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 *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.