for FH), our additional questions still related closely to the content and had developed from the assumptions of the programme. In the second case (implementing respiratory care guidelines in Nepal), we used the opportunities that arose to study context and processes more explicitly. In the last case (the development of the performance indicator framework), we tried to address the content as well as the context and processes right from the start. In retrospect, the model by Walt and Gilson was helpful in analysing the issues related to specific health services research content. Using it to design and evaluate research proposals will be a next logical step.

Secondly, this thesis presents only three cases of health services research at work for national health policy and all cases took place in different policy contexts. Although these aspects may be regarded as shortcomings, the cases are not exceptional in the portfolio of health services research of the involved researchers. In addition, typical for the multidisciplinary character of health services research, we used a mix of methodological approaches in the three cases. We used an epidemiological approach in evaluating the screening programme, a qualitative approach in the case of respiratory care guidelines in Nepal and a more sociological approach in analysing the development process in the last case (regarding the development of the performance indicator framework).

Thirdly, we did not set up additional research to measure the usefulness of the results other than discussing the usefulness and utilization with those researchers who were directly involved. For example, if we would have conducted interviews with multiple actors in each of the three cases, we probably would have a more detailed picture of the usefulness and utilization of the work described in this thesis.

Fourthly, the increasingly interactive research process may have led the researchers away from independent and critical reflection because they became part of the implementation
processes described in Cases II and III. We believe that it is indeed a challenge to maintain scientific integrity when the interactive process reflects pressure towards expected outcomes or because of time frames relevant to other actors. However, these are challenges that can be overcome. In this respect, we do not consider the publications of Chapter 2 [21] and Chapter 6 [18] to be defeats because we had to formulate the conclusions more diplomatically than we might have done otherwise, but as successes of collaborative research that increased the usefulness of the content because of the involvement of a wider network of actors.

Finally, the different intensities in which we interacted with the policymakers and contractors in the different cases coincided with the level of what one might call ownership of the subject of the study. This ownership by the policymakers increased in chronological order. In Case I there was no ownership (or involvement in the development) by the MoH in the screening programme, in Case II the WHO had already developed the PAL package and in Case III there was a direct interest and involvement by the MoH in developing the framework. Furthermore, in Case III the context and process of our framework (the content) was the same as the policy context and process in which the framework has to be implemented. Interaction and ownership seem closely related. ‘Not to interact’ is not an option for policymakers if they are contractor, co-developer and end-user all at the same time. These observations complicate our retrospective analysis of the two strategies we focused upon, i.e. additional research questions and interaction processes. Comparative studies in similar contexts (including policy contexts) in which only one of the two strategies is studied can shed more light on the impact of either of the two.

**Implications**

**Implications for science**

The model by Walt and Gilson [20] offers a framework for policy analysis that we applied to health services research. We used it retrospectively to present health services research at work for national health policy. We have discussed the usefulness of the additional research questions based on general retrospective discussions of events and developments. Based on our experiences with this application of the model, we conclude it has potential for use in planning and designing health services research. Using it as a design format, it can help to translate health policy questions into researchable and policy-relevant health services research questions that address not only content but also context and process of a specific health services content. Further research is necessary to develop this model as a practical tool for research design and evaluation. For example, this could start with the development of a checklist for discussions with policymakers about translating the policy question into research questions. In future, context- and process-focused research questions should no longer be ‘additional’ but should be an integral part of any proposal for health services research for national health policy.
The initial research proposals in the three presented cases included a managerial evaluation, an economic as well as a developmental evaluation [22] and an instrumental research project [23] respectively. What the ‘best interaction model’ is may depend on the type of information needed. Therefore, in future studies we need to differentiate between the types of research when we address interaction processes. Also, in all three cases formal settings for interaction were organized. Further study is needed on how to optimize different formal interaction models to support specific research and policy demands.

Our three cases have shown that a combination of content-, context- and process-focused research questions (in addition to the initial research question) have most likely increased the usefulness of the research results from the initial content question. However, we need to design studies that measure the impact of this approach on usefulness and utilization of produced knowledge in a more systematic and structured way than we could in this thesis. Given the diversity of studies conducted in health services research, it may take some time and effort to collect a substantial amount of evidence that allows us to be more conclusive on this topic. To start with, researchers should develop ways to systematically monitor the use of their study results. This ‘post-marketing’ is common practice for commercial enterprises as well as in health care, where it is used to monitor adverse drug effects [24]. Health services research can benefit from such strategies and adjust them to its own needs. The ability to follow the ‘career path’ of research results once a research project has finished is valuable not only for the personal learning ‘career’ of individual researchers but also for health services research in general.

**Implications for policy**

We were able to address several additional research questions at a low cost because of the involvement of students. Because these content-, context- and process-focused questions should be an integral part of any proposal for health services research for national health policy, addressing them should not be fully dependent on the opportunities that the research setting may or may not provide. The finances necessary to answer them need to be included in the research budgets allocated by policymakers. In addition to the budgets allocated for commissioned research, health services research needs financial resources for academic exploration and participation in the scientific debate in international journals.

These cases show that the involved health services researchers at work for national health policy are keen to understand policy, policy context and policymaking. They also aim for their research results to be used in the policymaking process. The involved researchers are certainly willing to bridge the gap between the two communities of research and policy [25]. Policymakers can utilize this opportunity by facilitating interactive processes with researchers in which they jointly develop relevant and researchable questions that suite the policy information demands and produce results that have great potential for usefulness. In the Netherlands, Saan and De Haes [26] have stressed the need for more interaction and communication between research, practice and policy to create effective health promotion. Our cases contribute to the empirical evidence of interactive research-for-policy processes. From
these cases, policymakers can learn what interactive involvement can look like and how this contributes to producing useful knowledge. Experiences from others confirm this [27]. The activities developed at the MoH are important steps in the right direction. If successful, the push for a ministry-wide strategic knowledge agenda based on priority areas for information and knowledge can help policymakers to effectively use knowledge, develop research questions and evaluate utilization of research in a consistent and coherent fashion. Training policymakers on knowledge and information management during summer courses contributes to the personal competencies of individuals in dealing with information, knowledge and research for their policy processes [28;29]. An important challenge is to reach policymakers who are thinking about commissioning research, either directly or through subsidizing agencies such as ZonMw [30]. However, if successful, policymakers will be able to participate more actively – and interactively – in the knowledge-production process. Consequently, policy will be better informed with relevant and useful study results. As was our experience in Case III, policymakers with an affinity for research can strengthen the interaction process and contribute effectively to producing useful knowledge for the policy process, and in doing so, successfully bridge the gap between research and policy.

Policymakers and researchers need to discuss the policy information problem in order to define the right research questions. In Case II, for example, if we would have identified relevant context-, process- and actor-focused questions prior to starting the project, we might have chosen a different approach for the evaluation study. Rather than focusing on costs and effects, the study might have addressed health system performance for respiratory care in general. It calls for an open and creative mind – and some courage as well – to diverge from a path already chosen.

Implications for practice

**Implications for the training of health services researchers**

The cases in this thesis provide a glimpse of actual interactions between researchers and others. It showed that interaction is important but also that it is not always easy. Researchers must learn how to communicate and interact with partners who may introduce conflicting agendas. To guide researchers in their interaction with others, codes of conduct have been developed not only for health research in general [31] but also for health services research in particular [32]. These codes of conduct need to be given more attention by researchers and the development of a Dutch code of conduct for health services research deserves consideration. In addition to codes of conduct, health services research must be organized and financed in a way that optimizes the independence of the researchers. Furthermore, researchers sometimes perceive a trade-off between scientific independence and interaction in research, which compromises the objective of 'doing the right things, and doing the right things right'. Therefore, interacting with policymakers and other actors deserves attention when training researchers. Identifying communication and interaction with other actors as an integrated part of research in practice and consequently defining this as a learning objective can contribute to developing such skills for all researchers. These skills are of particular
importance at the start and at the end of a project: at the start, because policy information needs translation into relevant and researchable questions; at the end, because results need to be translated into solutions and follow-up actions.

Health services researchers need to have knowledge of the health system. This is particularly relevant when questions about context, process and actors need to be identified and discussed. Often, how the system functions or perceptions about it are not known in detail (Chapters 3 and 7) and additional research is needed.

Researchers can benefit from a long-term perspective. Junior researchers, especially those on PhD projects in the Netherlands (which typically last four years), will rarely see their results being put into practice, as most policy processes can only utilize the results long after the end of the study period. Developing a policy focus and understanding the world of policy-making will help researchers survive in a setting where systems reform, institutions change and familiar actors disappear as they redesign their career paths. All these factors cause delays. These cases showed that the national health policy processes are very dynamic, like the health services research that is at work for them. Although this challenges health services researchers when conditions and plans change, it should not distract them from enjoying the dynamics of applied research.

**Implications for the organization of health services research projects**

Health services research, which by definition has a multidisciplinary character, challenges not only the interaction between researchers and stakeholders, but also between researchers themselves. Focuses of collaborative research may need thorough discussion and negotiation before reaching an agreement when researchers come from different scientific backgrounds and theoretical schools. This also applies to the formulation of research questions that may reflect different scientific perspectives. In Case II, we could agree on the addition of a process-oriented research perspective at the beginning of the study development.

Identifying a combination of content-, context- and process-focused research questions needs to be done early in the research process. Discussing and developing these questions right at the start of a project with the policymakers and other actors is a way to increase the usefulness of the study results. This early-on agenda-setting is particularly relevant in settings where resources cannot be mobilized easily at a later stage.

As shown in Case I and Case II, evaluations can affect others in many ways. Health services researchers need to be aware of the sensitive nature of their work and address sensitivities in an appropriate way. To be clear and explicit about the terms of reference and the framework for the evaluation helps to facilitate the evaluation, even in circumstances that are not favourable to other stakeholders, especially the subject involved.

**Implications for the health services research process**

Users and other stakeholders can be expected to accept results from health services research if they can agree with the chosen approach. This became particularly relevant in Case I. If
we, the researchers, could have agreed to explore different approaches of cost-effectiveness analysis together with other stakeholders in a collaborative study, the debate would probably not have focused on technical issues.

Although it is important for health services researchers to publish their work in scientific journals, policymakers are likely to use different media to receive the context-specific information they need for the policymaking process. Health services researchers should therefore also publish in those journals more easily accessible to (and read by) policymakers. In Case 1, for example, the publication of the effects of participation in the screening programme on insurance in a Dutch journal for medical doctors [33] fuelled an important policy debate that may not have been picked up (at that time) if it would only have been published in an international scientific journal [34].

Formulating research products in the current terminology or the preferred language of the intended user can help health services researchers increase the usefulness of their research. For example, in Case III, in subsequent developments by the RIVM and the MoH the balanced scorecard (reflecting the language of the original concept and thus referring to corporate businesses) changed into a model with three ‘public interests’ (reflecting the current political language referring to the role of the government). With this terminology, the model is useful for the political and policy purposes of the intended user. Thus, researchers need to become sensitive to turning technical language into policy language.

**Conclusion**

In conclusion, the strength of this thesis can be found in the representation of how health services research at work for national health policy actually works in practice. The chosen framework directed our analysis perspective to areas that are not always identified in the initial research proposal: context, process and actors. Our experiences demonstrate the added value of formulating content-, context- and process-focused research questions and the importance of the interaction process with policymakers. Both strategies not only make health services research for national health policy workable, they enhance it as well.

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Summary
Introduction

This thesis addresses the issue of how health services research can produce useful knowledge for policymakers. Policymakers need information for their work processes and this information can consist of results from health services research. Other researchers have studied the determinants for policymakers’ use of scientific knowledge. Two of the determinants we focus on in this thesis are the quality of research and the interaction between policymakers and researchers. High-quality research and dynamic interaction between policymakers and researchers bridge the gap between them and increase the possibilities that policymakers will use the research results. But how can we facilitate such a process?

In this thesis, we present three cases of health services research at work for national health policy to provide the empirical basis for addressing the issue of how health services research can produce useful knowledge for policymakers. The cases concern the evaluation of a genetic screening programme for familial hypercholesterolaemia, the implementation of respiratory care guidelines in Nepal and lastly, the development of a measurement tool for health system performance.

The primary focus of scientific researchers is producing generalizable knowledge, not how to answer the here-and-now, context-specific questions policymakers may have. To produce useful knowledge for these policymakers it can be helpful to understand policy and policymaking processes. The policy analysis model of Walt and Gilson provides a conceptual framework for this. They identify content, context and process as inseparable dimensions of policy. Central to this model are actors who are part of each of the dimensions. Health services research can be considered to be the producer of knowledge used by the actors. This model helps health services researchers to better understand policy and policymaking processes. Likewise, policymakers will be better informed if they understand specific health services content – for example, the cost-effectiveness of an intervention or the design of a new instrument – within its context and processes and with regard to its actors.

In this thesis, we argue that the usefulness of research results can be increased not only by answering the generalizable question of ‘What works?’ but by answering the additional questions of how, why, where, when and for whom? These additional questions can be formulated in a combination of content-, context- and process-focused questions, with actors playing a central role in each of them. In addition, we address the interaction between researchers and policymakers. We retrospectively explore two questions to increase the usefulness of health services research for national health policy. Firstly, how can health services research include content-, context- and process-focused research questions (in addition to the initial research question), and secondly, how can health services researchers participate in an interaction process with policymakers?

For the three cases, we present the results of a combination of content-, context- and process-focused research questions. At the end of each case, in a retrospective chapter entitled ‘Health services research at work in Case …’, we address three aspects of the case. Firstly, we describe the position of the researchers in relation to other actors and to the inclusion of
the combination of content-, context- and process-focused questions (in addition to the initial research question). Secondly, we describe the interaction of the researchers with policymakers and lastly, we describe the follow-up of events and developments in each case. The thesis concludes with a discussion in Chapter 13, which reflects on the lessons learned for health services research by answering the central questions in this thesis: How can health services research include content-, context- and process-focused research questions (in addition to the initial research question) and how can health services researchers participate in an interaction process with policymakers?

Case I. A national screening programme for familial hypercholesterolaemia?
The first case in this thesis concerns the genetic screening programme for familial hypercholesterolaemia in the Netherlands (Chapters 2 – 5). Familial hypercholesterolaemia (FH) is an autosomal dominant disorder and those who suffer from it are predisposed to coronary artery disease (CAD) and premature cardiac death. The discovery of low density lipoprotein (LDL) receptor gene mutations in clinically diagnosed FH patients and the subsequent development of DNA tests to detect these mutations make it possible to diagnose FH patients before a first CAD event. This is particularly relevant for relatives of confirmed FH patients, who are at much higher risk of having an LDL receptor gene mutation than the general population. Therefore, a family-based genetic screening programme for FH was implemented by the Foundation for the Identification of Persons with Inherited Hypercholesterolaemia (StOEH), with financial support from the Dutch Ministry of Health, Welfare and Sport (MoH).

An evaluation study was initiated by the StOEH and the Department of Social Medicine at the Academic Medical Center (AMC) in Amsterdam. The decision on structural, nationwide implementation and long-term funding by the MoH depended in part on the results of the evaluation study. The main research questions in the evaluation concerned the psychosocial consequences of participation in the screening programme, the reliability of the genetic test and the cost-effectiveness of the programme. The case in this thesis focuses on research related to the question of costs and effects.

In Chapter 2 we present the study on the prevalence of hypercholesterolaemia among screened individuals with LDL-receptor gene mutations. The assumption at the start of the programme was that these mutations were nearly 100% penetrant. In this study we found an 80.2% prevalence of hypercholesterolaemia in genetically diagnosed men and 83.3% in women. These results are important for analysing the programme’s cost-effectiveness, as it raises the question of whether screened persons with an LDL receptor gene mutation but with no hypercholesterolaemia can benefit from participation. In addition, this study contributes to the knowledge about FH and to a further description of the target population, which mainly consists of asymptomatic carriers of an LDL-receptor gene mutation.

Health benefits for participants in the screening programme depend on treatment of hypercholesterolaemia. To evaluate costs and effects, it is important not only to know how many FH patients are diagnosed, but especially how many of them had not yet been diagnosed with hypercholesterolaemia. To estimate the proportion of patients with FH who had been
identified with hypercholesterolaemia in general practice prior to screening, we analysed the patients’ medical record forms. This study shows that those with no recorded history of cholesterol measurements or hypercholesterolaemia are on average 22 years younger than those with no such information in their records. This means that particularly younger patients are brought to the attention of their GPs. This allows for proper, early referral of these patients and increases their chances of preventing CAD. This study confirms the potential added value of genetic screening for individual FH patients over passive identification by GPs.

In Chapter 4 we address the cost-effectiveness of the programme. This study combined the screening results of 2229 participants with the Framingham risk function for cardiovascular diseases and with national, disease-specific costs of illness. Based on modelled outcomes, a comparison was made between survival and costs with and without a screening programme. The costs per gained life year vary between 25,500 and 32,000 euros, depending on the treatment scenario. The costs of diagnosis are many times lower than those of treatment, due to the costs of drugs (statins). We conclude that the cost-effectiveness ratio of the screening programme demands an explicit political decision. Furthermore, clear guidelines should be developed for treating persons with genetically diagnosed FH, because not all of them have hypercholesterolaemia.

Chapter 5 presents information on including other research questions, the interaction with other actors and the follow-up of events in this case. We show that the research questions in Chapters 2 and 3 were answered using only limited resources, in part by formulating the question within the framework of the original research proposal and budget, and by involving students as research interns. The interaction between researchers and policymakers was limited to presenting and discussing the study results. As researchers, most of our contacts were with the supervisory scientific group and with researchers and staff at the StOEH. The decision by the MoH to include the evaluation study results in the decision-making process has stressed the importance of this interaction.

Based on multiple administrative and managerial developments, we arrived at the conclusion that in 2006 it is no longer a question of whether or not a screening programme for FH is advisable. Instead, the discussion is focused on the conditions for its implementation. Our health services research has contributed to these developments and discussions by producing relevant scientific knowledge and by interacting and having open discussions with policymakers. Several results were used in the policy process that resulted in the minister of health’s decision to continue and expand the screening programme, to carry out further evaluations and to promote further research.

Case II. Nationwide implementation of respiratory care guidelines?

The second case in this thesis concerns the implementation of respiratory care guidelines in Nepal. The WHO is developing the Practical Approach to Lung Health (PAL) initiative for this specific topic. With a set of clinical guidelines, training materials and implementation strategies, PAL aims to improve the quality of care provided by primary care health workers in middle- and low-income countries for children (over 5 years of age), youths and adults.
with respiratory symptoms. An evaluation of costs and effects of a pilot-implementation of PAL was carried out in Nepal. The results of this health services research contributes to the further global development of PAL as well as to the decision-making process regarding nationwide implementation of PAL in Nepal. In addition to the initial research question about costs and effects, we formulated several content-, context- and process-oriented research questions. Chapters 6, 7 and 8 each address one of these questions. Chapter 9 provides additional information and reflections on this case.

Firstly, in discussions within the collaborative research group we observed that the study proposal did not explicitly address the content of PAL or the context in which PAL was going to be implemented in Nepal. We hypothesized that with such information, the outcome of the cost-effectiveness assessment could be better understood and could potentially guide policy changes. In Chapter 6 we describe how we studied (ex ante) whether successful implementation of PAL can be expected, given the quality of the innovation, the effectiveness of the implementation strategy and the social system in which PAL needs to be implemented. This study shows that in 2002 the implementability of PAL was challenged on all aspects studied. The chances of effective implementation can be increased by improving the guideline quality and by strengthening the implementation strategy. In order to successfully transfer global knowledge into local practice, we need to develop additional multifactorial sustained interventions that tackle other culture- and health system-specific barriers as well.

Chapter 7 describes the health care context of PAL from the perspective of the patient. To better understand how patients with respiratory symptoms experience the health care system, we interviewed patients being treated for tuberculosis. We explored which route they had been following through the health care system before they were diagnosed and treated for tuberculosis. These routes often started in the ‘medical shop’ (drug retail shop, mostly run by a health worker) and, by way of intricate routes with multiple providers, led to facilities with more highly qualified and competent staff where tuberculosis was diagnosed. Several health system factors influenced these routes, such as the anticipated service level and the quality and costs of care. Furthermore, this study showed that patients were not referred to a higher-level facility if treatment failed but changed providers themselves based on self-referral. Interestingly, once a provider considered a diagnosis of tuberculosis, the referral to a specialized laboratory or treatment centre promptly followed. Often the provider explained that treatment for tuberculosis was free of charge and was probably available within a short distance from home. We conclude that the route taken by patients could likely be shortened if care providers refer patients more effectively and if the service level is increased. The results of this study are relevant for improving passive case-finding strategies for tuberculosis.

One might assume that those involved in the PAL initiative in Nepal will give PAL priority over other interventions. However, it is unclear whether others agree with this prioritization of PAL. In Chapter 8 we describe a study of the relative importance of different health care programmes. We conducted a discrete choice experiment with 66 policymakers and health professionals involved in mid-level health care management and public health provision. The
results showed that health care interventions are prioritized that target a) severe diseases, b) many beneficiaries and c) middle-aged people, d) have significant individual health benefits, e) lead to poverty reduction and f) are very cost-effective. If we apply this prioritization to a large set of interventions, we can conclude that compared to other interventions, PAL in Nepal is also considered to be important by those who are not directly involved. This multi-criteria approach can be an important step forward to a rational approach to priority-setting in developing countries.

In Chapter 9 we describe how we were able to conduct the different studies presented in this case. The study of the implementability of PAL was set as a precondition for the involvement of the author of this thesis in the study of costs and effects. The study of the patient routes through the health care system was conducted by a Dutch student and a research assistant, which kept the use of limited resources in check. It was possible to explore the relative priority of PAL during a workshop initiated by the WHO and the National Tuberculosis Centre (NTC) in Kathmandu. In this workshop we presented the preliminary study results of the PAL evaluation and discussed the future of PAL in Nepal.

The health services research in this case was characterized by the active collaboration between evaluation researchers and PAL designers and implementers. This facilitated a rapid response to some of the findings in the evaluation study. Furthermore, this collaboration resulted in the joint publication of the study of implementability of PAL. This has increased the support for improvements such as structural supervision. In addition, a steering committee made up of Nepalese policymakers, representatives of research institutes and non-governmental organizations provided relevant context-specific information and critical reflection.

Although the development of PAL has made considerable progress worldwide, in Nepal no activities to expand PAL’s implementation have been developed since the pilot implementation. Obstructing factors include the lack of collective funds to invest in PAL. Also, the replacement of a charismatic director at the NTC (and who was a major supporter of PAL) had an adverse effect on further development of PAL in Nepal. However, the study results have contributed to the discussion at the NTC on involving the private health care sector in activities to improve the quality of care in general, and passive case-finding for tuberculosis in particular.

Case III. An instrument for national health system performance measurement?
The third and final case in this thesis concerns health services research for developing a performance indicator framework for the Dutch health system (Chapters 10-12). The driving forces behind the MoH’s initiative to develop such a framework were the developments regarding international comparisons of health system performance conducted by the WHO and the Organization for Economic Cooperation and Development (OECD) among others, and the development of a major health system reform in the Netherlands. We provided research assistance in this development process, and as a result we were in a position to design a completely new instrument to be used within an organizational environment that is characterized by dynamic policy contexts and processes. We hypothesized that whether or
not this new tool would actually be used would depend largely on the context and processes that coexisted. Therefore, we formulated additional research questions regarding policy contexts and processes and these are found in Chapter 11. Additional information about this case is presented in Chapter 12.

In Chapter 10, we describe the development of the conceptual framework for performance indicators for the Dutch health system. This framework is based on a balanced scorecard with four managerial perspectives on health care: patient/client orientation, financial aspects of care, the provision of high-quality care, and the system’s ability to learn and innovate. Subsequently, we linked this model to Lalonde’s model of determinants of health. This resulted in a model that shows the relationship between health and health care. Furthermore, this model facilitates the presentation of performance information from different perspectives. It reflects the strategic need of the health system to contribute to the attainment of health by providing high-quality care that is accessible to all citizens. Simultaneously, the framework responds to the sector-specific information needs of policymakers. The selected indicator areas reflect the policy and management function of the government and the defined public goals of the health system. The MoH officially endorsed this framework in February 2003. Subsequently, representatives of many departments at the MoH developed indicators for the 26 indicator areas.

The context and policy processes of the development of the framework are the central theme in Chapter 11. This development took place within the dynamic context of a ministry where several other policy processes evolved simultaneously. These included a reduction of the administrative burden and the renewed financial accountability and reporting system. Some of these processes strengthened the development of the framework, while others obstructed this. At international level, participation by the Netherlands in similar development processes within the OECD and the European Union had a strong and positive influence on the national development process. This not only stresses the importance of performance measurement but also shows the importance of creating opportunities to align the content of these processes with others. The intensive and interactive cooperation and the multiple feedback opportunities with many stakeholders, both from the MoH and from ‘knowledge institutions’ such as the National Institute for Public Health and the Environment (RIVM), the Netherlands Institute for Health Services Research (NIVEL) and Prismant contributed to the evolution of strong support for the performance indicator framework. Using an active communication strategy, the MoH informed other parties such as health care insurers, providers and patient representatives about the framework. After completing the conceptual framework, the RIVM was commissioned with the task of making the framework operational. The framework formed the basis for the new health system’s accountability document, the \textit{Balance of Care}.

Several developments have taken place since our research involvement in this case. A number of adjustments were made to the conceptual framework (as presented in Chapters 10 and 11) in the implementation phase. Firstly, the explicit linkage between the balanced scorecard and public health information was abandoned, and instead of the initial four managerial perspectives to health care, three public goals (quality, affordability and accessibility) now characterize
the categories of performance indicators. The first Balance of Care document was published in May 2006. It provides information on 20 indicator areas, consisting of 125 indicators. Secondly, the development of specific indicators for patient and consumer experiences with health care has led to the introduction of new measurement tools based on the American CAHPS® and Dutch Quote questionnaires. This new family of instruments has now been adopted as the standard for measuring patient experiences in the Netherlands. A national institute will be founded to coordinate the research and implementation of these questionnaires. Thirdly, the research carried out in the Netherlands regarding system performance measurement has influenced the developments at the OECD, which adopted the altered framework for comparing health system performance of the participating member states. We conclude that our work contributed directly to the development of health system performance, both nationally and internationally. Furthermore, the context and process explorations showed that the ultimate result was accomplished because and in spite of complex and dynamic political policy processes.

Discussion

In the discussion in Chapter 13, the last chapter of this thesis, the main findings are listed for the two research questions: ‘How can health services research include content-, context- and process-focused research questions (in addition to the initial research question)?’ and ‘How can health services researchers participate in an interaction process with policymakers?’ The three cases presented (genetic screening for familial hypercholesterolaemia, implementation of respiratory care guidelines and a framework for performance indicators) show that the possibilities for carrying out research not initially included in the proposals depended largely on additional available resources such as finances and staff. By involving students, several studies could be conducted with no prior funding. In non-academic institutions, such solutions are probably less feasible. In our studies we found stakeholders were enthusiastic about additional research if they could expect to benefit from the results and if it involved no additional costs.

Regarding the interaction between health services researchers and policymakers, we conclude that interaction increased consecutively. In the first case, the policymakers were only involved at the end of the study period because they were mainly interested in the results they needed for their policy decisions. In the second case there was much more interaction, both formally and informally, and many discussions took place between the health services researchers and the developers and implementers of PAL. In the third case, the choice for an intensive and interactive research process was made beforehand. The MoH was the contractor, funding agency, co-developer and intended user. The differences in interaction can be explained in part by the differences in the information needs of the policymakers. However, there is also room for explicitly choosing for more or less interaction.
The usefulness of the research results of the three cases is described from two perspectives. Firstly, from the perspective of the process of use, whereby the interaction between researchers and policymakers is one of the factors, and secondly, based on the actual use of the research results. The reflections on the use of study results suggest that the results of the combination of content-, context- and process-focused questions have strengthened the usefulness of the initial research.

The discussion continues by addressing methodological considerations. Firstly, the policy analysis model was applied retrospectively to health service research at work. As a result, the presented cases are not evenly focused with regard to content, context, process and actors. A logical next step would be to use the model as a reference framework for developing new research proposals and projects.

Secondly, the three cases had very different policy contexts. However, the cases presented here are not exceptional in the portfolio of health services researchers and the heterogeneity can be considered to be representative.

Thirdly, the exploration of the usefulness of the study results was based on our own observations. In follow-up research, measuring the actual use of research results needs more attention.

Fourthly, a possible drawback of the interaction process in the second and third case could have been that the researchers were limited in their academic freedom because they were involved in the development and implementation process. However, we do not feel this played a role in our studies.

The implications for science, policy and the practice of health services research are discussed in the last section of this chapter. Implications for science include further research of applications of Walt and Gilson’s policy model during the design, planning and evaluation of health services research. In the near future, content-, context- and process-focused research questions need to be integrated into health services research and should no longer be considered to be ‘additional’ questions. Also, the question ‘What is the best interaction model?’ deserves more attention. The answer depends in part on the information needs of the policymakers.

Finally, it is advisable for health services researchers to monitor the actual use of their study results (post-marketing) to be able to contribute to the empirical knowledge on the use of scientific results in policy. Implications for policy focus mainly on the importance of open communication between policymakers and researchers to make it possible to identify and formulate the right research questions early on in the collaboration. Implications for practice of health services research focus on training researchers, organizing research projects and the process of conducting research. In training health services researchers, the topic of collaboration with multiple stakeholders (and interests) needs more attention, as does knowledge of health systems and health policy processes. In organizing and planning research projects, there needs to be sufficient space for discussing the scientific perspective, preferably early on in the research process. This applies not only to the communication between researchers and other stakeholders but also among the researchers themselves. The implications for the process of research focus on the communication with other parties. This is of specific impor-
tance regarding the overall research approach, using the correct terminology and publishing study results relevant for policymakers.

In conclusion, the strength of this thesis can be found in its representation of how health services research at work for national health policy actually works in practice. Our experiences demonstrate the added value of formulating content-, context- and process-focused research questions and the importance of the interaction process with policymakers. Both strategies not only make health services research for national health policy workable, they enhance it as well.
Samenvatting
Inleiding

In dit proefschrift worden drie casus gepresenteerd van gezondheidszorgonderzoek dat is uitgevoerd ten behoeve van nationaal gezondheidsbeleid. Op basis van deze casus wordt ingegaan op de vraag hoe gezondheidszorgonderzoek bruikbare kennis kan produceren voor de makers van nationaal gezondheidsbeleid. De voorbeelden betreffen achtereenvolgens een evaluatie van een genetisch opsporingsprogramma voor familiaire hypercholesterolemie, een evaluatie van de ontwikkeling en invoering van klinische richtlijnen voor de behandeling van longziekten en, tenslotte, de ontwikkeling van een meetinstrument voor de prestatie van een gezondheidssysteem.

Beleidsmakers hebben informatie nodig voor hun werkproces. Soms bestaat die informatie uit resultaten van gezondheidszorgonderzoek. Er is al veel onderzoek gedaan naar beïnvloedende factoren voor het gebruik van wetenschappelijke kennis door beleidsmakers. De kwaliteit van het onderzoek en de interactie tussen beleidsmakers en onderzoekers zijn twee van zulke factoren en staan in dit proefschrift centraal. Kwalitatief goed onderzoek en actieve interactie tussen beleidsmakers en onderzoekers verkleinen de kloof tussen beleidsmakers en onderzoekers en vergroten de kans op onderzoeksresultaten die bruikbaar zijn voor beleids-makers. Maar hoe geef je dat proces vorm?

Wetenschappelijk onderzoekers zijn primair gericht op het produceren van generaliseerbare kennis, en niet op het beantwoorden van de hier-en-nu-vragen van beleidsmakers. Om in die context van beleidsmakers bruikbare kennis te kunnen produceren, kan het voor onderzoekers behulpzaam zijn om kennis te hebben van beleid en beleidsprocessen. Het beleidsanalyse-model van Walt en Gilson biedt daarvoor een conceptueel kader. Zij onderscheiden inhoud, context en proces als drie dimensies van beleid die in een driehoeksverhouding onlosmakelijk met elkaar verbonden zijn. Centraal in deze driehoek stellen zij de actoren die elk weer onderdeel zijn van, en invulling geven aan, inhoud, context en proces. Gezondheidszorgonderzoek heeft een plek in dit model wanneer we het beschouwen als de producent van kennis die gebruikt wordt door de actoren. Dit model kan gezondheidszorgonderzoekers helpen om meer inzicht te krijgen in beleid en beleidsprocessen. Analoog hieraan kunnen beleidsmakers beter worden geïnformeerd wanneer ze een specifiek en inhoudelijk aspect van gezondheidszorg (zoals de kosteneffectiviteit van een programma, of het ontwerp van een nieuw meetinstrument) kunnen begrijpen in samenhang met de context, het proces en de betrokken actoren. In dit proefschrift wordt betoogd dat de bruikbaarheid van het onderzoek kan worden verhoogd wanneer niet alleen de vraag “Wat werkt?” wordt beantwoord, maar ook “Wat werkt, hoe, waarom, waar, wanneer en voor wie?”. Deze vragen kunnen worden geoperationaliseerd door een combinatie van inhoud–, context– en procesgeoriënteerde onderzoeksvragen te formuleren waarbij actoren steeds een centrale rol innemen. Daarnaast gaan we in op de interactie tussen de onderzoekers en beleidsmakers. In dit proefschrift worden twee vragen retrospectief geëxploereerd. Ten eerste, hoe kunnen bij gezondheidszorgonderzoek inhoud–, context– en procesgeoriënteerde vragen worden geïncludeerd in
aanvulling op de initiële onderzoeksvraag, en ten tweede, hoe kunnen gezondheidszorgonderzoekers deelnemen aan een interactieproces met beleidsmakers?

In drie casus worden resultaten gepresenteerd van een combinatie van inhoud-, context- en procesgeoriënteerde onderzoeksvragen. Per casus wordt een toelichting gegeven waarbij de inclusie van aanvullende onderzoeksvragen en de interactie met beleidsmakers en andere betrokkenen (bijvoorbeeld opdrachtgevers, uitvoerenden van zorg en begeleidingsgroepen) centraal staan. Bovendien wordt er voor elke casus beschreven welke ontwikkelingen er zijn geweest sinds het hier beschreven onderzoek was afgegrond en wordt ingegaan op het gebruik van de onderzoeksresultaten. In de discussie (hoofdstuk 13) tenslotte wordt op basis van de resultaten uit de drie casus een antwoord gegeven op de twee onderzoeksvragen uit de inleiding van het proefschrift.

**Casus I. Landelijke opsporing van familiare hypercholesterolemie?**

De eerste casus in dit proefschrift betreft het opsporingsprogramma voor familiare hypercholesterolemie in Nederland (hoofdstuk 2 t/m 5). Familiare hypercholesterolemie (FH) is een dominante, niet geslachtgebonden erfelijke aandoening die wordt gekarakteriseerd door een extreem hoog cholesterolgehalte en het voorkomen van coronaire hartziekten (CHZ) op jonge leeftijd bij de patiënt en/of zijn familie. De diagnose FH wordt vaak pas na het optreden van CHZ gesteld. Echter, met de ontdekking van mutaties in het low-density lipoproteïne (LDL) receptor gen, die de oorzaak van FH vormen, en het beschikbaar komen van genetische diagnostiek, kunnen dergelijke mutaties worden aangetoond voordat de eerste klinische verschijnselen optreden. Vooral voor familieleden van mensen met FH is dit relevant aangezien zij, vanwege het erfelijke karakter, eveneens een sterk verhoogde kans hebben om FH en dus CHZ te ontwikkelen. Daarom startte in 1994 met financiële steun van het ministerie van Volksgezondheid, Welzijn en Sport (VWS) de experimentele fase van een opsporingsprogramma op basis van familieonderzoek en DNA-analyse, uitgevoerd door de Stichting Opsporing Erfelijke Hypercholesterolemie (StOEH). In samenwerking tussen de StOEH en afdeling Sociale Geneeskunde van het AMC ontstond het initiatief voor een evaluatieonderzoek. Het onderzoek werd gefinancierd door ZonMw (toen nog Praeventiefonds geheten). De resultaten van deze evaluatie werden door VWS betrokken bij de beslissing over continuering van financiering van het programma. De hoofdvragen uit de evaluatie richtten zich op de psychosociale gevolgen van deelname aan het opsporingsprogramma, de betrouwbaarheid van de genetische test en de kosteneffectiviteit van het de opsporingsprogramma. In dit proefschrift wordt vooral ingegaan op het deelonderzoek naar de kosteneffectiviteit.

In hoofdstuk 2 wordt onderzoek gepresenteerd naar de prevalentie van hypercholesterolemie onder opgespoorden met een mutatie van het LDL-receptor gen. De aanname ten tijde van de start van het opsporingsprogramma was dat LDL-receptor gen mutaties bijna 100% penetrant waren. Uit dit onderzoek blijkt echter dat op het moment van de opsporing 80.2% van de mannen en 83.3% van de vrouwen met een DNA-diagnose FH ook daadwerkelijk hypercholesterolemie hadden. Dit onderzoek is belangrijk voor de analyse van de kosteneffectiviteit...
van de opsporing omdat het de vraag oproept of opgespoord met FH, maar zonder hypercholesterolemie, toch gezondheidswinst kunnen boeken. Daarnaast levert deze studie een bijdrage aan de kennis over FH en aan een verdere beschrijving van de doelpopulatie die bestaat uit veelal asymptomatische dragers van een mutatie van het LDL-receptor gen. Deelnemers aan het opsporingsprogramma kunnen gezondheidswinst boeken door behandeling van hypercholesterolemie. Voor de evaluatie van kosten en effecten is het niet alleen belangrijk te weten hoeveel FH patiënten worden opgespoord maar vooral welk deel nog niet bekend was met hypercholesterolemie. Daarom hebben we bij een steekproef van FH patiënten statusonderzoek verricht in de huisartsenpraktijk. Deze studie, gepresenteerd in hoofdstuk 3, laat zien dat de FH patiënten bij wie voorafgaand aan de opsporing geen cholesterolmeting werd vermeld in de huisartsenstatus (61%), gemiddeld 22 jaar jonger zijn dan de patiënten bij wie al wel een meting werd vermeld. Dit betekent dat van alle patiënten die door het screeningsprogramma worden opgespoord, met name jonge FH patiënten voor het eerst onder de aandacht van de huisarts worden gebracht. Hierdoor kunnen deze patiënten al in een vroeg stadium verwezen en behandeld worden en kunnen coronaire hartziekten mogelijk worden voorkomen. Dit onderstrept de potentiële meerwaarde voor de individuele FH patiënt van genetische screening boven passieve opsporing door de huisarts.

In hoofdstuk 4 behandelen we de kosteneffectiviteit van het opsporingsprogramma. In deze studie zijn de gegevens van 2229 deelnemers aan het opsporingsprogramma gecombineerd met de Framingham risicofunctie voor hart- en vaatziekten en met nationale, ziektespecifieke kostengegevens. Hierdoor kon op basis van gemodeleerde uitkomsten een vergelijking worden gemaakt tussen overleving en kosten met en zonder opsporingsprogramma. De kosten per gewonnen levensjaar variëren tussen de 25.500 en 32.000 euro, afhankelijk van het behandelingsscenario. De kosten van de opsporing zijn vele malen lager dan die van de behandeling, vooral door de kosten van geneesmiddelen (statines). Geconcludeerd wordt dat de kosteneffectiviteitratio van de opsporing expliciete politieke besluitvorming over het programma nodig maakt. Bovendien moeten duidelijke richtlijnen moeten worden ontwikkeld voor de behandeling van personen met een positieve DNA diagnose voor FH aangezien niet allen hypercholesterolemie hebben.

De algemene toelichting van deze casus (hoofdstuk 5) laat zien dat we de onderzoeksvragen uit hoofdstuk 2 en 3 konden beantwoorden met beperkte middelen, onder andere door ze te formuleren binnen het oorspronkelijke studievoorstel en -budget (hoofdstuk 2) en door studenten aan het onderzoeksteam toe te voegen (hoofdstuk 3). De interactie met beleidsmakers was beperkt tot de bespreking van de onderzoeksresultaten. De gezondheidszorgonderzoekers hadden vooral contact met wetenschappers in de begeleidende klankbordgroep en met onderzoekers en stafleden van de StOEH. Het besluit van VWS om de evaluatieresultaten te laten meewegen in de beslissing over continuering van de financiering van het programma heeft het belang van die interactie onderstreept. Op grond van tal van bestuurlijke en organisatorische ontwikkelingen kan worden geconcludeerd dat de vraag of er een opsporingsprogramma voor FH wenselijk is, niet langer aan de orde is 2006. De discussie gaat momenteel vooral over de voorwaarden waaronder
dit programma kan worden geïmplementeerd. Ons gezondheidszorgonderzoek heeft aan deze ontwikkelingen bijgedragen met de productie van relevante wetenschappelijke kennis en door interactie en open discussie met beleidsmakers. Meerdere van onze resultaten zijn gebruikt in het beleidsproces dat resulteerde in de beslissing van de minister van VWS om het opsporingsprogramma te continueren en uit te breiden, evaluaties uit te voeren en verder onderzoek te stimuleren.

**Casus II. Landelijke implementatie van richtlijnen voor behandeling van longziekten?**

De tweede casus betreft de ontwikkeling en invoering van klinische richtlijnen voor behandeling van ademhalingsziekten bij oudere kinderen en volwassenen in Nepal (hoofdstuk 6 tot en met 9). De Wereldezondheidsorganisatie (WHO) ontwikkelt Practical Approach to Lung Health (PAL), een pakket van richtlijnen en implementatiestrategieën om de kwaliteit van zorg door eerstelijnsgezondheidswerkers in midden- en lage-inkomenslanden te verbeteren. In Nepal is een evaluatie uitgevoerd van de kosten en effecten van een proef-implementatie van PAL. De uitkomsten van dit gezondheidszorgonderzoek dragen bij aan de verdere wereldwijde ontwikkeling van PAL en aan het besluitvormingsproces over landelijke invoering van PAL in Nepal. In aanvulling op de initiële onderzoeksvraag over kosten en effecten van PAL hebben we meerdere inhouds-, context- en procesgeoriënteerde onderzoeksvragen geformuleerd. In hoofdstuk 6, 7 en 8 worden er drie beschreven, waarna in hoofdstuk 9 een algemene toelichting van deze casus wordt gepresenteerd.

Uit discussies binnen de groep van gezondheidszorgonderzoekers en de betrokkenen van de WHO en het National Tuberculosis Centre (NTC) bleek dat er in de opzet van de kosten en effecten studie niet voorzien was in een evaluatie van de inhoud van het PAL pakket. Bovendien was er geen nadere analyse gepland van de context waarin PAL geïmplementeerd werd. In hoofdstuk 6 beschrijven we hoe we, ex-ante, onderzocht hebben in hoeverre een succesvolle implementatie van PAL te verwachten valt op grond van de kwaliteit van de innovatie, de effectiviteit van de implementatiestrategie en het sociale systeem waarin PAL geïmplementeerd moest worden. Deze studie laat zien dat, anno 2002, de “implementeerbaarheid” van PAL wordt bedreigd op alle onderzochte aspecten. De kansen op effectieve implementatie kunnen worden vergroot door verbetering van de kwaliteit van de richtlijnen en versterking van de implementatiestrategie. Om internationaal beschikbare kennis in deze specifieke locale setting toe te kunnen passen zullen aanvullende, multifactoriële en duurzame interventies ontwikkeld moeten worden.

In hoofdstuk 7 beschrijven we de gezondheidszorgcontext voor PAL vanuit het perspectief van de patiënt. Om te weten hoe patiënten met longklachten het gezondheidszorg systeem ervaren en gebruiken hebben we een groep tuberculosepatiënten geïnterviewd. We onderzochten welke route ze door het zorgsysteem hadden afgelegd alvorens ze gediagnosticeerd en behandeld werden voor tuberculose. Deze routes begonnen meestal in de lokale geneesmiddelenverkopers en leidden, veelal via meerdere gezondheidsposten en zorgaanbieders naar klinieken met hoger gekwalificeerd personeel en betere voorzieningen waar de diagnose tuberculose werd gesteld. Verschillende systeemfactoren beïnvloedden deze routes zoals de
veronderstelde kwaliteit en kosten van zorg en het niveau van de dienstverlening. Bovendien bleek dat patiënten niet door zorgaanbieders werden verwezen maar vooral op eigen initiatief (en dat van hun familie en bekenden) op zoek gingen naar een andere zorgaanbieder als verbetering van de klachten uitbleef. Opvallend was dat pas wanneer de zorgaanbieder eenmaal dacht aan tuberculose, de verwijzing naar een laboratorium of behandelcentrum direct volgde. Meestal werd daarbij (terecht) vermeld dat de behandeling van tuberculose gratis was en vaak dichtbij huis verkregen kon worden. We concluderen dat de route die patiënten afleggen waarschijnlijk kan worden verkort wanneer zorgaanbieders patiënten beter verwijzen naar andere zorgaanbieders en wanneer de dienstverlening wordt verbeterd. De resultaten van deze studie zijn relevant voor de verbetering van strategiën voor passieve opsporing van tuberculose. Ook bij de ontwikkeling en implementatie van PAL zal rekening gehouden moeten worden met de beschreven systeemspecifieke kenmerken die de toegang tot, en het gebruik van zorgvoorzieningen beïnvloeden.

Van betrokkenen bij het PAL initiatief in Nepal kunnen we veronderstellen dat ze de implementatie van PAL belangrijk vinden. Het is echter niet duidelijk of anderen, dat ook vinden. In hoofdstuk 8 beschrijven we onderzoek naar het relatieve belang van verschillende gezondheidszorgprogramma’s. Hiervoor hebben we met 66 gezondheidszorgfunctionarissen een keuze-experiment uitgevoerd. Hieruit bleek dat prioriteit gegeven wordt aan gezondheidszorginterventies die zich richten op mensen in de arbeidsproductieve levensfase, op aandoeningen waarbij groot individuele gezondheidswinst te bereiken valt, op een ernstige aandoening waar grote aantallen patiënten aan lijden, die kosteneffectief zijn en die bijdragen aan armoedebestrijding. Wanneer we deze prioriteitsstelling toepassen op een groot aantal zorginterventies dan kunnen we concluderen dat PAL in Nepal (in vergelijking met andere programma’s) ook door niet-direct betrokkenen belangrijk wordt gevonden. Deze multi-criteria benadering is mogelijk een belangrijke stap op weg naar rationale prioriteitsstelling in gezondheidszorgbeleid in ontwikkelingslanden.

In de algemene toelichting in hoofdstuk 9 beschrijven we hoe we de verschillende onderzoeksvragen hebben kunnen uitvoeren. De studie naar de kansen voor succesvolle implementatie van Pal was als voorwaarde gesteld aan de betrokkenheid van de auteur van dit proefschrift bij de kosten en effecten studie. De studie naar de routes door het gezondheidszorgsysteem werd uitgevoerd door een Nederlandse stagiair en een Nepalese onderzoeksassistent. De studie naar de relatieve prioriteit van PAL hebben we kunnen uitvoeren tijdens een workshop die we op verzoek van de WHO en het NTC in Kathmandu hebben gehouden. Tijdens die workshop hebben we de voorlopige onderzoeksresultaten gepresenteerd en de eventuele toekomst van PAL in Nepal besproken.

Het gezondheidszorgonderzoek in deze casus werd gekenmerkt door een actieve samenwerking met de ontwikkelaars en uitvoerders van PAL in Nepal. Hierdoor konden al tijdens de evaluatieperiode verbeteringen in de implementatiestrategie worden voorgesteld. Bovendien hebben we door die samenwerking de kritische resultaten van de studie naar de “implementeerbaarheid” van PAL gezamenlijk kunnen publiceren en daarmee het draagvlak voor de voorgestelde verbeteringen vergroot. Een stuurgroep bestaande uit Nepalese beleidsmakers,
vertegenwoordigers van onderzoeksinstituten en niet gouvernementele gezondheidszorgorganisaties zorgden bovendien voor relevante contextspecifieke informatie en kritische reflectie. Hoewel de ontwikkeling van PAL wereldwijd een vlucht heeft genomen zijn er in Nepal nog geen activiteiten ontwikkeld om PAL landelijk te implementeren. Belemmerende factoren zijn, onder andere, het tekort aan collectieve middelen om in de implementatie van PAL te investeren en de vervanging van de charismatisch directeur van het NTC, tevens vervent bepleiter van PAL, door een meer terughoudende directeur. De onderzoeksresultaten hebben wel bijgedragen aan de discussie binnen de NTC om de private sector te betrekken bij activiteiten voor de verbetering van de kwaliteit van zorg voor patiënten met longziekten en in het bijzonder de passieve opsporing van tuberculose.

Casus III. Een raamwerk van prestatieindicatoren voor het Nederlandse gezondheidssysteem?

In de derde en laatste casus in dit proefschrift staat de ontwikkeling van een meetinstrument voor de prestaties van het Nederlandse gezondheidssysteem centraal (hoofdstuk 10 tot en met 12). Geïnspireerd door internationale ontwikkelingen omtrent vergelijking van gezondheidsystemen, onder andere door de WHO en de Organisatie voor Economische Samenwerking en Ontwikkeling (OESO), heeft het ministerie van VWS in 2001 het initiatief genomen een raamwerk voor prestatieindicatoren te ontwikkelen. Door daarbij onderzoeksondersteuning te bieden kregen wij, gezondheidszorgonderzoekers, de mogelijkheid een geheel nieuw instrument te ontwerpen dat zou worden gebruikt in de beleidspraktijk van VWS om de prestaties van het gehele gezondheidssysteem te monitoren en te sturen. Naast de initiële vraag over de ontwikkeling van het raamwerk (hoofdstuk 10) hebben we een aanvullende vraag geformuleerd over de beleidscontext en –processen die de ontwikkeling van het raamwerk beïnvloedden (hoofdstuk 11). In hoofdstuk 12 is een toelichting op de derde casus gepresenteerd.

In hoofdstuk 10 beschrijven we de ontwikkeling van het conceptuele raamwerk van prestatieindicatoren voor het Nederlandse gezondheidssysteem. Als basis voor het raamwerk hebben we een “balanced scorecard” gekozen met daarin vier management-perspectieven op gezondheidszorg: patiënt/cliënt oriëntatie, financiële aspecten van de zorg, het aanbieden van zorg van hoge kwaliteit en het vermogen van het gezondheidszorgsysteem om te leren en te vernieuwen. Vervolgens hebben we dit model gekoppeld aan het Lalonde model voor determinanten van gezondheid. Het resultaat is een model dat de relatie tussen volksgezondheid en het gezondheidszorgsysteem zichtbaar maakt. Daarnaast biedt het model de mogelijkheid om vanuit verschillende perspectieven prestatie-informatie te presenteren. Het model reflecteert de strategische behoefte van het gezondheidssysteem om bij te dragen aan het verwerven en behouden van gezondheid door kwalitatief goede zorg aan te bieden die bovendien toegankelijk is voor alle burgers. Tegelijkertijd voorziet het in de informatiebehoeften van beleidsmakers over de prestaties van het gezondheidssysteem in alle sectoren (curatieve zorg, zorg voor chronisch zieken, preventie en welzijn). De geselecteerde indicatorgebieden reflecteren de beleid- en managementfunctie van de overheid en de gedefinieerde publieke doelen van het gezondheidssysteem. Dit raamwerk is in februari 2003 formeel door
VWS aangenomen en aansluitend werden indicatoren voor de 26 indicatorenbieden ontwikkeld door vertegenwoordigers van verschillende afdelingen binnen VWS. In hoofdstuk 11 staan de context en de beleidsprocessen van de ontwikkeling van het raamwerk van prestatieindicatoren centraal. Deze ontwikkeling vond plaats in de interne dynamiek van een ministerie van categoriele beleidsprocessen van belang waren, zoals het terugdringen van de administratieve lasten en een nieuwe benadering van de financiële verantwoording en rapportage op het ministerie. Sommige beleidsprocessen versterkten de ontwikkeling van het raamwerk terwijl andere deze juist belemmerden. Op internationaal niveau heeft de betrokkenheid van Nederland bij vergelijkbare ontwikkelingstrajecten van OECD en Europese Unie sterk bijgedragen aan de ontwikkeling van het raamwerk in Nederland. Het onderstrept niet alleen het belang van prestatiemeting, maar er kon daardoor ook veel inhoudelijke afstemming plaatsvinden tussen de internationale en nationale ontwikkelingen. De intensieve en interactieve samenwerking en de vele formele terugkoppelingsmomenten met tal van betrokken groepen en individuen binnen VWS en bij kennisinstituten zoals het Rijksinstituut voor Volksgezondheid en Milieu, het NIVEL en Prismant hebben ertoe bijgedragen dat voor het raamwerk van prestatieindicatoren een breed draagvlak is ontstaan. Bovendien heeft VWS met een actieve communicatiestrategie de zorgaanbieders, verzekeraars en patiëntenvertegenwoordigers geïnformeerd over de doelstellingen, functies en gebruik van het raamwerk. Eind 2004 heeft het RIVM de opdracht gekregen het raamwerk verder te operationaliseren en te implementeren, en tevens te gebruiken als basis voor een nieuw verantwoordingsdocument over de prestaties in de zorg, de Zorgbalans. Sinds de afronding van onze onderzoeksondersteuning zijn er verschillende ontwikkelingen geweest. Bij de operationalisatie van het raamwerk (zoals gepresenteerd in hoofdstuk 10 en 11) zijn een aantal wijzigingen aangebracht. Zo is de expliciete koppeling van de balanced scorecard met volksgezondheidsinformatie verdwenen en zijn de indicatorenbieden opnieuw ingedeeld in niet vier maar drie perspectieven (kwaliteit, betaalbaarheid en toegankelijkheid). In mei 2006 is de eerste uitgave van de Zorgbalans verschenen met daarin de informatie over 26 indicatorenbieden uitgewerkt in 125 indicatoren. Ten tweede heeft de uitwerking van de indicator patiënt- en consumentervaringen geleid tot de introductie van nieuwe vragenlijsten voor het meteen van patiënten ervaringen met de zorg. De combinatie van de Amerikaans CAHPS® en Nederlandse Quote vragenlijsten is inmiddels uitgegroeid tot de standaard voor het meten van patiëntenervaringen in Nederland. Een landelijk instituut dat deze ontwikkeling gaat coördineren is in oprichting. Ten derde, op internationaal gebied heeft het Nederlandse onderzoek ten behoeve van de ontwikkeling van het raamwerk navolging gekregen bij de OECD. Die heeft een verdere uitwerking van het Nederlandse raamwerk geadoopteerd als het raamwerk voor het meten en vergelijken van de prestaties van de gezondheidssystemen van al haar lidstaten. We concluderen dat ons werk een directe bijdrage heeft geleverd aan de ontwikkeling van prestatiemeting van gezondheidssystemen, zowel nationaal als internationaal. Bovendien is uit de context- en procesbeschrijving gebleken dat het uiteindelijke resultaat tot ontwikkeling is gekomen ondanks, dankzij en vanwege de dynamiek van de politieke beleidsprocessen.
Discussie

In het laatste hoofdstuk van dit proefschrift, de discussie (hoofdstuk 13), worden de belangrijkste resultaten met betrekking tot de twee hoofdvragen in dit proefschrift op een rij gezet. Uit de drie casus (opsporing van familaire hypercholesterolemie, implementatie van richtlijnen voor longziekten en een raamwerk voor prestatieindicatoren) is gebleken dat het uitvoeren van onderzoek, in aanvulling op onderzoek ten behoeve van de initiële onderzoeksvragen, sterk beïnvloed werd door de beschikbare middelen zoals financiering en staf. Door de inzet van stagiaires konden verschillende studies worden uitgevoerd waar geen financiering voor was. In niet-academische instellingen zijn dergelijke oplossingen waarschijnlijk veel minder voor de hand liggend. In onze studies is gebleken dat betrokkenen (opdrachtgevers en beleidsmakers) enthousiast zijn over aanvullend onderzoek wanneer de verwachting bestaat dat de resultaten hun belang ondersteunen en er geen extra kosten mee zijn gemoeid.

Met betrekking tot de interactie tussen gezondheidszorgonderzoekers en beleidsmakers constateren we dat er in de drie casus in toenemende mate interactie plaatsvond. In de eerste casus waren de beleidsmakers pas tegen het einde van de studie betrokken omdat zij vooral behoefte hadden aan de evaluatieresultaten om op grond daarvan beleidsbeslissingen te kunnen nemen. In de tweede casus was er veel meer interactie, zowel formeel als informeel, en werd volop gediscussieerd over de ontwikkeling van PAL. In de derde casus werd vooraf gekozen voor een interactieve benadering. VWS was hierin opdrachtgever, financier, eindgebruiker en medeontwikkelaar. Voor een deel kunnen deze verschillen in benadering verklaard worden door een verschil in de informatiebehoeftte van de beleidsmaker. Daarnaast was er echter ook sprake van een bewuste keuze voor meer of minder interactie.

De bruikbaarheid van de onderzoeksresultaten uit de drie casus wordt op twee manieren beschreven. Ten eerste op grond van een typering van het gebruikproces —waarbij interactie een van de factoren is— en ten tweede op grond van het feitelijke gebruik. De beschouwing op het gebruik van de onderzoeksresultaten suggereert dat de resultaten van de combinatie van inhoud–, context– en proces georiënteerde onderzoeksvragen de bruikbaarheid van het initiële onderzoek heeft verstrekt.

In de discussie worden vervolgens een aantal methodologische beperkingen besproken. Ten eerste is het beleidsanalyse model retrospectief toegepast op gezondheidszorgonderzoek in de praktijk. De gepresenteerde casus zijn daarom niet evenwichtig gericht op inhoud, context, proces en actoren. Een logische volgende stap is dit model te hanteren bij de ontwikkeling van nieuwe onderzoeksprojecten. Ten tweede, de drie casus kenden elk een heel verschillende beleidscontext. Echter, de hier gepresenteerde casus zijn geen uitzonderingen in de portfolio van gezondheidszorg-onderzoekers en de heterogeniteit van context is in die zin dus representatief. Ten derde hebben we onze beschouwingen van de bruikbaarheid van het onderzoek gebaseerd op onze eigen observaties. In vervolgonderzoek zal meer aandacht kunnen worden besteed aan het vaststellen van het feitelijke gebruik van de onderzoekresultaten. Ten vierde, een mogelijk bezwaar van het interactieproces in de tweede en derde casus zou kunnen zijn dat de onderzoekers in hun wetenschappelijke vrijheid beperkt
werden omdat ze zelf deel gingen uitmaken van het ontwikkelings- en implementatieproces. Hoewel het behoud van wetenschappelijke vrijheid een uitdaging kan zijn wanneer tegenstrijdige belangen spelen, zijn we van mening dat dergelijke belemmeringen in deze casus geen belangrijke rol hebben gespeeld. Tenslotte bespreken we in dit laatste hoofdstuk de implicaties van deze bevindingen voor wetenschap, beleid en de praktijk van gezondheidszorgonderzoek. De *implicaties voor de wetenschap* hebben onder andere betrekking op verder onderzoek naar de toepassing van het model van Walt en Gilson bij het ontwerp, planning en evaluatie van gezondheidszorgonderzoek. In de nabije toekomst dienen inhoud-, context- en procesgeoriënteerde vragen geïntegreerd te zijn in gezondheidszorgonderzoek en moeten ze niet langer als “aanvullende vragen” worden beschouwd. Daarnaast verdient de vraag “wat is het beste interactie model” aandacht. Het antwoord is mede afhankelijk van de informatiebehoefte van de betrokken beleidsmakers. Tenslotte is het wenselijk dat gezondheidszorgonderzoekers het gebruik van onderzoeksresultaten gaan bijhouden (“post-marketing”) om op die manier bij te dragen aan de empirische kennis over het gebruik van onderzoeksresultaten. De *implicaties voor beleid* richten zich vooral op het belang van open communicatie tussen beleidsmakers en onderzoekers om al in een vroeg stadium de goede onderzoeksvragen te formuleren. De *implicaties voor de praktijk* van gezondheidszorgonderzoek richten zich op training van onderzoekers, de organisatie van onderzoeksprojecten en op het proces van onderzoek. In de training van gezondheidszorgonderzoekers dient meer aandacht te zijn voor het leren omgaan met andere partijen (met andere belangen), kennis van het gezondheidssysteem en kennis van beleidsprocessen. Bij de organisatie van gezondheidszorgonderzoeksprojecten dient veel ruimte te zijn voor bespreking van het wetenschappelijke perspectief, al vroeg in het onderzoeksproces. Dit geldt niet alleen voor de communicatie met beleidsmakers maar ook voor onderzoekers onderling. Voor het onderzoeksproces hebben de implicaties vooral betrekking op de communicatie met andere partijen. Het betreft het belang van overeenstemming over de aanpak van het gezondheidszorgonderzoek, het gebruik van de juiste terminologie en het publiceren van onderzoeksresultaten in tijdschriften die voor beleidsmakers relevant zijn. In conclusie, dit proefschrift laat zien hoe gezondheidszorgonderzoek voor nationaal gezondheidsbeleid in de praktijk werkt. We hebben gewezen op het belang en de meerwaarde van een combinatie van inhoud-, context- en procesgeoriënteerde onderzoeksvragen en op het belang van het interactieproces met beleidsmakers. Beide zijn een verrijking van gezondheidszorgonderzoek.
Dankwoord
Dankwoord

Hoewel de omslag van dit proefschrift alleen de naam van de promovendus vermeld is het zeker niet zo dat het een éénmansproductie is. In de afgelopen acht jaar hebben velen een belangrijke rol gespeeld in het werk dat uiteindelijk geleid heeft tot mijn “boekje”. Het pad ernaartoe was niet rechtlijnig. “Life is what happens while you are busy planning it” was voor mij een ontuchterende constatering in onrustige tijden wanneer pogingen om “werk” en “privé” te plannen weer eens anders uitpakten. Ik ben oprecht dankbaar voor de bijdragen die ik van velen langs en op dit pad heb ontvangen.

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Curriculum Vitae