For everybody who is a patient,
was a patient
or will be a patient.

And for Auke
who has been patient
About the cover

An important choice you need to make when coming close to the end of a PhD-thesis is what you want on the cover. In my case, I wanted to avoid clichés as ‘the patient as a vulnerable and dependent person’ and ‘the researcher with his test tube’ since it would not cover the content nor the context of the research presented. Patient participation in health research creates turbulence and uncertainty. It is a process of exploring and discovery, with barriers and solutions. Patient participation mostly entails working together and complementing each other. Through this PhD research many things became elucidated, while some issues are still in the dark.

The painting on the cover, made by a talented artist, Victor, embodies all these impressions. This painting has been in my life for quite some time now. I remember the painting in the process of becoming this image; I must have been around 13 years old. Now the painting adorns my grandparents’ house. I love the vibrant colours, the turbulence and the ‘undiscovered’ area in the upper right corner. It invites you to explore, despite the turbulence you can come across.

When reading this thesis, you will come across different fragments of this painting. It is an expression of what you will find in that part of the thesis and I hope it will invite you to read on.
# Content

## PART 1 Setting the stage
- Chapter 1. Introduction 3
- Chapter 2. Theoretical background 7
- Chapter 3. Research design 17

## PART 2 Into the system
- Chapter 4. Towards a needs-oriented health research system 37

## PART 3 Setting priorities
- Chapter 5. How to stimulate inclusion of patients’ perspectives 71
- Chapter 6. Patient involvement in agenda setting 91
- Chapter 7. Setting the research agenda for medical products 99

## PART 4 Towards structural involvement
- Chapter 8. Don’t forget the professional 125
- Chapter 9. Towards structural patient participation in health research 143

## PART 5 Conclusions
- Chapter 10. Conclusion and Discussion 163

## Summary 181
## Samenvatting 189
## References 199
## Acknowledgement/Dankwoord 221
Account

Chapter 4 to 10 are based on co-authored articles that are published, in press or in review for publication, in peer reviewed journals. In these chapters, I have chosen to maintain the ‘we-form’ in which the articles were original written. Also the terminology used in the articles is not adapted which leads to some inconsistency in terminology throughout the thesis.

Chapter 4

Chapter 5

Chapter 6

Chapter 7

Chapter 8
M.P.T. de Wit, J.E. Elberse, J.E.W. Broerse, T.A. Abma. (accepted). Don’t forget the professional – The value of the FIRST model for guiding the structural involvement of patients in rheumatology research. Health Expectations

Chapter 9
Chapter 10

J.E. Elberse & J.E.W. Broerse (In preparation for publication). The embedding of patient participation in the health research system.
Preface

Travelling the world, meeting new people, exploring different cultures, seeing new sceneries, experiencing other tastes and senses is one of my favorite activities. Although the research presented in this book is mostly based in the Netherlands, it has been one of my favorite trips, offering all above mentioned aspects. Looking back, it has been an interesting route.

Since I was little, I always wanted to understand how the world works. Studying Molecular Sciences provided many answers, but raised even more new questions. It showed how the world works in a ‘technical’ way. Molecular Sciences brought me from a first internship in a very prestigious and well organized laboratory to a second internship in Nairobi, Kenya where things were not so organized. However, Kenya provided an amazing surrounding. Still, I missed the connection to society in the scientific work I was doing. For whom was I doing this research and what do they think about it? In Kenya I met someone who introduced me to Science, Technology and Society studies. This showed me the insights I was looking for: how to connect science and society. Science, Technology and Society studies led me to Maastricht and Oslo. It provided more insight into how the world works in a ‘societal’ way. I came across the collaboration between patients and medical researchers, which fascinated me. And it still does. I got the opportunity to investigate in detail how patients can participate in order to improve medical science, which I really much enjoy.

With this work I wanted to contribute to science as well as society. I feel that, together with my colleagues, I was able to accomplish this. We contributed to an increased understanding on how patient participation in health research can be shaped effectively. Also, we shed light on what patient participation can mean for a potential system innovation towards a needs-oriented health research system. In terms of social relevance, that was closely related to the core of my research: how can health research become more responsive towards society, and to be more precise, to the needs of patients. There is an increased understanding on how needs-articulation processes of patients and other stakeholders can be shaped and optimized to provide patients the opportunity to become genuinely involved. In addition, the lessons learned provided indications of improve how patients can also be involved in the conduct of research. During this research, many people and societal organizations were given a voice and ‘society’ became actively involved in science. I hope this work may further raise awareness about the potential benefits of involving patients in health research. Since patient participation in health research involves a radical change in the health research system, an open mind is required. And, similar to traveling, if you have an open mind, the world will show you the most beautiful places.
Part 1. Setting the stage
Chapter 1 Introduction

“Active patient participation should be enjoyable instead of a burden”, stated a participant at a dialogue between patients and researchers on greater patient involvement in health research. The sentiment is illustrative of the barrier to participation experienced by many patients and researchers as well as a vision of what patient participation could be for health research. It captures in a nutshell the topic of this thesis: the challenges and possibilities for current developments concerning patient participation in health research and the potential change it can bring about.

The involvement of patients in health research has grown over the past two decades, with patients and patient groups demanding that their voices be heeded. This trend began in the 1980s when people with disabilities mobilized under the slogan “Nothing about us without us” (Charlton 2000). The slogan neatly sums up the message that research should not only be conducted ‘on’ patients but ‘with’ patients actively involved in decision-making. This normative argument for greater patient involvement acknowledges their role as the end-users and primary stakeholders of health research. Greater patient participation would increase both the political legitimacy and improve the quality of the decisions that are currently being made (Fiorino 1990; Goodare and Smith 1995).

A second reason to actively involve patients in health research is that it can lead to higher levels of social support for, and acceptance of, research (Whitstock 2003). If patients are involved in deciding the topics, the legitimacy of research is enhanced (Rowe and Frewer 2000; Rowe and Frewer 2004). Patient involvement would improve the implementation of knowledge and innovation as well as the societal sharing of knowledge.

Third, scholars have argued that active patient involvement would improve the quality and relevance of health research as their experiential knowledge – acquired through living with an illness, sickness and/or disease – can complement the scientific knowledge of researchers (Chalmers 1995; Popay and Williams 1996; Entwistle, Renfrew et al. 1998; Goodare and Lockwood 1999; Nordin 2000; Telford, Beverley et al. 2002; Caron-Flinterman, Broerse et al. 2005; Needham 2008). By involving patients, research can become more responsive to their needs (Oliver 1995; Entwistle, Renfrew et al. 1998; Boon, Moors et al. 2011).
Health researchers today increasingly consult patients and policy-makers in health research-related organizations increasingly consider patients as stakeholders and repositories of experiential knowledge (Boote, Telford et al. 2002; Beresford 2007; Oliver 2008). Patients now contribute to decision-making and enter partnerships with researchers in different health research-related domains (O’Donnell and Entwistle 2004; Shea, Santesso et al. 2005; Serrano-Aguilar, Trujillo-Martin et al. 2009). The voice of patients is gaining weight within health research (Caron-Flinterman 2005).

Greater patient involvement is supported by the changing relationship between science and society. Science increasingly takes into account perspectives from a variety of stakeholders and is becoming more democratic and participatory (Klein 2001; Nowotny, Scott et al. 2001; Chopyak and Levesque 2002). The public is increasingly empowered and demands more legitimacy and accountability from science (Collins and Evans 2002; Bucchi and Neresini 2008). At the same time, the authority of experts is withering (Cornwall and Jewkes 1995; Rowe and Frewer 2000; Kerr, Cunningham-Burley et al. 2007) while patients are emerging as prominent actors in health care (Traulsen and Noerreslet 2004; Barbot 2006; Epstein 2008; Williamson 2008). In the light of these developments, it is hardly surprising that patients and patient groups also want to become more involved in health research. But despite the increasing incidence of patient participation and support for the idea that it can benefit health research, patient participation in health research remains far from the norm.

Patient participation implies that patients become central actors within health research systems. But in the current system of health research, the role of patients is often restricted to being the subjects of study or the beneficiaries of its knowledge and products (Oliver 2006). The health research system is responsible for generating knowledge through ‘scientific inquiry’ focusing on people’s health and well-being (Pang, Sadana et al. 2003). The system has been highly successful; it has generated an enormous amount of knowledge and innovation that has allowed many to live longer, fuller lives. The current system – built upon established relationships between actors, stabilized ways of interaction, set procedures, standardized routines, and shared values and views on research (Telford and Faulkner 2004; Caron-Flinterman, Broerse et al. 2007; Broerse, Elberse et al. 2010) – has developed over time. Its culture, structure and practices are difficult to change.

However, there is a dilemma in the way the health research system is currently functioning. Despite its successes, the system is rather supply-driven, since research is driven by the ideas and interests of the suppliers of knowledge and innovation: researchers and the pharmaceutical industry. The system’s supply-driven nature leads to gaps in knowledge development, to neglected and underrepresented areas of research that would cater to the needs of patients. Research on topics selected by researchers and experts does not necessarily correspond to patients’ needs; nor is it always pursued in ways preferred by patients (Chalmers 1995; Grant-
Greater patient involvement and integration of their experiential knowledge would lead to the identification of topics that are currently neglected.

Although patients today are increasingly involved in health research, the pace of change is slow. Active patient involvement still only occurs on a limited scale, and difficulties, struggles and challenges remain. Giving patients a voice implies a major change in the organizing, thinking and doing of the health research system and its actors (Caron-Flinterman, Broerse et al. 2007). There is, however, limited scientific knowledge to build upon as only a few patient participation initiatives have been thoroughly evaluated; there are as yet few proven methodologies for patient participation in health research. In addition, the lessons that have been learnt are rarely exchanged between practitioners in the field.

This study aims to achieve greater insight into how the trend of increasing patient participation in health research can be enhanced and how patient participation can be embedded in the health research system. In addition, it aims to understand whether and to what extent increased patient participation could facilitate a shift towards a more needs-oriented system. Can the growing role of patients address the observed gaps between ‘supply’ and ‘needs’ in the current health research system? Addressing these questions will ultimately contribute to making patient participation, rather than a burden, a truly enjoyable endeavour.
Chapter 2 Theoretical background

In this chapter, the relevant theoretical concepts for this study are presented. First, I describe patient participation in more detail, followed by important notions of transition theory.

Patient participation in health research

Patient participation in health research can be defined as patients being actively involved in and having influence on decision-making processes in health research (Elberse, Caron-Flinterman et al. 2011). Health research spans the entire range from (bio)medical research, clinical research, public health research, epidemiological research to care research. In the literature, different terms are used to indicate patient participation (Boote, Telford et al. 2002) e.g. patient and public involvement (PPI) (Howe, MacDonald et al. 2006), (health) consumer participation, consumer involvement (O’Donnell and Entwistle 2004; O’Donnell and Entwistle 2004; Telford, Boote et al. 2004), service user involvement (Barber, Beresford et al. 2011) or (potential) care user involvement. In this thesis, the term ‘patient participation’ is used to indicate patients, patient representatives, patient groups, or other actors that represent the views and perspectives of patients in decision making being actively involved in and having an influence on decision-making processes in health research (Elberse, Caron-Flinterman et al. 2011).

Patient participation takes several forms ranging from providing information to researchers about their disease or experiences to being an influential counterpart in research. Different models are developed to distinguish different levels of involvement (Arnstein 1969; Cornwall 1996; Caron-Flinterman 2005; Oliver, Rees et al. 2008). Often a distinction is made between (1) ‘consultation’ whereby patients are consulted regarding their views, ideas and needs, but the decision still lies with the researchers, (2) ‘collaboration’, whereby patients collaborate with researchers on an equal footing and decisions are taken jointly, and (3) ‘control’, whereby decisions are made by patients (Beresford 2010). In Table 2.1 the six levels of a ladder of patient participation in health research processes are described (Arnstein 1969; Caron-Flinterman 2005; Abma and Broerse 2007; Oliver, Rees et al. 2008). These levels concern different degrees of involvement in and influence on decision-making processes by patients. The lowest rung ‘subject of study’ is considered non-participation as patients have no influence whatsoever. Patients have traditionally been involved at this level. With ‘passive consultation’, the influence of the patient is minimal; they provide information to a researcher (e.g. by filling in questionnaires) without having any influence on which information is collected and what is done with the information. Also, patients cannot give a certain ‘weight’ to the provided information. ‘Active consultation’ involves the qualitative collection of patient views (e.g. via interviews or focus group discussions) whereby
patients have an active/interactive role. Patients can influence which information is provided. They can also decide what they would like to share with the researcher or how important certain information is. The level of ‘advice’ implies the involvement of patients in advisory or decision-making bodies without guaranteeing their actual influence on decision-making processes. It is still up to the experts to take the input of patients into account. The highest two levels, ‘partnership’ and ‘patient control’, imply real shifts in decision-making power. In partnerships, patients and experts take decisions jointly. The different perspectives are taken into account, and genuine deliberation and negotiation can lead to final outcomes supported by both parties. Patient control takes this one step further to a situation where the decision-making power is transferred from experts to patients (Turner and Beresford 2005; Faulkner 2010; Polich 2012). An example would be a patient organization that commissions research and decides what is researched, how and by whom.

<table>
<thead>
<tr>
<th>Table 2.1 Ladder of patient participation in health research</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patient control</td>
</tr>
<tr>
<td>Partnership</td>
</tr>
<tr>
<td>Advice</td>
</tr>
<tr>
<td>Active consultation</td>
</tr>
<tr>
<td>Passive consultation</td>
</tr>
<tr>
<td>Subject of study</td>
</tr>
</tbody>
</table>
A higher level of involvement is not automatically ‘better’, and the described levels should not be seen as a normative model. Moreover, not every patient aspires to be involved on a high level. Some patients are not interested or willing to participate in health research at all, not wanting to invest time and energy in such an endeavour. Also, this is a legitimate choice for patients to make (Van de Bovenkamp, Grit et al. 2008). Instead of aiming for a high level of involvement, it is more important that the level of participation suits the situation (Caron-Flinterman 2005). In fact, all levels of involvement are important and possibly even necessary. Although the participation ladder is a useful tool in characterizing initiatives according to the level of patient involvement and influence on the decision-making process, it does not indicate how to organize patient participation effectively (Titter and McCallum 2006; Titter 2009). It does not provide insight on how to put patient participation into practice.

In principle, patients could be involved in all phases of the research process: agenda-setting (e.g. Petit-Zeman, Philpots et al. 2010; Stewart, Caird et al. 2011; Lloyd, White et al. 2012), research design (e.g. McCormick, Brody et al. 2004; Ali, Roffe et al. 2006; Staniszewska, Jones et al. 2007; Boote, Baird et al. 2010; Boote, Dalgleish et al. 2012), proposal appraisal (e.g. O’Donnell and Entwistle 2004; O’Donnell and Entwistle 2004; Saunders, Girgis et al. 2007; Teunissen, Visse et al. 2011), patient recruitment (Donovan, Brindle et al. 2002; Langston, McCallum et al. 2005), study execution and analysis (Coupland, Maher et al. 2005; Faulkner 2006; Wright, Corner et al. 2006; Morrow, Ross et al. 2010), interpretation of research results (Ross, Donovan et al. 2005), dissemination of research results (McLaughlin 2006), and implementation and utilization of research results (Abma, Nierse et al. 2009). Some scholars who studied or experimented with patient participation indicated that involvement throughout the research process will lead to the highest impact (e.g. (Hanley, Bradburn et al. 2003; Minogue, Boness et al. 2005; Hewlett, De Wit et al. 2006; ZonMW 2006; Wyatt, Carter et al. 2008).

Furthermore, patients can participate in different types of health research, varying from biomedical research (Caron-Flinterman 2005), clinical research (Epstein 1995; Goodare and Lockwood 1999; Collyar 2000; Hanley, Truesdale et al. 2001; Marsden, Bradburn et al. 2004; PatientPartner 2011; PatientPartner 2011), health technology assessment (Oliver, Milne et al. 2001; Abelson, Giacomini et al. 2007; Bridges and Jones 2007; Facey, Boivin et al. 2010), systematic reviews (Boote, Baird et al. 2011), and evaluation (Baur, van Elteren et al. 2010) to health service research (Ong and Hooper 2003; McIntyre, Novak et al. 2010).

When discussing patient participation, there are some misconceptions which are important to briefly address here. Firstly, patient participation does not mean that patients are going to take over all the tasks of researchers and experts, since scientific training and competences are needed for many tasks. Patients are not going to decide on ‘which mice model to use’ or ‘how to execute lab work’. Secondly, as soon as people become ‘patients’, they are perceived as ignorant,
somebody to feel sorry for, although everybody can become a patient, including doctors, lawyers or teachers. When people are ill, they still have knowledge, ideas and competences, and they should be seen as a person instead of only ‘a patient’ (Schipper 2012). Thirdly, there is a misconception that patients do not appreciate basic research. If patients were to become involved in health research, they would only focus on research that they themselves can benefit from in the short term. The last assumption has been refuted by the research of Caron-Flinterman et al. (Caron-Flinterman 2005; Caron-Flinterman, Broerse et al. 2005; Caron-Flinterman, Broerse et al. 2007). However, patient participation is not a panacea. Often is cost more time and money to involve patients actively (Boote, Baird et al. 2010) and some scholar stress to possibility to overburden patients (Van de Bovenkamp, Trappenburg et al. 2010; Cotterell, Harlow et al. 2011).

**Structural patient participation in health research**

Many initiatives, including agenda-setting projects, are often one-off events, involving attending one meeting or a temporary project (Jordan, Dowswell et al. 1998; Stevens, Wilde et al. 2003). Or patients are involved ad hoc at the request of researchers or policymakers. Caron-Flinterman et al. concluded that these strategies are not always effective (Caron-Flinterman, Broerse et al. 2007). One-off or ad hoc involvement can have several limitations (Jordan, Dowswell et al. 1998). Especially ad-hoc involvement with limited resources in a short time span may result in a low quality of participation or even tokenistic involvement. Furthermore, as described by Boote et al., in case of one-off events, the research design and questions are already fixed, so patients cannot influence them (Boote, Baird et al. 2010). Also, collaboration between patients, funding agencies and researchers are not sustained. Established relations disappear, and knowledge and contacts are lost. Initiatives are not continued, and there is hardly any feedback or future planning (Stevens, Wilde et al. 2003).

Therefore, it is increasingly argued that patient participation in health research should be more structural (Kirwan, Ahlmen et al. 2005; Hewlett, De Wit et al. 2006; ZonMW 2006; Abma, Nierse et al. 2009). Since the active involvement of patients is new for both patients and researchers, the parties need to adapt to their role in collaboration (Howe, MacDonald et al. 2006). Howe (2006) describes that the experiential knowledge of patients was only acknowledged after a long period of cooperation between patients and researchers. Consequently, it may take a longer period before relevant outcomes of the participation process become visible. With a more structural role of patients in research, established relationships and gained knowledge and competences are maintained and patients are given a stronger voice.

In this thesis, structural patient participation is defined as patients having a place in decision-making structures and influence on the decisions taken, with patients and researchers having a
long-term commitment for collaboration in different phases of health research. Both parties deliberate and interact regularly and share decision-making power equally (Beresford 2007; Lindenmeyer, Hearnshaw et al. 2007; Abma, Nierse et al. 2009; Ward, Thompson et al. 2010). In an ongoing dialogue with researchers, patients provide experiential knowledge which is integrated with scientific knowledge and practices (Jinks, Ong et al. 2009; Kjeken, Ziegler et al. 2010; INVOLVE 2012). Structural patient participation will lead to a redefinition of power relations between patients, researchers and other important players in the field, which is often considered difficult by professionals or even undesirable (Williamson 2001; Beresford 2007; Lindenmeyer, Hearnshaw et al. 2007; Hubbard, Kidd et al. 2008; Thompson, Barber et al. 2009; Ward, Thompson et al. 2010).

Transition theory

As described above, in the long term the structural involvement of patients seems most desirable. Nonetheless, realizing the structural participation of patients in health research can be expected to be challenging. Changes are needed in the current structures, practices and culture of health research. According to Caron-Flinterman et al. and Broerse et al. (Caron-Flinterman, Broerse et al. 2007; Broerse, Elberse et al. 2010), transition theory could provide ideas for understanding the difficulty of changing systems and guidance and instruments for embedding patient participation in the health research system.

In this thesis, transition theory will be used as a conceptual framework to analyse how to enhance the embedding of patient participation in the health research system. The concept of ‘transition’ or ‘system innovation’ refers to a socio-technological process of change of a societal (sub)system, involving both established patterns of action and the societal structures in which these actions take place (Rotmans 2005; Grin, Rotmans et al. 2010). Transitions involve multiple actors from the institutional sphere: government, private for-profit sector, science and civil society. But while transition theory is mainly focused on social-technological transitions in the field of energy (Verbong and Loorbach 2012), agriculture (Klerkx and Leeuwis 2009) and mobility (Geels, Kemp et al. 2012) where technology is the central focus, little work is being conducted using transition theory in the area of health or health research (Bunders and Broerse 2010). In this thesis the focus will be on socio-practical change in the health research system.

Two central notions in system innovation and transition theory are the multi-phase concept (Rotmans, Kemp et al. 2001) and the multi-level perspective (Geels 2005; Geels and Schot 2010). Due to difficulty of change, system innovations are often slow processes, taking 20-30 years, although breakthroughs might be relatively fast. The multi-phase concept describes a process in which a system transforms from one equilibrium state via quick and unstable developments to a
new relatively equilibrium state (See Figure 2.1). However, the direction and the end goal of the system innovation are not fixed. In this process of change, four phases can be distinguished as described by the multi-phase concept, which is visualized as an S-curve (Rotmans, Kemp et al. 2000; Kemp and Loorbach 2003; Rotmans 2003):

1. A predevelopment phase, in which no visible changes occur.
2. A take-off phase, in which the first signs of change become noticeable and change begins.
3. An acceleration phase, in which the pace of change increases, visible structural changes take place through an accumulation of socio-cultural, knowledge development, environmental, and institutional changes that influence each other, including processes of collective learning, diffusion and embedding.
4. A stabilization phase, in which the pace of change decreases and a new stabilized situation arises (Loorbach 2007).

Not all system innovation processes are completely successful and reach a new stabilized situation. A ‘lock-in’ can occur when the process quickly slows down and no acceleration of the pace takes place. With a lock-in, particular solutions are developed which are not always the best choice for a long-term perspective (Loorbach 2007). In other potential system innovations, a backlash may be observed. The development seems to go at high speed, but results do not materialize and settle in. Everything returns more-or-less back to ‘normal’.

![Figure 2.1 Four phases of a transition process](image)

To conceptualize the process of system innovations, a multi-level concept was developed (See Figure 2.2) (Geels 2002; Geels 2005; Grin, Rotmans et al. 2010). This concept offers a descriptive, analytic framework. The multi-level concept describes three levels of social organization that are involved in system innovations on which interference of processes take place (Geels and Kemp 2000; Rotmans, Kemp et al. 2001; Berkhout, Smith et al. 2004; Rotmans 2005):
1. The landscape level comprises the broad context for niches and regimes, consisting of shared cultures, worldviews, values and paradigms, but also the macro economy, demography and the natural environment. Changes at a broader societal landscape level (macro level) may stimulate and accelerate the transition. However, these trends are beyond the direct influence of people (Rotmans 2005).

2. The regime level consists of networks of stakeholders and organisations that have shared assumptions and interact via dominant practices and rules. The regime functions in a stabilized manner, whereby current structures, practices and culture steer the thinking and acting of the people in the regime.

3. The niche level concerns individual stakeholders and their actions and practices where ‘niches’ may be developed in which social or policy innovations can arise. In system innovation processes, systemic changes are mostly initiated at this level, with societal actors acting as ‘change agents’: people or organisations who try to stimulate change, setting up various small experiments, so-called ‘niche experiments’ within this level. Change agents undertake actions and are willing to invest time and resources. The activities or behaviour of actors in a niche differ from the current regime (Rotmans 2005). Niche experiments play a crucial role in system innovations to test and experiment with novelties like new practices.

Figure 2.2 The multi-level model (adapted from (Geels 2002))

Besides the multi-phase and multi-level concepts, the concepts ‘culture-structure-practice’ are described as relevant notions for system innovation (Rotmans and Loorbach 2006; Rotmans and Loorbach 2010; Van Raak 2010; De Haan and Rotmans 2011). Van Raak (2010) defines culture as ‘a set of values, perceptions and interpretative frames that are shared by most of the involved actors’; practice as the actual actions undertaken by actors which are relevant for the functioning
of the system; and structure as ‘the physical, economic, legal, financial, organisational and power structures that facilitate and/or constrain the behaviour of the actors involved’ (Van Raak 2010). The current culture, practice and structure of the health research system developed over time, resulting in a complex, stabilized way of organizing, thinking and acting (Frenk 1992). The culture and structures are shaped by the practices of the stakeholder groups involved and at the same time, practices are encouraged or limited by the structures and culture (Loorbach 2007; Van Raak 2010).

Steering notions

Although transitions can probably be neither controlled nor steered, they can be influenced, particularly with respect to direction and speed, through so-called ‘transition experiments’. “A ‘transition experiment’ is an innovation project with a societal challenge as a starting point for learning aimed at the contribution to a transition” (Van der Bosch and Rotmans 2008). Transition experiments are small-scale, practical experiments with a high potential to contribute to transitions (Rotmans 2005; Raven, Van der Bosch et al. 2010). They can be used as an instrument to stimulate a system innovation (Kemp and Van der Bosch 2006; Loorbach 2007). The focus of a transition experiment is a societal challenge, such as how to address certain needs instead of a technical starting point like a new innovation (Van der Bosch and Rotmans 2008; Raven, Van der Bosch et al. 2010; Van der Bosch 2010).

In transition experiments three steering mechanisms can be applied in order to enhance a system innovation or transition: (1) deepening, by learning as much as possible in a specific context, (2) broadening, by linking and repeating the experiment in different contexts, and (3) scaling up, by embedding the experiment in the incumbent regime, changing the dominant way of organizing, thinking and acting (Rotmans and Loorbach 2006; Van der Bosch and Rotmans 2008; Raven, Van der Bosch et al. 2010). These steering mechanisms focus on the importance of creating spaces for learning processes at different levels, while at the same time stimulating interaction between experiments and the broader context, and actively working on embedding the new practices to increase the impact of the experiment at a higher scale level (Van der Bosch and Rotmans 2008; Van der Bosch 2010). During the system innovation, all three steering mechanisms are essential, and it is important to pay attention to them simultaneously (Broerse, Essink et al. 2010).

Deepening

Deepening involves social learning processes in a specific context, implying shifts in thinking about culture, structure and practice, their relationship with other actors and the broader context, as well as the interaction between the new culture, structure and practices (Röling 2002; Van der Bosch and Rotmans 2008). Experimenting with and learning from transition experiments could
lead to the enhancement of a shift in thinking and doing (Schot and Geels 2007). Learning can be considered an interactive process to develop or obtain knowledge, competences, norms and values. Attention should be paid to experiment-based learning of the possibilities and constraints. It is important to develop ‘proof-of-principle’ or ‘best practices’: Does it work, in which form, and what are the benefits or added value? These best practices are essential to convince new actors to become involved.

It is also relevant to gain insight into the expectations and attitudes of the actors involved (Raven 2005). It is important to shape the different expectations to ensure that they are realistic and to identify which aspects vary. During transition experiments, it is possible that expectations may change. Alignment between the stakeholders is needed in order to integrate the different expectations into a shared vision. New, innovative experiments demand open-minded people, willing to experiment with new practices (Van der Bosch and Rotmans 2008; Van der Bosch 2010). These ‘change agents’ provide a strong drive, which is essential for system innovation.

**Broadening**

Broadening entails repeating experiments in different contexts (Raven 2005; Geels and Raven 2006; Rotmans and Loorback 2006) and linking experiments to other functions. Experiments which are considered successful can be repeated in a different setting, while applying the lessons learned and adapting the experiment to the new context. Guiding principles can be established based on previous experiments to provide direction and support for new experiments (Raven, Van der Bosch et al. 2010).

**Scaling up**

Scaling up refers to activities to embed the new culture, structure and practices at the regime level (Geels and Raven 2006). When scaling up, a shift in thinking, doing and organizing is taking place in which the dominant regime changes fundamentally. This steering notion is the most challenging one. Much resistance can be encountered, and institutional barriers have to be overcome. It is of major importance to involve key figures who have the willingness to change and have access to resources and support (Van der Bosch 2010). Also, it is important that landscape developments support this shift.
Chapter 3 Research design

In this chapter, I present the objectives and main research questions. The methodologies used to answer the main research question are described, followed by issues of validity and the research team.

Objectives and main research question

This thesis has three main objectives:

1. To contribute to an increased understanding of how patient participation in health research can be shaped effectively;
2. To contribute to an increased understanding of the embedding of patient participation in the health research system;
3. To acquire insight into the extent to which increased patient participation may contribute to a transition towards a more needs-oriented health research system.

The following main research question is formulated:

How to realise patient participation in health research in such a way that it becomes embedded in the research system and contributes to a system innovation towards a more needs-oriented health research system?

Research approach

The main research approach applied in this thesis is the case study. The case study approach is used to study and learn from and to obtain a better understanding of certain phenomena in a real-life setting (Yin, 2003). The real-life settings of interest in this study are transition experiments involving patient participation in health research in a concrete situation. In these settings, different actors like funding agencies, patient organisations and research departments are experimenting with patient participation in health research, using them as ‘testing grounds’ to develop, test, learn from and improve current research practises. The use of transition experiments is thus appropriate to gain in-depth insight into the ‘how’ of patient participation and its embedding in the health system.

For the selection of appropriate case studies, the following criteria were used:

1. The case should be a transition experiment; i.e. it should be an innovative project with a societal challenge as a starting point for learning – patient participation in health
research – aimed at the contribution to a system innovation – of the health research system.

2. In the transition experiment, patient participation in health research should take place in a real-life setting. This implies that actors in the field need to be willing to take the initiative to ask researchers from the Athena Institute to become involved.

3. The transition experiment should provide opportunities for answering the scientific questions posed in this study.

4. In the transition experiment, the role of patients is preferably ‘partnership’, i.e. their input is considered equal to that of the researchers, but should be at least at the level of ‘active consultation’, meaning a clear commitment to include the patients’ input visibly in the outcome.

5. The selected transition experiments should – together – be sufficiently diverse in context and requirements in order to obtain new insights into how to shape or embed patient participation in health research.

Four transition experiments provided the main data for this research. The selected ones focused on two different phases of the research process, namely ‘research agenda setting’ and ‘doing research’. The phase of ‘research agenda setting’ is an area where a validated methodology for patient participation is available and various ‘best practices’ exist. Therefore, these transition experiments provide good opportunities to address the three formulated objectives of this thesis. The phase of ‘doing research’ is an area where there is currently very little practical experience, and little is known about how to realise patient participation.

The health research system

To gain insight into the health research system and the state-of-the-art of patient participation in health research in the Netherlands, we conducted an inventory study. We analysed the health research system, various initiatives for patient participation, current barriers to patient participation as well as the manifestations of and possibilities for the use of the various steering aspects for realizing system innovation towards a needs-oriented health research system. As such, this research set the stage for the investigation of some aspects in more detail in the transition experiments.

This inventory study addresses the following sub-questions:

1. Which developments are taking place in the Netherlands related to patient participation in health research?

2. Which activities are undertaken by two defined change agents who are experimenting with patient participation in the Netherlands?
3. Which barriers are encountered related to the incumbent health research system?
4. How can the steering aspects ‘deepening’, ‘broadening’ and ‘scaling up’ be applied to this system innovation?

A broad variety of research methods is used, including literature review, interviews, case study, informal conversation, observation and attendance at relevant meetings and conferences. The inventory study is described in detail in part 2 – Chapter 4 – of this thesis.

**Patient participation in health research priority setting**

Three transition experiments were developed, implemented and analysed to gain insight into patient involvement in health research agenda-setting and its potential to enhance a system innovation towards a needs-oriented health research system. They are described in detail in Part 3 of this thesis (Chapters 5-7). In all three transition experiments the Dialogue Model is applied to research agenda-setting. The transition experiments were initiated by the Dutch Heart Foundation, the Netherlands Asthma Foundation and the Health Council of the Netherlands and took place in real-life settings, thus providing the perfect opportunity to learn more about different challenges and possibilities, barriers and facilitators regarding patient participation in research agenda-setting.

**The Dialogue Model**

A ‘research agenda’ is a list of relevant research topics in a specific field. It can be used by funding agencies, governments and research organizations and can be formulated in several ways, informally or formally, with a political mandate or, most of the time, by the scientific community. About a decade ago, there was very little knowledge on how to realize patient involvement in research agenda-setting (Abma and Broerse 2007; Abma and Broerse 2010). In the Netherlands there was a growing interest among patient organizations and funding agencies to involve patients in establishing a research agenda. Therefore, the challenge of realizing patient participation in health research agenda-setting was taken up there. The Dialogue Model was developed which operationalizes collaboration between different stakeholders, including patients (Caron-Flinterman, Broerse et al. 2005; Nierse, Abma et al. 2006; Abma and Broerse 2007; Abma and Broerse 2010; Schipper 2012). The Dialogue Model is partly based on participatory and interactive methodologies and the Interactive Learning and Action approach (Broerse 1998; Broerse and Bunders 2000; Caron-Flinterman 2005; Roelofsen, Broerse et al. 2008).

In the Dialogue Model, research is not framed by the relevant interests of experts but developed in interaction with various stakeholders. Stakeholders are those actors with a stake in the research carried out, and who have an interest in the outcome of this research. The model is grounded in the notion that participation is a dialogue process between stakeholders. It is assumed that the
different stakeholders each have their own perspective, and that all these perspectives are relevant for addressing the issues at hand. Instead of shifting control from one group to another, this approach emphasizes dialogues and equal space for contributions (Abma and Broerse 2007; Abma and Broerse 2010; Schipper 2012). It excludes neither professionals nor patients but includes the perspectives of all stakeholders. Dialogue seems a productive way to incorporate patient participation. The Dialogue Model seeks a combination of consultation and collaboration. Articulation of research topics by different stakeholder groups is an important process within the Dialogue Model. In a facilitated way, the different stakeholders translate their needs, wishes and experiences in daily life into research topics.

Since 2003 several research agendas have been established by means of the Dialogue Model, leading to its further development and validation (Abma and Broerse 2007; Abma and Broerse 2010). In these projects, different research funding agencies and patient organizations were involved in agenda-setting on health research. Some of these projects were financed by ZonMW, and others by charity foundations. Research agendas were formulated for spinal cord injury (Abma 2005; Abma 2006), asthma/COPD (Teerling, Caron-Flinterman et al. 2004; Caron-Flinterman, Broerse et al. 2005; Caron-Flinterman, Broerse et al. 2006), intellectual disabilities (Nierse, Abma et al. 2006; Nierse, Abma et al. 2007; Nierse and Abma 2011), diabetes (Broerse, Zweerkhorst et al. 2006), renal failure (Abma, Nierse et al. 2007; Schipper, Abma et al. 2010; Nierse, Schipper et al. 2011), neuromuscular diseases (Nierse, Abma et al.; Nierse, Abma et al. 2007), burns (Broerse, Zweerkhorst et al. 2010) and dementia (Broerse, Konijn et al. 2011) using the Dialogue Model.

The Dialogue Model comprises six phases and has an emergent design in practice. This means that not all activities are set beforehand, but are developed during the process itself in interaction with the different stakeholders. This approach enables the facilitators of the Dialogue Model to adapt activities to the needs and wishes of the involved stakeholders. Also, data and insight from previous phases can be incorporated in the following phases.

1. **Initiation and preparation.** A project team is established. Relevant stakeholder groups are defined. A first assessment is made of the problems, ideas, opinions and wishes of patients and other stakeholders, and a start is made with creating conducive social conditions. The identified stakeholders are motivated to become involved. Also, the scope of the research agenda is set.

2. **Consultation.** Various stakeholder groups are consulted separately to develop a list of issues that are relevant from the perspective of each stakeholder group. The separate consultation is needed to deal with the asymmetry between patients and professionals; patients can form their own perspective without being tempted to go along with professional opinions, and professionals can become familiarized with experiential
knowledge. Different methods can be used for consultation like focus groups or interviews, depending on what is most suitable for the stakeholders.

3. **Prioritization.** Stakeholder groups value the different research topics and rank them in order of importance, resulting in a research agenda. Often, questionnaires are used to gain insight into priorities for a large number of people in the patient group. Focus groups or the Delphi method can also be effective for ranking research topics.

4. **Integration.** Participants exchange information, address conflicts and integrate research agendas through dialogue, resulting in one joint research agenda. Often the dialogue takes place in the context of a dialogue meeting.

5. **Programming.** The joint research agenda is translated into a coherent program or action plan.

6. **Implementation.** Participants determine and take action, monitor progress and evaluate results. For example, focusing on the topics described in the research agenda in a call for proposals.

The Dialogue Model to patient participation in research agenda-setting explicates six key principles according to which the process needs to be conducted (See Box 3.1). It is expected that when these principles are taken into account, patients can meaningfully contribute to setting a research agenda.

<table>
<thead>
<tr>
<th>Box 3.1 The six key principles of the Dialogue Model</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. <strong>Active engagement of patients:</strong> Extra attention is paid to the wishes and needs of patients and their inclusion in the various phases. Ideally, patients are involved as early as possible.</td>
</tr>
<tr>
<td>2. <strong>Good social conditions:</strong> Realization of a genuine dialogue between stakeholders requires the creation of conducive social conditions, including openness, trust and respect.</td>
</tr>
<tr>
<td>3. <strong>Respect for experiential knowledge:</strong> The research methods used need to incorporate an understanding of the questions and concerns of patients into the process.</td>
</tr>
<tr>
<td>4. <strong>Dialogue:</strong> A genuine dialogue needs to ensure that participants listen to each other during the process and learn about their own and each other’s perspectives and experiences, which may eventually result in an adjustment of their opinions.</td>
</tr>
<tr>
<td>5. <strong>Emergent and flexible design:</strong> Since the issues of stakeholders cannot be known in advance, the design cannot be pre-ordained. The design emerges gradually in conversation with all parties, although the basic ground pattern and separate phases of the methodology are preset.</td>
</tr>
<tr>
<td>6. <strong>Process facilitation:</strong> Since the process should be fair, the collaboration between stakeholders is fostered by an independent process facilitator (with no stake in the content of the outcome) who creates the conditions for successful participation and dialogue.</td>
</tr>
</tbody>
</table>

Source: (Abma and Broerse 2007; Abma and Broerse 2010)
The transition experiments
Three transition experiments related to patient participation in agenda-setting were set up, implemented and analysed. All three had a different focus and learning aims since they were conducted under different circumstances and requirements. The first one aimed to set a shared research agenda in the area of congenital heart condition, the second entailed updating and extending an existing shared research agenda in the area of respiratory conditions, and the third aimed to set a research agenda for future medical products, including 15 different disease domains.

The case of JUMP

Box 3.2 JUMP

In 2007, the Dutch Heart Foundation wished to establish a new funding body, which would fund research on congenital heart conditions. The new funding body was called JUMP. JUMP had the aim to stimulate research that could contribute to the improvement of quality of life of children and adolescents with a congenital heart condition. To gain insight in what is considered quality of life, the Dutch Heart Foundation deemed it essential that children and adolescents with a congenital heart condition and parents of children with a congenital heart conditions become actively involved. Paediatric cardiologists, who conduct most of the research in this area, were also involved as important stakeholders in the process. The Dutch Heart Foundation considered it essential that a state-of-the-art research agenda was developed, to which both paediatric cardiologists as patients could relate to. The Athena Institute, VU University Amsterdam, was commissioned to set up and conduct a participatory research agenda process (Elberse, Caron-Flinterman et al. 2007). The project took place from June – October 2007. A process was developed based on the steps and principles of the Dialogue Model to establish a research agenda for congenital heart conditions.

In the initiation phase, a project team was recruited, including employers of the Dutch Heart Foundation, three paediatric cardiologists of different academic hospitals, a father of a child with a congenital heart condition, representing the patient organization Patientenorganisatie Aangeboren Hartafwijking (PAH) and two researchers of the Athena Institute, VU University. The project team was called ‘the Partnership’, and decisions were made as equal partners. An actor analysis was done to gain insight into the Dutch field and important actors. An inventory of wishes and needs for social conditions regarding the agenda-setting process were also made. A quick scan of the literature established the current and recently finished health research projects in the area of congenital heart conditions. Within the project team, the process was discussed, and there was agreement on how to continue in the next phases.

In the consultation phase, different consultation techniques were used. Thirteen semi-structured interviews with paediatric cardiologists and one group interview with five paediatric cardiologists and one thorax surgeon were held. In these interviews, current research directions
and important future research directions were discussed. Also, the expected improvement in quality of life of research directions was discussed. The researchers had the opportunity to set priorities and explain why they considered these topics a priority. Three focus group discussions at different locations with 33 parents were conducted using semi-structured exercises to stimulate needs articulation. The final exercise was to prioritize the discussed topics and questions and brainstorm on why these topics were important. In addition, three group interviews were conducted with seven adolescents. A similar approach was used as with the parents. Interviews and focus group discussions were audio-taped after receiving informed consent from the respondents and transcribed. All consulted people received a summary of the attended focus group or interview for checking. The data derived from the consultations were analyzed separately by two researchers. Clusters were formed. This phase led to the creation of lists of research topics from both the professionals and the children and parents.

Integration and further prioritization took place during a dialogue meeting with the different stakeholders – professionals in the area of paediatric cardiology (paediatric cardiologists), parents of children with a congenital heart condition and adolescents with a congenital heart condition. The aim of this dialogue was to formulate a shared research agenda in which all involved stakeholders could recognize their views and input. This resulted in a research agenda JUMP that would be used to allocate their funding.

Because the Dialogue Model had been applied several times for agenda-setting processes involving different stakeholders, this case study of the Dutch Heart Foundation, especially the dialogue meeting which took place between the professionals and patients, provided a good opportunity to study how patients could be genuinely involved in dialogue and how input from patients was taken up in detail. It is one thing to strive for patient-expert partnerships, and quite another to operationalize a genuine partnership. Experts generally are the dominant decision-makers in health research, so there is no history of equal partnership between patients and experts to build upon. The relationship between experts and patients can be described as asymmetrical due to the traditional difference in social status (Wallerstein 1999), as well as to the value assigned to the knowledge of both groups (Callaghan and Wistow 2006; Dewey 2008). The higher social status of experts is derived from their high level of education, income and prestige. While the relevant knowledge of patients is based on their subjective experience in daily life, the knowledge of experts is science based and therefore often considered to be more objective and superior (Boote, Telford et al. 2002; Caron-Flinterman, Broerse et al. 2005). This asymmetrical relationship can be expected to form an obstacle to the realisation of a genuine partnership between experts and patients. Therefore, different inclusion strategies were developed and tested to prevent the exclusion of patients and patients’ perspectives in the dialogue.

The process was considered a transition experiment, focusing mainly on deepening. This case study addressed three sub-questions:

5. Which exclusion mechanisms occur in a dialogue between patients and researchers?
6. Which inclusion strategies can be applied to overcome the exclusion of patients and patients’ input in a dialogue meeting?
7. How can a genuine dialogue be realized between patients and researchers?

To evaluate the applied inclusion strategies during the dialogue meeting, different methods of data collection were used including audio and video recordings, ad libitum observation sampling, document analysis, and semi-structured, evaluative interviews with 22 congenital heart condition care users and 8 experts. The results were grouped according to the previously specified categories: i.e. the setting (circumstances), what is done (behaviour), and what is said (verbal) and analysed using a list of exclusion mechanisms and inclusion strategies. In Chapter 5, the case – the dialogue meeting – research methods and results are described in detail.

*The Netherlands Asthma Foundation: The case of updating and extending the research agenda for asthma and COPD*

**Box 3.3 Updating and extending a research agenda**

In 2009, the Netherlands Asthma Foundation (NAF) took the initiative to update and extend their research agenda. In 2004, the NAF had already included patients and professionals in setting their research agenda (Caron-Flinterman, Broerse et al. 2005; Caron-Flinterman, Broerse et al. 2006). At that time, it was a pioneer in the area of patient participation in health research. In 2009, the NAF concluded that the agenda needed to be revised. Based on the experiences in 2004, it wanted patient participation as a structural part of their procedure for setting their research agenda. It expressed its need for a manageable and effective methodology, which genuinely involved patients with limited resource investment. It considered this a prerequisite for the structural involvement of patients in research agenda-setting. A method was needed which applied the principles of the Dialogue Model, but could be conducted in a short period of time with limited resources.

Again, the Dialogue Model was taken as a starting point. A project team was established comprising three staff members of the NAF (one of whom was also a patient), a researcher (the author of this thesis) and a senior research advisor of the VU. It was decided that the project team could make the decisions during the process after receiving feedback from the stakeholders. This implies that the level of participation of stakeholders was advisory, rather than partnership. In the consultation phase, the level of involvement was active consultation. The project started in May 2009 and ended in September 2009. The research agenda for research on asthma and COPD was extended to include four additional respiratory diseases; pulmonary fibrosis, pulmonary arterial hypertension and the respiratory aspects of cystic fibrosis and sarcodiosis.
Since the NAF already was involved in an agenda-setting procedure including patients in 2004 (Teerling, Caron-Flinterman et al. 2004; Caron-Flinterman, Broerse et al. 2005; Caron-Flinterman, Broerse et al. 2006), this transition experiment is of interest to gain insight into why the NAF wanted to imbed patient participation in their procedure of agenda-setting and how a suitable methodology could be developed for this purpose. It provided opportunities to focus on ‘deepening’ and ‘scaling up’.

This transition experiment addressed two sub-questions:

8. To what extent can an existing research agenda-setting procedure be developed, which can be implemented in the structure of a health funding agency and takes the principles of the Dialogue Model into account?

9. How do priorities of professionals and patients overlap or differ and what does this entail?

Methods for data collection involved three focus groups with patients (n=31), seven interviews with experts, a survey filled in by the members of the scientific advisory board (including 18 experts and two patients), followed by a discussion session. A web-based questionnaire among patients (n=201 of which 169 included in analysis) was used to prioritize research topics. The experiment, the research methods and results are described in detail in Chapters 4 and 6.

The case of Medical Products; New and Needed

<table>
<thead>
<tr>
<th>Box 3.4 Medical products; New and Needed</th>
</tr>
</thead>
</table>
| The Health Council of the Netherlands (GR) (Gezondheidsraad) is an independent advisory board which provides requested and unsolicited advice regarding public health policy to the Dutch Minister of Health, Welfare and Sport (VWS) (Bal, Bijker et al. 2004; Hendriks, Bal et al. 2004). In 2010, the GR was requested to give advice to VWS regarding a research agenda for future medical products. Medical products were defined as drugs, medical devices related to diagnosis and care, and tissue replacement products. It was assumed that new and innovative medical products are essential to cope with challenges in current health care, such as curbing rising costs, improving quality of care, and meeting the needs of growing numbers of elderly and chronically ill people (WHO 2010).

The appointed GR committee, comprising twelve members of whom one was a patient representative from a major patient organisation, designed an advisory project to address the request from the Minister (Gezondheidsraad 2010; Gezondheidsraad 2011). The advisory project consisted of three parts that answered the following questions:

1. What is the current position of the Netherlands in the area of medical products development? |
2. Which medical products are needed according to patients, professionals and informal carers?
3. How can development of these medical products be stimulated?

While consultation of important players in the field like scientific and industrial experts would have been more common (Bijker, Bal et al. 2009), the committee decided that involvement of patients, informal carers and health professionals was essential for establishing an agenda responsive to the needs of these ‘end-users’ regarding future medical products. An end-user is defined as someone who will consume, use or work with the medical product.

The current position of the Netherlands in the area of medical product development (question 1) was addressed by the GR. Representatives of the industry and other potential developers of medical products were consulted by the GR in a process parallel to the consultation of patients. Indications on how to stimulate developments (Question 3) were collected by the GR. Research on questions two was assigned to the Athena Institute, VU University, given their expertise in agenda-setting with patients, informal carers and health professionals.

To conduct the process of needs articulation of patient groups, informal carers and health professionals (Question 2), a project team of six researchers was established. The GR committee considered it too arbitrary and not representative to involve only one or two patients or patient representatives; therefore, it was decided that patients from a wide range of disease domains (15 disease domains) were to be consulted. In most agenda-setting projects, one disease domain is included, so the inclusion of 15 posed new methodological challenges. Based on previous experiences with public and patient involvement in research agenda-setting and interactive policy-making, a participatory approach was developed based on the Dialogue Model. It comprised four phases:

1. Exploration,
2. Consultation and prioritization,
3. Integration, and
4. Follow-up.

Patients, informal carers and health professionals were consulted using interviews and focus group discussions. All consultations were audio-taped, after receiving informed consent from the participants. A summary was sent to all participants for checking.

Actions took place between December 2009 and January 2011. On a regular basis, meetings with GR staff and committee members were organized to discuss progress and reflect on the preliminary results. In January 2011, the GR presented and published their advice. In the second part of the advice, the input provided by patients from 15 disease domains was clearly recognizable. Even more than in a background document, the input of all consulted groups was described in detail.

This transition experiment was an interesting case to learn from for two reasons. First, in almost all agenda-setting processes applying the Dialogue Model, the focus had been on a single disease
domain. In this case 15 disease domains were involved, all with their own needs, characteristics and diversity in how patient groups were organized. This posed new challenges for realizing patient participation in research agenda-setting. Second, the input from patients and carers was used in an advisory process to provide advice to the government specifically on medical products. This implied that the predefined focus on medical products was a strict boundary within which to work. This transition experiment provided the opportunity to broaden the use of the Dialogue Model towards governmental policy-making in the area of health research. In this agenda-setting procedure, patients were consulted on the level of ‘active consultation’.

This case study gave input to the deepening and broadening mechanisms of system innovation. It addressed two sub-questions.

10. How can a broad range of patient groups become genuinely involved in a scientific advisory process?
11. How can needs articulation with a highly specified focus be realized, and is this feasible and expedient according to patients?

In the preparation phase, 29 exploratory interviews were held with patient organisations. In the consultation phase, a total of 182 patients and patient representatives participated in consultation interviews (n=33) and focus groups (n=15), of which 89 were considered to be patient representatives. The process and outcomes of the needs articulation of patient groups were analysed. To gain insight into the usefulness of patient involvement according to the GR committee and staff and the Ministry of Health, interviews were conducted with the chairperson of the GR committee, a scientific secretary of the GR, and a staff member of the Ministry of Health. Informal conversations with patients and patient representatives and unrequested feedback from patients provided insight into how they experienced the process. The advice written by the GR was analysed to investigate the extent to which patients’ needs were incorporated. Furthermore, short-term activities as a result of the advice were identified. The experiment, research methods and results are described in detail in Chapter 7.

**Patient participation in the conduct of health research**

During the aforementioned transition experiments, it became apparent that patient participation in health research agenda-setting might be suitable to induce a change towards a more needs-oriented system, since the needs of patients are articulated and integrated in research agendas. However, it was also noted that little changed in how research is conducted. The experiments insufficiently addressed structural involvement in other stages of health research since patients were only involved in one stage of the research process, agenda-setting. The initiated agenda-setting projects may be considered an important first step, but they do not automatically imply
continuation of patient inclusion in decision-making networks in later stages of the health research process, i.e. it is doubtful whether they have a profound impact at the regime level. Therefore, there was a strong need for an initiative that aimed for structural patient participation in the conduct of health research projects which could serve as a transition experiment.

In search for such a transition experiment, an initiative of the Dutch League of Arthritis Patient Associations (Reumabond) was discovered: Network Patient Research Partners. This initiative aimed at the involvement of patient research partners in rheumatology research projects in the Netherlands. It provided the unique opportunity to study how patient participation can be realized in a structural manner and how it can be imbedded in the current structures, culture and practices of health research. Also, it provided the opportunity to investigate whether structural involvement in health research in the conduct may induce a shift towards research which is conducted more in line with the perspectives of patients. This initiative is innovative and quite advanced in realizing patient participation in the conduct of health research. Therefore, it provided an excellent opportunity to explore (a) how patient participation in the conduct of health research could be put into practice and (b) which facilitators and barriers are experienced by the stakeholders involved.

**Box 3.5 Network patient research partners**

The Reumabond increasingly received requests from researchers and research funding agencies to provide input on research proposals or questionnaires, judge research proposals for funding or provide help for the recruitment of study participants. The Reumabond felt that this often ‘ad-hoc’ involvement entailed the danger of tokenism and would not lead to optimal use of the patient perspective in health research. To avoid tokenism and to imbed the patient perspective more structurally in research in the area of rheumatology in the Netherlands, the Reumabond took the initiative to set up the ‘Network Research Patient Partners’ in 2008, financed by the Dutch Arthritis and Rheumatism Foundation (Reuma Fonds). It was a 3-year pilot with the aim to achieve the structural involvement of patients in rheumatology research by creating a network of so-called ‘patient research partners’. Patients who were interested in becoming involved in health research were trained to become a patient research partner (Hewlett, De Wit et al. 2006; Abma, Nierse et al. 2009; De Wit, Elberse et al. submitted). The idea is that patient research partners can be actively involved in all stages of health research. They were interlinked with researchers from a specific research project, preferably in couples, and together they formed a partnership.

The network was set up according to the FIRST model (Hewlett, De Wit et al. 2006). FIRST stands for ‘Facilitate’, ‘Identify’, ‘Respect’, ‘Support’ and ‘Training’. The model was developed within the context of research steering groups at the Academic Rheumatology Unit of the Bristol Royal Infirmary and the workshop-based conferences of Outcome Measurement in Rheumatology Research (OMERACT) (De Wit, Boonen et al. 2011). *Facilitate* refers to facilitating
the involvement of partners in team meetings. To identify partners, projects and roles, it is recommended that suitable candidates be identified through the clinic or patient organizations. Projects addressing clinical interventions, outcomes or service delivery issues could benefit the most from partner involvement. The term ‘roles’ refers to research tasks that partners fulfill. Thirdly, for a successful partnership, mutual respect is a prerequisite. Respect is associated with confidentiality and acknowledgement of the contribution of the partner. Support is defined as all actions taken to help partners to work and communicate in a successful partnership. Furthermore, training is considered essential for partners. During training, the focus should lie on basic understanding of the research process and of measuring outcomes.

The network was managed by a network coordinator. The budget included the salary and travel expenses of the coordinator, reimbursement of the expenses of the partners and two training courses. The project started with a small number of partnerships with the intention to cover all Dutch rheumatology centers at a later date. In the first tranche 12 partners were selected by the Reumabond and trained by an external agency (http://www.tools2use.eu/indexE.html). They joined research teams at the academic rheumatology centers of Leiden and Maastricht. In the second tranche, in 2009, 15 partners were selected, trained and started working in projects at the VU Medical Centre and Reade (Amsterdam) and Radboud University Medical Centre and St Maartenskliniek (Nijmegen). The partners became members of a research team, forming a partnership and contributed their experiential knowledge to a variety of research projects. In total, 16 professionals were involved.

Since the innovative approach of patient participation in health research, the VU University offered support and evaluation of the pilot by means of monitoring and analysis. In accordance with the Reumabond, it was decided that two monitors would be appointed; an external researcher from Athena Institute, VU University, and an internal researcher, who was related to the Reumabond and one of the main founders of the project (but he simultaneously conducted research on patient participation in the area of rheumatology). The researchers were supported by two senior researchers, who were also considered external experts who could provide advice and reflect on developments in the network based on their extensive experiences with patient participation.

Five additional sub-questions were formulated based on this transition experiment in order to answer the main research question.

12. How can the FIRST model be a used for setting up and guiding the involvement of patients in the conduct of health research?
13. What is the function of a patient network in realizing and embedding the structural involvement of patients in health research?
14. What are the facilitators for the structural involvement of patients in the conduct of health research?
15. What are the barriers in the current health research system to the structural involvement of patients?
16. Which strategies can be developed and applied to overcome the barriers and consolidate facilitators for embedding the structural involvement of patients in the conduct in health research?

The initiators of Network Patient Research Partners were interested in having their initiative monitored and evaluated. An active and reflexive approach was chosen (Linnan and Steckler 2002; van Mierlo, Regeer et al. 2010). This form of monitoring and evaluation interconnect analysis and support. The evaluators, called ‘monitors’, work ‘within’ a project to gather in-depth, inside data and observe and question practices (Hoes 2011). An emergent research design was followed: data from earlier phases formed the input for validation and discussion in later phases. This approach enabled the monitors to collect data using mixed methods, and to provide immediate advice and support to participants when needed. The insights of the monitors were used to directly improve the guidance of participants; the monitors were available to solve problems, identify flaws in the network, guide and support participants if partnerships were at stake, and assist in improving collaboration. Data collection took place using a variety of qualitative and quantitative research methods.

In Chapter 8, the effectiveness of the FIRST model is analyzed, addressing sub-questions 8 and 9. In Chapter 9, the facilitators and barriers related to the current culture, practices and structures of health research in the area of rheumatology experienced by the partners and researchers are described based on a system innovation perspective. Strategies developed by the monitors to consolidate facilitators and overcome barriers are also described. Chapter 9 addresses sub-questions 14-16.

**Applying steering notions**

Although it is important to pay attention simultaneously to the steering notions ‘deepening’, ‘broadening’ and ‘scaling-up’, during all transitions experiments described in this thesis, the focus lied on one main steering mechanism. In ‘JUMP’ (Box 3.2), ‘Updating and extending the research agenda’ (Box 3.3) and ‘Network patient research partners’ (Box 3.5), the main focus was on deepening, because we wanted to gain detailed insight into the processes taking place. In these cases we wanted to learn as much as possible in a specific context. From these insights, lessons were learned which were applied in other experiments. Based on these transition experiments it was considered if a proof-of-principle could be established. Furthermore, attention was paid to shaping and aligning expectations of different parties involved. In ‘Medical products’ (Box 3.4) we focused on broadening, applying the knowledge of the Dialogue Model on setting a research agenda for a specific area of research, including 15 disease domains, instead of one specific disease domain. The Dialogue Model was tested in a different context. In ‘Updating and extending the research agenda’ (Box 3.3), besides attention on deepening, there was also attention for scaling up. It was the explicit goal of Netherlands Asthma Foundation to develop a comprised
method which could be embedded as general agenda setting procedure of the Netherlands Asthma Foundation.

Validity

Multiple strategies have been used within this study to minimize the effects of researcher bias and influence and to enhance the validity of the results and conclusions. The strategies comprise the use of:

a. Rich data: Throughout the research the primary data was extensively documented. Interviews and focus groups were audio-taped and transcribed. Extensive notes were made of attended meetings including date, aim and participants, and other observations.

b. Triangulation: A variety of methodologies was used to collect data to reduce the limitations of a specific methodology and researcher bias. In setting up and conducting the research, more researchers were involved (see Box 3.6, research teams). In the analytical elements of the study, multiple researchers were involved. Coding and agreements on coding schemes were done by at least two researchers.

c. Member checks: These entail systematically asking for feedback on data and conclusions from the people who were involved. After focus group discussions and interviews, a draft report was sent to the participants to check if the researchers correctly interpreted the input and to confirm its accuracy. In this way, mistakes and misunderstanding by the researchers were minimized.

d. Continuous reflection: During the research continuous reflection with colleagues, peers and project participants was organized to reduce researcher bias.

e. External advisors: In the case of Network Patient Research Partners, two senior advisors who had sufficient distance from the project were appointed. They provided solicited and unsolicited advice and reflection.

f. Saturation: In the project, saturation of the data was sought as much as possible. Interviews and focus group discussions were preferably conducted until no new issues came up.

The research teams

The research teams involved in the projects described in this thesis varied in composition. Overall, the author of this thesis was involved in the teams as the main executing researcher. Jacqueline Broerse was involved in the management and supervision of the research projects and other activities related to my work. In Box 3.6 a list of the researchers involved per project is provided.
### Box 3.6 Research teams

#### JUMP (Box 3.2)
In the case of JUMP the research team comprised:
- Janneke Elberse (Researcher at Athena Institute, VU Amsterdam)
- Francisca Flinterman (at that time, researcher at Athena Institute, VU Amsterdam)
- Tjard de Cock-Buning (Prof. at Athena Institute, VU Amsterdam)

Janneke and Francisca were responsible for the design and execution of the project. Tjard was involved as advisor and facilitator of the dialogue.

#### NAF (Box 3.3)
In the case of updating and extending the research agenda, the research team comprised:
- Pim de Boer (Head of research department, NAF)
- Janneke Elberse (Researcher at Athena Institute, VU Amsterdam)
- Dorothee Laan (Research department NAF)
- Truus Teunissen (Employee ‘patient participation’ NAF).
- Tjard de Cock-Buning (Prof. at Athena Institute, VU Amsterdam)
- Jacqueline Broerse (Prof. at Athena Institute, VU Amsterdam).

Janneke and Dorothee were responsible for conducting the research. Pim was head of the research team. Truus, Tjard and Jacqueline were involved as advisors.

#### Medical Products (Box 3.4)
In the case of setting the agenda for medical products, the research team comprised:
- Carina Pittens (Researcher at Athena Institute, VU Amsterdam)
- Janneke Elberse (Researcher at Athena Institute, VU Amsterdam)
- Janine van de Kraats (at that time, researcher at Athena Institute, VU Amsterdam)
- Marjolijn van Wijk (at that time, intern at Athena Institute, VU Amsterdam)
- Tjard de Cock-Buning (Prof. at Athena Institute, VU Amsterdam)
- Jacqueline Broerse (Prof. at Athena Institute, VU Amsterdam).

Carina, Janneke, Janine and Marjolijn were responsible for conducting the research. Tjard and Jacqueline were involved in the facilitation, analysis and reporting. Jacqueline was project leader.

#### Network Patient research partners (Box 3.5)
In the research project ‘Network Patient Research Partners’ the research team consisted of:
- Janneke Elberse (Researcher at Athena Institute, VU Amsterdam)
- Maarten de Wit (Researcher, EMGO+, VUmc/ External representative of the Reumapatiëntenbond)
- Jacqueline Broerse (Prof. at Athena Institute, VU Amsterdam).
- Tineke Abma (Prof. at EMGO+ Metamedica VUmc)

Maarten and Janneke were appointed as ‘monitors’ to monitor and evaluate the network and conduct the research. Jacqueline and Tineke were involved as external advisors.
Outline of the thesis

In Chapter 1, introduction I discussed the aim of the inducement of the study and the theoretical concept (patient participation in health research, transition theory and steering notions) relevant in this thesis. In Chapter 2, theoretical background, I explained important concepts used in this thesis. In Chapter 3 research design, the objectives of the study are posed, as well as the main research question. The method used to address the objectives is described in detail, including descriptions of the transition experiments. Chapter 4 describes the health research system. In addition, two pioneers who experiment with patient participation in the Netherlands, several other initiatives and barriers encountered for patient participation in health research due to features of the system are discussed. Furthermore it describes how the steering notions ‘deepening, broadening and scaling up’ can be used in light of a system innovation. In Chapter 5, the dialogue of the JUMP casus is presented focusing on how a genuine dialogue between patients and professionals can be enhanced. Several inclusion strategies are developed and applied to overcome exclusion mechanisms. Additional inclusion strategies are suggested. Chapter 6 focuses on the updating and extending of the research agenda of the Netherlands Asthma Foundation. A condensed method was developed based on the Dialogue Model to update the existing research agenda. The lessons learned are discussed. In Chapter 7, a process to articulate for patients’ needs for medical products is described. The aim of the process was to set a research agenda for future medical products for 15 disease domains. The needs articulated by patients were used as input for an advisory process of the Health Council of the Netherlands. Results and encountered dilemmas are discussed. Chapter 8 analysis the extent to which the FIRST-model is effective guiding for establishment of a network of patient research partners who form partnerships with researchers to become structurally involved in ‘doing research’. Achievements and pitfalls are described. Chapter 9 scrutinized the same network to gain insight in facilitators and barriers for structural involvement of patients in ‘the conduct of research’. Strategies are proposed to enhance the participation of patients in the conduct of research. In Chapter 10, I draw conclusions based on the different transition experiments to answer the main research question. In addition, results are discussed and recommendations for future research are made. At the end of the book, an English and Dutch summery are included.
Part 2. Into the system
Chapter 4 Towards a needs-oriented health research system

Introduction

In this chapter the focus will be on the health research system and its system innovation from a supply-driven towards a needs-oriented system by means of active patient involvement.

The function of the health research system is to generate knowledge through ‘scientific inquiry’ focusing on wellbeing and improving the health of people (Pang, Sadana et al. 2003). It spans the entire range from biomedical research, clinical research, public health research, epidemiological research to care research. The health research system is located at the intersection of the health system and the general research system. It contributes to the health care system by generating knowledge and innovations (Hanney and Gonzalez Block 2006). Research systems rely on particular research methods and techniques that are accepted as ‘scientifically’ valid and reliable within the contemporary presiding paradigm. The health research system comprises a wide variety of actors that interact in a stabilized manner, following standard procedures and actions, and sharing scientific paradigms. The most important actors are researchers and research institutes, health professionals, national funding agencies, charity foundations, policymakers, the pharmaceutical industry, patient organizations and individual patients (Caron-Flinterman, Broerse et al. 2007). In the incumbent regime of health research, patients and patient organizations are not considered powerful actors playing an ‘active’ role in health research; patients are ‘objects of study’. The health research regime is basically supply-driven: the ideas and interests of the knowledge and innovation suppliers – researchers and the pharmaceutical industry – are driving the research process.

Although the knowledge generated by health research has led to considerable improvements in quality of life and a longer and healthier lifespan, the health research system is also criticized. Newly developed health innovations do not always address relevant problems in current health practices, nor do they effectively meet the needs of patients (Chalmers 1995; Grant-Pearce, Miles et al. 1998; Tallon, Chard et al. 2000; Corner, Wright et al. 2007; Serrano-Aguilar, Trujillo-Martín et al. 2009; Opar 2010; Petit-Zeman, Firkins et al. 2010). Mismatches have been identified between what is scientifically known and investigated and what patients would like to know about their disease or treatment, or would like to be treated for (Liberati 1997). For example, people with rheumatoid arthritis indicate that fatigue is an important issue for them, but hardly any research is done addressing this issue (Kirwan, Hewlett et al. 2005). In another case, research in the area of dementia is very much focused on treatment and medication while little is known about how to
live with dementia in dignity. Also, most tests for dementia focus on developed limitations, like not being able to write or read anymore, instead of still existing possibilities. This is experienced as negative and burdensome (Broerse, Konijn et al. 2011). The methods used in health research are not always considered ‘patient-friendly’ because they are too invasive or painful in relation to possible benefits. Moreover, innovations are developed for a ‘general population’ rather than being focused on individuals like personalized medicine. In randomized clinical trials for example, interventions are often tested on a ‘homogenous, standardized’ group of patients. Interventions, prescriptions and other innovations are therefore not necessarily suitable for each individual (Schuitmaker 2010). Questions like how will treatments affect the elderly (Cherubini, Oristrell et al. 2011; Gurwitz and Goldberg 2011), pregnant women or children (Burns 2003; Pandolfini and Bonati 2005) are often not addressed. These mismatches seem to be rooted in certain features of the health research system that have so far been the cornerstone of its success. These features have led to preferences for certain topics or domains and specific methodologies, while not taking into account patient perspectives, resulting in knowledge gaps. The focus of research does not always reflect the needs of patients or other stakeholder groups like care providers.

In the last three decades, patients have obtained more influence in the decision-making processes in the health care domain, and now this development is also becoming apparent in health research. Patients are demanding a stronger voice within health research (Caron-Flinterman 2005; Schipper 2012), and there is growing support for the idea that the involvement of patients in health research could lead to a more needs-oriented focus, addressing their needs and wishes. Taking into account the mismatches in health research described above, it would be interesting to investigate if this more active role for patients could induce a system innovation towards a more needs-oriented health research system.

Patient participation in health research can be defined as patients being actively involved in and having an influence on decision-making processes in health research (Elberse, Caron-Flinterman et al. 2011). Usually, various levels of patient participation are distinguished, ranging from providing information to researchers about their disease or experiences to being an influential counterpart (Abma, Nierse et al. 2009). Patients’ experiential knowledge acquired by living and coping with an illness, sickness and/or disease, can complement scientific knowledge, resulting in better informed decisions (e.g. (Popay, Williams et al. 1998; Nordin 2000; Telford, Beverley et al. 2002; Caron-Flinterman, Broerse et al. 2005). Patient participation may increase the levels of social support for and acceptance of research, and enhance legitimacy (Whitstock 2003). It is also argued that patients have the right to be consulted in decisions that directly affect their lives. In principle, patients could be involved in all phases of the research process, from agenda-setting to implementation and utilization of the results (Abma, Nierse et al. 2009). Various scholars emphasize that involvement throughout the research process will lead to the highest impact (Hanley, Bradburn et al. 2003; Minogue, Boness et al. 2005; Hewlett, De Wit et al. 2006; ZonMW
2006; Wyatt, Carter et al. 2008). Patient participation will lead to a redefinition of power relations between patients, researchers and other important players in the field (Williamson 2001; Beresford 2007; Lindenmeyer, Hearnshaw et al. 2007; Ward, Thompson et al. 2010).

There are different societal trends supportive of a more needs-oriented health research system. First of all, the changing role of patients in the health care system; patients have become increasingly more outspoken and empowered over the past three decades (Traulsen and Noerreslet 2004; Barbot 2006; Epstein 2008; Williamson 2008). Various patient organizations and representatives advocate for their active involvement in care, research and policy (Williamson 2010), and this involvement is increasingly recognized as important by other actors (Baggott and Forster 2008). Particularly with respect to health care services, patients have become more actively involved through client advisory boards and client satisfaction surveys on the quality of care facilities as well as shared decision-making on individual treatment (Blume and Catshoek 2003; Elwyn, Frosch et al. 2010; Van de Bovenkamp, Trappenburg et al. 2010; Brown, Butow et al. 2011). Second, trends like ‘democratization of science’, ‘increasing societal demand for utilization of knowledge’, and ‘public accountability’ (Frodeman 2000; Fuller 2000; Nowotny, Scott et al. 2001; Chopyk and Levesque 2002; Collins and Evans 2002; Scott 2007) may put pressure on the health research system to become more needs-oriented.

In numerous countries, patients are increasingly involved in health research. Especially the UK seems to be a front runner; developments started there already in the mid-1990s. In 1996, INVOLVE was established with funding from the National Institute for Health Research (NIHR). It is a national advisory group which stimulates and supports active patient participation in care, public health and research (Hanley, Bradburn et al. 2003). The involvement of patients and the public is institutionalized in the UK, where the idea of patient participation is already more accepted than elsewhere. In the UK, different actors like charity funds, research institutions and patient groups are experimenting with patient participation to gain insight into effective methods and appropriate settings. Initiatives can be seen in other countries like Canada, Sweden (Keizer and Bless 2010; Kjeken, Ziegler et al. 2010), Italy (Mosconi, Colombo et al. 2007), France (Vololona 2003; Barbot 2006) and especially Australia (Saunders and Girgis 2010; Payne, D’Antoine et al. 2011). The Netherlands seems to be an important player since relatively many experiments with various approaches are taking place there. In this chapter we will focus on the Dutch situation. Different actors in the Netherlands have taken up the challenge of realizing patient participation in health research. They have set up experiments because there is (1) a lack of knowledge of effective methods, (2) no database of best practices where knowledge is aggregated and analyzed, and (3) uncertainty about how to realize a more needs-oriented health research system. These experiments provide insight into effective methods for patient participation and how a system innovation can be induced.
In the Netherlands different ‘transition’ experiments are being conducted, which provide the opportunity to learn about the potential for patient participation in health research and possible future directions for a system innovation. This chapter presents the activities of one of the pioneers in patient participation in health research in the Netherlands: the Netherlands Asthma Foundation. Also another important pioneer is presented: ZonMw. This is followed by a discussion of the various other activities involving patient participation in health research in the Netherlands as a manifestation of niche-level initiatives that might lead to a system innovation. Furthermore, the most important features of the health research system are described based on literature study, document analysis, interviews, informal conversations and observation. Many niche-level initiatives encounter the regime features that subsequently act as barriers to the structural involvement of patients in health research. Various strategies based on insights from transition management studies will be discussed that may provide a way forward, including processes of ‘deepening’, ‘broadening’ and ‘scaling up’.

The Netherlands Asthma Foundation as pioneer of patient participation

The Netherlands Asthma Foundation (NAF) is considered one of the pioneers of patient participation in health research in the Netherlands. It was founded in 1960 and is a research-funding agency as well as patient organization. The NAF advocates for the interests of people with asthma and chronic obstructive pulmonary diseases (COPD). Recently, they extended their focus to lung conditions in general, including ‘orphan lung diseases’, which will eventually lead to a name change into the ‘Lung Foundation’. The NAF funds scientific research in the area of lung conditions and stimulates the improvement of care for people with a lung disease. They are financed by donations, and on average 25% of their income is spent on health research, which is around 4 million Euros per year. The NAF is an important funder of lung-related research in the Netherlands. It is one of the biggest health funding agencies in the Netherlands. Its patient section fights for patients’ rights, providing information on lung diseases to patients and citizens and trying to stimulate awareness of lung problems. There is a strong relationship between these two sections. Because of the combination of funding agency and patient organization, the NAF has an extensive network including both researchers and patients.

In 2003, the program managers for research and patient participation suggested that it could be important to involve patients more in agenda-setting for three reasons. It was assumed that patient participation could result in a broader perspective on lung research due to their experiential knowledge; it could provide insight into their needs and ideas, leading to a more needs-oriented focus. Second, the NAF wanted to investigate the extent to which they had spent research funding that corresponded to the needs of patients. Furthermore, it suspected that the connection between the patient organization and funding agency could be strengthened by
involving patients in research agenda-setting. At the same time not much was known about how to realize patient participation in health research decision-making. The research agenda of the NAF had previously been set by the scientific advisory board in consultation with leading researchers and health professionals (Caron-Flinterman, Broerse et al. 2005). For the 2005-2008 research agenda the NAF wanted to include the perspectives of patients as well as health professionals and researchers.

Since no methodology was available, the NAF contacted the Athena Institute of the VU University, Amsterdam, where experience was available on involving resource-poor farmers in research agenda-setting through the so-called Interactive Learning and Action (ILA) approach (Broerse and Bunders 2000; Roelofsen, Broerse et al. 2008; Swaans, Broerse et al. 2009). Researchers from the VU adapted the previously developed methodology to the context of patient participation in research agenda-setting. The project was considered an experiment by all partners involved, involving testing of the adapted methodology.

The agenda-setting project took place from July 2003 to June 2004. In the ILA approach, research topics are identified and priorities set according to the frames of reference of researchers but developed in interaction with various stakeholders, in this case patients, researchers and health professionals. Each of the different stakeholders is assumed to have their own perspective, and all these perspectives are relevant for addressing the issues at hand. Enhancing a dialogue about relevant topics among and between stakeholders will lead to personal and mutual understanding of the different contextual perspectives and experiences. This will result in a better informed and more needs-oriented research agenda. The approach comprises six phases (Abma and Broerse 2010) of which the first four phases were mainly executed by VU University and the last two phases by the NAF:

1. **Initiation and preparation.** A project team is established. A first assessment is made of the problems, ideas, opinions and wishes of patients and other stakeholders, and a start is made in creating conducive social conditions.

2. **Consultation.** Various stakeholder groups are consulted separately using interviews and focus groups to develop a list of issues that are relevant from the perspective of each stakeholder group. The research topics are grouped and interactively evaluated by the stakeholders during a plenary session.

3. **Prioritization.** Stakeholder groups value the different research topics and rank them in order of importance, resulting in a research agenda.

4. **Integration.** In a carefully structured dialogue meeting, participants exchange information, address conflicts and integrate research agendas, resulting in one joint research agenda.

5. **Programming.** The joint research agenda is translated into a coherent program or action plan.
6. **Implementation.** Participants determine and take action, monitor progress and evaluate the results.

This approach follows different principles to create an appropriate setting to include patients. In Box 4.1 these principles are described.

<table>
<thead>
<tr>
<th>Box 4.1 The six key principles of the Dialogue Model</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. <strong>Active engagement of patients:</strong> Extra attention is paid to the wishes and needs of patients and their inclusion in the various phases. Ideally, patients are involved as early as possible.</td>
</tr>
<tr>
<td>2. <strong>Good social conditions:</strong> Realization of a genuine dialogue between stakeholders requires the creation of conducive social conditions, including openness, trust and respect.</td>
</tr>
<tr>
<td>3. <strong>Respect for experiential knowledge:</strong> The research methods used need to incorporate an understanding of the questions and concerns of patients into the process</td>
</tr>
<tr>
<td>4. <strong>Dialogue:</strong> A genuine dialogue needs to ensure that participants listen to each other during the process and learn about their own and each other’s perspectives and experiences, which may eventually result in an adjustment of their opinions</td>
</tr>
<tr>
<td>5. <strong>Emergent and flexible design:</strong> Since the issues of stakeholders cannot be known in advance, the design cannot be pre-ordained. The design emerges gradually in conversation with all parties, although the basic ground pattern and separate phases of the methodology are preset</td>
</tr>
<tr>
<td>6. <strong>Process facilitation:</strong> Since the process should be fair, the collaboration between stakeholders is fostered by an independent process facilitator (with no stake in the content of the outcome) who creates the conditions for successful participation and dialogue</td>
</tr>
</tbody>
</table>

Source: (Abma and Broerse 2007; Abma and Broerse 2010)

The execution of this experiment on research agenda-setting is extensively described by Caron-Flinterman et al. (Caron-Flinterman, Broerse et al. 2005; Caron-Flinterman, Broerse et al. 2006) and Teerling et al. (Teerling, Caron-Flinterman et al. 2004). This experiment showed that patients are able to articulate their main problems and needs, and can prioritize research topics in a well-founded way in a facilitated process. Patients also suggested new research topics which were not mentioned by the other stakeholders, such as co-morbidity and interaction between medications. They did not just prioritize care research, social research or focus only on their own problems as was expected by some researchers; they prioritized bio-medical research topics and thought of future generations. Finally, the topics in the research agenda were divided into three levels of priority (high, medium and low), resulting in a research program.

The programming phase was initially delayed due to some controversy over the implications of the research agenda for the structure of the NAF research program. The research agenda was used from 2005 until 2009. Although not yet systematically evaluated, it seems that the topics
indicated as important by patients, such as co-morbidity and drug interaction, were not taken up by the research community; no project proposals on these topics were submitted.

Despite the problems encountered in the programming and implementation phases, the NAF considered patient involvement important and beneficial. In 2008 the research agenda was regarded as ‘expired’, and it was decided that it should be updated and extended. Besides asthma and COPD, the NAF now includes rare chronic lung diseases (pulmonary fibrosis, pulmonary arterial hypertension and the respiratory aspects of cystic fibrosis and sarcodiosis). Again, the research agenda had to highlight important areas of research, reflecting both the priorities of patients as well as those of researchers (state-of-the-art science). Since the first agenda-setting project took considerable time and effort, it was deemed necessary to develop a less time-consuming and cheaper version of the methodology. The aims for this new version according to the NAF were to make the perspectives of patients, researchers and physicians visible in a workable research agenda and to realize a justifiable level of patient participation. The different steps as described in the ILA approach were followed in the new version as well.

During the initiation phase (2009), a project team was established comprised of three staff members of the NAF (one of whom was also a patient), a researcher and a research advisor of the VU. Due to time constraints, it was decided that the project team could make the decisions during the process after receiving feedback from the stakeholders. This implies that the level of participation of stakeholders was advisory.

In the consultation/prioritization phase, different methods were used to gain insight into the current priorities on research topics. A questionnaire was sent to members of the scientific advisory board (20 researchers/health professionals and two patients) in which they could indicate which topics of the former agenda should be kept, changed, or removed or new ones added. The priorities on rare lung diseases were obtained from semi-structured interviews with the researchers and physicians. This input was discussed together with the questionnaire in a plenary session with the scientific advisory board. During this meeting, the former research agenda was scanned, updated, validated and prioritized. Topics which were considered less relevant due to new knowledge were removed, while emerging topics were added to the list.

To assess the problems, needs and perspectives of patients, three focus groups were organized (31 people with different lung conditions). After documenting their problems and needs in the first exercise, the topics were prioritized to gain insight into the arguments and rationale behind the priority-setting. After analysing the results of the focus groups, 30 research topics remained and were clustered in seven different themes, such as causes, disease development, and intervention. Subsequently, a web-based questionnaire was developed for the patient stakeholder group to validate and prioritize the research topics (165 respondents). The results showed that
patients suffering from different lung diseases indicated topics specifically relevant for their lung disease along with general topics like disease causes and development. For example, people suffering from lung fibrosis assigned high priority to the reduction of the side-effects of Prednisone, while people suffering from pulmonary hypertension indicated improving the method of administering medication as important.

In the integration phase, the project team evaluated and compared the perspectives from the different stakeholders. Two separate agendas were formulated: one for asthma/COPD research and one for research on the rare chronic lung diseases. The two agendas were sent to the involved people to check for completeness. Finally, the scientific advisory board of the NAF examined the agendas for feasibility from a scientific perspective.

The development and execution of this adapted process were realized in a time span of only 5 months. All groups were actively involved. Patients were satisfied with this approach and considered focus groups a useful method to gain insight into their experiential knowledge and to provide the opportunity to meet and share personal stories (peer support). Within the two agendas, the overlap between research themes was substantial with respect to basic, translational and applied research. In the details, the priorities of patients and professionals differed (Elberse, Laan et al. 2012). Interestingly, also during this second experiment, differences were observed with respect to the topics of co-morbidity and drug interactions, which were given high priority by patients, while again researchers did not prioritize these topics. The topics of side-effects of drugs, unpleasant administration techniques and specific drugs for children were introduced by patients. This may be explained by differences in the frameworks of patients and professionals. While professionals are often highly specialized, focusing on part of the respiratory condition, patients have a more contextualized focus and consider ‘co-morbidity’ as part of their condition. However, the NAF has indicated that funding research in relation to drug development does not fall within their mandate; it is considered the domain of pharmaceutical companies. Other differences were observed with respect to the topics ‘smoking’ and ‘stop smoking’. These topics were prioritized by health professionals, while patients and researchers indicated that they did not consider these topics relevant. Patients felt that it was not necessary to fund more research on ‘smoking and stop smoking’ because they already know that smoking is bad. Some had decided to quit smoking already, while others decided not to stop despite the known risks. They felt more research and knowledge would not convince those who still smoked to quit. According to some professionals, great benefits can be obtained for respiratory conditions if people stop smoking (or don’t start).

The NAF is not only a pioneer in the area of involving patients in research agenda-setting, it has developed other initiatives in the area of patient involvement. Most of them are developed and improved by a process of ‘trial and error’ since hardly any guidance can be found in the literature. First, the NAF has changed its guidelines for proposal writing. When researchers now apply for
funding, they need to submit a 'lay summary' so that patients are capable of judging the proposals. This summary should state the relevance of the research for patients and if and how patient participation will be realized in their research. Different patients judge the summaries for relevance, burden vs. benefits, possible impact on quality of life, intelligibility of information, and the level of involvement of patients. The judgment of the patients is taken into account when funding decisions are made.

Second, a pool of about 21 patients has been established by the NAF. Patients receive a 2-day training course on patient participation to prepare them to engage in research and gain insight into how researchers work and think. They receive basic knowledge of evidence-based medicine, ethics and research methodologies. They learn how to influence the research process in different phases and are trained in communication skills to collaborate with researchers in a constructive manner. Also, methodologies to consult other patients to generate a more broadly shared or intersubjective ‘patient perspective’ are learned. Furthermore, the NAF provides follow-up training sessions focusing on specific competences also needed to engage in health care and research policy. These patients are being asked to act as advisors or evaluators on scientific or research advisory boards developing guidelines in respiratory care, project-related ethics boards, and advisory boards for the improvement of care, or as patient representatives during congresses. By training patients, NAF aims to establish a group of ‘professional patients’ who are empowered and competent to become an accepted voice in decision-making processes, advocating the needs of patients. It is assumed that professional patients who are capable of integrating individual patient stories into a more broadly shared patient perspective will be more easily accepted as an equal partner in the field.

Third, the NAF participates in various international projects in which patient involvement plays a role. Examples include PROactive and U-BIOPRED in the Innovative Medicines Initiative (IMI). The main aim of PROactive is to develop Patient Reported Outcomes (PROs) measuring how people with COPD experience physical activities. PROs can be used in clinical trials as outcome measures. In order to develop PROs, patients are involved on the level of active consultation to provide insight into essential elements regarding physical activities. PROactive also established an ethics board that included patients from the different European countries involved. Independently of the professional care members, these patient members follow the initiation and progress of the project and its outcomes closely from an ethical and safety point of view. U-BIOPRED focuses on different types of severe asthma and aims to find biomarkers for this disease with the hope that this understanding will consider individual characteristics and be used for further therapy-related investigations leading to personalized medicine. The role of patients as active partners is similar to those in PROactive. In both projects patients provide advice on communication and dissemination aspects.
Fourth, the NAF tries to make patient involvement visible for the research community by presenting its patient involvement initiatives at scientific congresses and via publications (Laan, Elberse et al. 2010; Laan, Elberse et al. 2010; Elberse, Laan et al. 2012). During its annual scientific symposium, the NAF strongly promotes active patient involvement in scientific lung research. Examples are presented to show researchers the benefits of active patient involvement (AstmaFonds 2008; AstmaFonds 2009). It lobbies for more patient involvement nationally as well as internationally. It participates in discussions with patient organisations, industry, insurance companies, research organisations and funding agencies.

The vision of the NAF with respect to patient participation is that it should become ‘normal practice’. Therefore, it experiments with and lobbies for patient participation in research. As described above, the NAF undertook different experiments, learning and improving along the way. It developed forms of patient participation when no appropriate methodologies were available. It has assumed a leading role especially related to health funding agencies in the Netherlands. Several funding agencies ‘copy’ initiatives, e.g. the Burns Foundation, by involving people with burns in research agenda-setting, and the Dutch Cancer Society (Koningin Wilhelmina Fonds, KWF Kankerbestrijding), by involving patients in funding procedures.

ZonMW

The Organisation for Health Research and Development (ZonMW) is also an important player in the field of patient participation in health research in The Netherlands. ZonMW finances health research varying from basic research to prevention or care research. It is mainly supported by the Dutch Ministry of Health, Welfare and Sports (VWS). ZonMW applies different approaches for enhancing patient participation in research.

Involvement of patients is not the general policy within ZonMW, and the decisions concerning this are left to the program secretaries. Currently, there are around 80 program secretaries, and patient participation is realized in about a quarter of the programs. Improvements could be made especially in the more biomedical oriented programs (Van Bijsterveldt and Van Mechelen 2011). Recently, ZonMW organized seminars for program coordinators and secretaries to provide more insight into patient participation and create more enthusiasm. Also, workshops are held to develop more knowhow and stimulate the employees to involve patients in their programs.

Several program secretaries have decided to involve patients in the agenda-setting procedure for their program, e.g. mental disabilities (Nierse, Abma et al. 2006; Nierse and Abma 2011) and Health Technology Assessment and spinal cord injuries (Abma 2005; Abma 2006). In most cases, relevant patient organizations were consulted in these initiatives. Although the input of patients
was not always clearly visible in the programs, the initiatives contributed to the understanding of how to involve patients.

ZonMW is also experimenting with an adapted procedure for the ‘call for proposals’, whereby patient participation within a research project is a condition for getting funding in some programs. The level of patient participation during the proposed research project needs to be indicated by the applicant. ZonMW made this change in the hope that researchers would consider patient participation when applying for a grant. Although using patient participation as a strict criterion for funding has proven to be difficult, it does force researchers to think about it. Researchers indicate that they are indeed stimulated to consider patient participation but meanwhile do not feel pressured by ZonMW because there are no additional budgets available to involve patients in their research.

In some programs, patients help appraise the project proposals. ZonMW established a patient advisory board which can be asked to review proposals. Initially, patients were asked to review complete proposals, containing much scientific terminology, often in English, in a short amount of time. This was unrealistic, and they indicated that they could not form a good judgment on the basis of the material provided and on such short notice. Nowadays, applicants are requested to write a summary in layman’s terms, including only essential elements of the proposal. Also, the number of proposals per person has been reduced. The process is regularly evaluated and improved.

In 2009 ZonMW started the special program ‘Patient participation in care, research and policy’ with the aim to stimulate research on patient participation to gain insights into its experiences, methods, effects, impact and criteria. It wants to stimulate the evaluation of the different initiatives, foster structural implementation of patient involvement in these three areas, and create awareness of the importance of patient involvement by the different actors (Van de Bovenkamp, Grit et al. 2008; ZonMW and VSBfonds 2009). It aims to improve the quality and relevance of health care, research and policy by applying lessons learned in this program. In total, a budget of 2.7 million was available for the program.

Besides the different approaches to realizing patient participation in health research, ZonMW tries to connect people with experiences with, or interest in, patient participation in health research and to stimulate mutual learning. By organizing annual symposia on patient participation in research for funding agencies, researchers and patients, different stakeholders have the opportunity to meet each other and to discuss methods, results and lessons learned. This facilitates the creation of alignment between different stakeholders and contributes to the development of shared visions and goals. It provides the opportunity for different initiatives to become more visible and to establish a network. Since the start in 2003, the number of
participants at this annual symposium has been growing steadily, indicating that an increasing number of people have become aware of, and are interested in, patient participation in health research. It should be noted, however, that the symposium is rarely attended by health and biomedical scientists.

**Other initiatives in the Netherlands**

Besides the NAF and ZonMW, there are other important players in the field. Several other funding agencies are also experimenting with patient participation in their practices, albeit on a smaller scale. Different funding agencies have involved patients in establishing their research agenda, e.g. the Burns Foundation (Broerse, Zweekhorst et al. 2010), the Diabetes Foundation (Broerse, Zweekhorst et al. 2006), the Kidney Foundation (Abma, Nierse et al. 2007), the Heart Foundation (Elberse, Caron-Flinterman et al. 2007; Elberse, Caron-Flinterman et al. 2011), and the Dutch Alzheimer Society (Broerse, Konijn et al. 2011) or have included patients in their scientific advisory board (e.g. Diabetes Foundation, the Kidney Foundation and the Dutch Arthritis and Rheumatism Foundation).

Among patient organizations, the Dutch Arthritis Patients’ League (RPB) plays a leading role in patient participation. It aims to realize a more structural and sustainable involvement of patients in research. Therefore, it set up the ‘network patient research partner’ in 2008, financed by the Dutch Arthritis and Rheumatism Foundation. Patients are being trained to become a ‘patient research partner’ (Hewlett, De Wit et al. 2006; De Wit, Elberse et al. submitted) who can be actively involved in all stages of health research. This network is realizing a level of partnership between researchers and patients. This initiative is innovative and quite advanced, although collaboration between the patient research partners and researchers remains challenging (Elberse, De Wit et al. 2009; De Wit, Elberse et al. submitted).

Activities concerning patient participation can also be witnessed in the area of clinical trial research and pharmaceutical companies’ practices, e.g. Duchene Parents’ Projects and PatientPartner. PatientPartner is a partnership between different European organizations funded by the European Commission, which focuses on ‘promoting the role of patients in the clinical trial context’ (Smit 2009; PatientPartner 2011). The KWF and BOOG Study Centre (Borstkanker onderzoeksgroep, BOOG) are experimenting with the active involvement of patients in the set-up and execution of clinical trials. The KWF financed a pilot study in 2011 to involve women with breast cancer on the level of consultation and advice. Since little information was available on the experiences of patients in clinical trials and patient involvement in breast cancer clinical trials, a qualitative study was conducted to explore the possibilities for more active involvement. The study was a collaboration between KWF, BOOG, and the Breast Cancer Patient Organization
(Borstkankervereniging Nederland, BVN) and was executed by Athena Institute, VU University, Amsterdam (Broerse, Pittens et al. 2012; Pittens, Elberse et al. 2012). Along with interviews and focus groups with people with breast cancer, clinicians and nurses, an advisory board of 6 female breast cancer patients (former) was established. Under supervision of a researcher, they wrote an advisory report on the possibilities for active involvement in breast cancer clinical trials during three meetings. Input from a literature study, exploratory interviews and focus groups of people with breast cancer, clinicians and nurses, complemented with personal experiences, formed the main input for the advice. Additional attention was paid to implementation of the advice and the attitudes of clinicians and other stakeholders towards active patient involvement.

Looking at the various activities undertaken by funding agencies, patient organisations and others, it becomes evident that most concern patient participation in research agenda-setting. The approach is taken in different settings (funding agencies, advisory bodies, patient organizations). It is not surprising that this approach is often employed to realize more patient involvement by different actors (Abma and Broerse 2010). Firstly, there is a thoroughly evaluated methodology for agenda-setting available. Secondly, the different charity foundations, organized in the Collaborative Health Foundations, regularly meet and inspire each other. Thirdly, setting a research agenda seems to be an isolated, clearly defined step in the research process. The process has a clear span of control and leads to a tangible end product for a research-funding agency. Last but not least, the involvement of patients in setting a research agenda does not demand major changes in procedures.

There are several concerns, however. Various scholars have raised concerns whether after participatory agenda setting, it will not be ‘business as usual’, i.e. professionals once again dominate decision-making processes on what topics are researched in health research (Caron-Flinterman 2005; Abma and Broerse 2007). Several studies can be found in international scientific literature that describe patient participation in agenda setting (e.g. (Oliver 2006; Welfare, Colligan et al. 2006; Owens, Ley et al. 2008; Tong, Sainsbury et al. 2008; Stewart, Caird et al. 2011), articles that focus on patient participation in the phases after agendas have been set (i.e. programming and implementation) are very scarce. What is the role of patients in these phases? And what happens with the topics put on the agenda by patients (Broerse, Elberse et al. 2009; Elberse, Visse et al. 2012)? Little structured insights are available on experiences with, and achievements and pitfalls of, patient participation during the subsequent phases of programming and implementation. The role of patients seems marginal after priorities are set. Funding agencies as well as researchers have their own procedures, culture and structures. Not all see the benefits of continues involvement of patients, not all feel attracted to engage in new topics prioritized by patients. Often there is a lack of knowledge to involve patients more structurally, also in implementing the research priorities of patients.
Although agendas do change due to patient involvement, there are indications that the topics funded did not change significantly because the topics introduced by patients are often not interesting to researchers. In case of Burns, itching was highly prioritized by patients, and the Dutch Burns Foundations put it on their research agenda (Broerse, Elberse et al. 2010; Broerse, Zweekhorst et al. 2010). However, for some years, no proposals for research were received. More attention was given to this topic to stimulate researchers writing proposals. Extra incentives seemed needed to stimulate researchers writing a proposal on this topic. This example point out that even when research topics considered important by patients are put on the research agenda of a funding agency, it does not guarantee that the research topic is picked by the research community. Additional actions seem needed. Currently, there is too little insight in the impact of patient participation in research agenda setting on the health research system, although, according to Stewart et al., setting priorities with different stakeholders does not have much impact on health care and health policies (Stewart, Caird et al. 2011). Addition research executed by EMGO+, VUmc and the Athena institute, VU University, which is financed by ZonMw/VSBfonds is currently in a final phase. By means of a two-year evaluation study insight was gained in what happens with the priorities set by patients as well as the role patients play after priority setting. Important findings are that patients have a surplus value as they add new topics to the agenda and prioritize different topics compared to professionals. However, these ‘new’ topics are not always implemented by funding agencies or picked up by researchers.

Another concern is that collaborations between patients, funding agencies and researchers are often not sustained. Initiatives are usually one-off events, with no continuity, feedback or future planning (see also Jordan, Dowswell et al. 1998; Stevens, Wilde et al. 2003)). Furthermore, the majority of funding agencies still does not include patients in their agenda-setting and appraisal procedures. Fourthly, there is a lack of thoroughly evaluated initiatives which is also extensively mentioned in the literature (Beresford 2007; Staniszewska, Herron-Marx et al. 2008; Serrano-Aguilar, Trujillo-Martín et al. 2009; Broerse, Zweekhorst et al. 2010). The methodologies employed and lessons learned are rarely evaluated, and feedback from experiments is restricted. Consequently, there are as yet few ‘proven’ effective methodologies or ‘best practice’ models to guide those individuals and organizations that would like to experiment with patient participation in health research. Fifthly, health researchers rarely take up the role as initiator or advocate of patient participation. They rarely participate in organized debates on patient involvement in research (Moens 2010). Finally, resistance is experienced among researchers, policymakers and health professionals in realizing the required changes for the effective involvement of patients in research.
Regime features leading to barriers to patient involvement

Although the number of initiatives for realizing patient participation is increasing, involvement of patients in research is still far from being common practice within the health research system, and barriers are encountered. The cases described above show that there are still many challenges to deal with to make patient participation and the use of experiential knowledge in health research more structural. Based on the case studies complemented with interviews with a broad range of stakeholders, observations, additional case studies and the scientific literature on patient participation, it becomes apparent that the encountered barriers are related to certain features of the health research system. Realizing structural patient participation seems to require a radical change in thinking and doing from the different actors involved. However, the current culture, practice and structure of the health research system developed over time, resulting in a dominant, stabilized way of organizing itself, thinking and acting (Van Raak 2010). Below, the features that form a barrier for patient participation are identified.

Academic research community

Decision-making dominated by experts in research
Traditionally, decisions are made by experts. While the NAF, ZonMW and a number of other funding agencies are slowly changing their procedures for funding by including patients in the decision-making processes, many funding agencies still do not consider this an improvement and prefer to retain a more academic structure. Including patients in decision-making processes means that the autonomy of researchers is challenged and that the focus on scientific quality may shift towards a focus on societal relevance. It means that the current procedures will need to be adapted to include the introduction of new criteria, such as societal relevance, more attention for benefits vs. burden, or providing a lay summary, but also room for new topics. Since there are few experiences to build upon, this process of adaptation is one of experimentation. For example, the first time patients appraised project proposals for ZonMW, they had major difficulties coping with the procedures: there were too many proposals with too little time to read them, they were mostly in English with much jargon, and no guidance was given on how to judge them. By ‘trial and error’ the procedure for patient involvement is being adapted and improved.

The sometimes negative opinion on patient involvement held by members of scientific advisory boards and the uncertainty about the additional value of their input make some charity funds reluctant to adapt structures and procedures to realize the involvement of patients (Caron-Flinterman, Broerse et al. 2007; Broerse, Elberse et al. 2010). An often heard argument is that patients are biased and subjective and will only focus on research projects which can lead to a personal benefit. Also, they lack the right knowledge as stated by an interviewee: “Patients lack the knowledge and experiences. They are not used to work with biomedical information so I think
it is really difficult." Furthermore, some researchers are convinced that they already know what patients need and want, and therefore involvement is not needed. According to an interviewed researcher: "I do not see the added value of involving patients in research (...) a good clinical researcher knows the patients and knows the things that are relevant."

Specialization
The health research system is highly specialized, categorized into a range of scientific disciplines (Pang, Sadana et al. 2003). Researchers often specialize in a certain type of research (e.g. genetics, tissue engineering) and focus on a specific disease (e.g. cancer, asthma, depression) or organ (e.g. heart, lung, kidney) instead of the patient. The strong specialization within the health research system complicates the interaction between researchers and health professionals, policymakers and patients. The problems which patients with chronic diseases face are often interdisciplinary and integral by nature, such as co-morbidity, psychosocial issues related to a somatic condition, pain or fatigue. Such problems rarely fit specializations. This leads to under-researched areas; interdisciplinary problems brought in by patients are often not addressed by researchers. An example is the topic of ‘itching’, which was considered an important research topic by burn survivors (Broerse, Zweekhorst et al. 2010). However, the Burns Foundation did not receive any research proposals on this topic. Another example is the topic of co-morbidity, which was mentioned by patients as an important topic in the research agendas of the NAF; this is considered a difficult topic by the scientific advisory board and the research field. Firstly, because of the question of who should fund research on co-morbidity, since the NAF only provides funding for research on respiratory conditions. Secondly, where can researchers be found who are able to do research across different disciplines. A similar difficulty was experienced by the topic drug interaction in the same agenda. A mono-disciplinary approach constrains a patient-oriented approach (Bensing 2000; Wilson 2000). Conducting interdisciplinary research is, however, often less attractive to many researchers, since there are few interdisciplinary approaches (Wilson, 2000), and this type of research leads to outcomes that tend to be less appreciated or understood by their peers.

The value of scientific knowledge
Researchers define ‘true’ knowledge as data generated through the use of robust scientific research methodologies (Caron-Flinteman, Broerse et al. 2005; Bijker, Bal et al. 2009). Non-scientific knowledge, such as the experiential knowledge of patients, is judged to lie much lower in the knowledge hierarchy (Blume 2005). Experiential knowledge is regarded as ‘a personal story’, as subjective and, thus, ‘difficult to generalize’, instead of being considered usable knowledge. Patients are emotionally attached and therefore endanger objectivity as is illustrated by the following quote of a researcher: “Patients have too many personal issues to give their opinions at the level of decision-making.” Researchers often do not know how to integrate experiential knowledge in their research; they focus on scientific excellence and theoretical purity, and the
input of patients does not correlate with that. Patients are usually considered lay people, without the required knowledge to contribute anything relevant to the scientific research endeavor (Caron-Flinterman 2005). Also, the idea of patients becoming co-authors of an article, after being involved in research, is not considered beneficial: “There are going to be problems with publications. Reviewers are going to say: that is a patient, not a researcher, we are not going to accept this. Yes, I think many researchers will be skeptical, it will not be accepted easily” (a researcher).

The strong focus on validated scientific methodologies in health research does not leave room to integrate experiential knowledge and thus forms a barrier to patient participation in health research. For example, researchers use validated questionnaires. In different case studies patients have pointed out that the questions were unclear, irrelevant or not well described. However, researchers are reluctant to change validated questionnaires. “About some question in a questionnaire patients say: that is a weird question. But if they fill it in and you don’t ask what they think about the question, in the end the results seems to have a good predictive value.” Changing opinions towards acknowledging patients’ experiential knowledge as a valuable input requires a fundamental change in the scientific paradigm, incorporating a broader, non-hierarchical view on knowledge, and an acknowledgement of the uncertainty and fallibility of scientific knowledge.

Scientific excellence

Within research, there is a strong focus on scientific excellence, generating scientific knowledge that can be published (see also (Smith 2003; Hessels 2010)). Spending time with patients to discuss a research project is often considered a distraction from research instead of a possible improvement of work. Researchers are more inclined to pick up research topics that lead to publishable results in disciplinary, internationally renowned journals. “People do the things that give them profit, if you get profit for multiple publications you’re going to write many publications. In that case it is not profitable to increase the length of your research process by patient participation since patient participation will take more time.” Research with mainly a societal impact rarely improves career perspectives. Since researchers are evaluated by the number of publications and impact factors of the journals they publish in, they prefer topics that are more likely to get published rather than relevant for society. Also, ‘high-tech’ research is preferred over research on more common symptoms, such as low back pain or ear infections, while high-tech research does not always lead to innovations which address the needs of patients or users. Frenk (Frenk 1992) stated that scientific excellence and practical relevance are difficult to combine because they are measured in totally different ways. Little attention is paid to communicating results to a non-scientific audience and implementing generated knowledge in practice (Lavis, Robertson et al. 2003; Jacobson, Butterill et al. 2004). Although growing attention for the societal relevance and practicality of research is being witnessed among funding agencies, career advancement and appreciation of researchers are still primarily related to publication rates and
citation scores as part of the required benchmarking by governments and university boards (Aksnes and Rip 2009; Hessels 2010).

These features of the research community – decision-making by experts in research, strong specialization, the high value of scientific knowledge and relevance – do not allow the system to operate optimally in contributing to improvement of the quality of life of each individual patient. Despite the positive effect of health research on the life of human beings, the health research system favors certain research, methodologies and topics above others, resulting in a gap between what is researched and what patients experience as problematic or functional.

Private research and industry community
Much health research is initiated and conducted by pharmaceutical companies, which are focused on medicine and treatment development. Most of them are big multinationals, striving to make a profit. In most pharmaceutical companies, patient participation is a rare phenomenon. Many barriers to realizing more patient participation seem to be in place in pharmaceutical companies. Firstly, as in the academic research community, most companies mainly consider patients as ‘subjects of study’. They are satisfied with the current situation and do not see a need for change. Secondly, there is a lack of awareness of the potential benefits of patient participation and how patients may become more actively involved (how to do it). At the same time, there is a lack of best practice examples. Thirdly, rules and regulations prohibit many forms of contact between pharmaceutical companies and patients, and it is unclear which forms of patient participation are allowed. Fourthly, local companies are usually not independent but affiliated to a multinational. These local companies have little influence on the policies of the central board of the multinational (Van Beest, Betten et al. 2008; Fransen 2009; Broerse and Elberse 2010). Despite the many barriers, there are developments visible (O’Connell and Mosconi 2006; Thornton 2008). Some initiatives, like the Patient-Industry and PatientPartner, lobby for a more active role of patients within private research.

Patient community
The patient community also has features that may not be conducive to their involvement in research. Patient organizations and communities are not always effectively organized to make a strong demand of the research community and are easily ignored. Traditionally, patient organizations focus on peer support, providing information and advocating for good care. To be actively involved in health research has been considered a possibility only in the last decade. Due to the growing attention paid to the patient community by different actors ranging from governmental organizations and care organizations, the workload of the patient community is also increasing. Therefore, patient organizations need to choose between different activities, and often the priority for becoming involved in health research is low. They prefer to invest time in care-related issues and peer-support activities.
The patient community usually lacks resources – money, manpower and time (because many people in the patient community work voluntarily). Although the Dutch government is trying to address this problem by investing money in the professionalization of patient organizations, it is a slow process. It is complicated by the ambiguous term ‘professionalization’. Some consider a patient organization professional if they employ ‘professionally’ educated staff or when the organization is well structured, while others believe that being professional entails the ability to represent the ‘general patient population’ or being able to represent the voice of the patients during discussions and decision-making.

Researchers wishing to involve individual patients in research sometimes complain about the limited interest patients have in participating in health research. There are various reasons for this. Firstly, patients feel they lack knowledge about scientific research and, thus, have little to contribute (Van de Bovenkamp, Trappenburg et al. 2010). They often do not value their experiential knowledge and lack self-confidence. The general thought is that decision-making on health research should be left to experts. Secondly, the jargon used by many researchers discourages patient participation. Thirdly, most patients consider themselves ignorant regarding the implications and benefits of health research on their personal life. The effects of care are often directly evident for patients while the effects of health research are ‘distant’ and ‘complex’. As a result, there has hardly been any direct interaction between researchers and patients in the past.

**Deepening, broadening and scaling up**

Although the number of initiatives at the niche level is increasing and supporting landscape developments are putting pressure on the health research system, there is still no sense of urgency to change the incumbent regime among different actors, particularly academic researchers and pharmaceutical companies. According to many actors, the health research system is functioning satisfactorily. It facilitates the development of high-quality medical innovations that improve the quality of life of millions of people. Dominant players consider the gap between obtained knowledge and patients’ needs rather small and therefore feel there is no need to involve patients. This idea is strengthened by the limited proof of the benefits and effectiveness of patient participation in research. Although there is a growing body of literature on patient participation in research, not much has been published on the effectiveness and impact on health research. As long as people are not convinced of the benefits, they will not take the time or effort to be part of these initiatives (see also Rogers 2003). Combined with the fear of delays and complication of decision-making processes, stakeholders believe that the benefits – if any – do not outweigh the disadvantages.
System innovations only occur when changes transcend from the niche level to the regime level (Kemp and Rotmans 2001). The next question is, how could a shift towards a more need-oriented health research system be enhanced? A system innovation requires knowledge, best practices and methodologies, resources like money and time, a shared vision, and a growing network with actors willing to invest time and take risks. Furthermore, the creation of supporting structures and regulatory frameworks is important. In the system innovation and transition literature, three central mechanisms are described through which experiments contribute to a system innovation or transition: (1) deepening, by learning as much as possible in a specific context, (2) broadening, by linking and repeating the experiment in different contexts, and (3) scaling up, by embedding the experiment in the incumbent regime, changing the dominant way of organizing, thinking and acting (Rotmans and Loorbek 2006; Van der Bosch and Rotmans 2008; Raven, Van der Bosch et al. 2010). These ‘steering’ notions focus on the importance of creating space for learning processes at different levels, while at the same time stimulating interaction between experiments and the broader context and actively working on embedding the new practices to increase the impact of the experiment at a higher scale level (Van der Bosch and Rotmans 2008; Van der Bosch 2010). During the system innovation, all three steering notions are essential, and it is important to pay attention to them simultaneously. In the current developments, some steering mechanisms are already being applied, mostly deepening and broadening. Momentarily, not many actions can be seen for scaling up patient participation in health research. In this section we will briefly introduce the notions ‘deepening’, ‘broadening’ and ‘scaling up’ from transition management and provide indications based on these notions of how to continue to enhance a system innovation towards more needs-oriented health research.

**Deepening**

Deepening involves social learning processes in a specific context, implying shifts in thinking about culture, structure and practice, their relationship with other actors and the broader context, as well as the interaction between new culture, structure and practices (Röling 2002; Van der Bosch and Rotmans 2008). Experimenting with and learning from transition experiments could lead to the enhancement of a shift in thinking and doing (Schot and Geels 2007). Learning can be considered an interactive process to develop or obtain knowledge, competences, norms and values. Attention should be paid to experiment-based learning of the possibilities and constraints of patient participation. It is also relevant to gain insight into the expectations and attitudes of the actors involved (Raven 2005). It is important to shape the different expectations to ensure that they are realistic and to identify which aspects vary. New, innovative experiments demand open-minded people, willing to experiment with new practices (Van der Bosch and Rotmans 2008; Van der Bosch 2010). Also, change agents with a strong drive are essential for a system innovation.

As described earlier, different experiments are taking place, and change agents with a strong drive to change are present. ZonMW is stimulating learning in the field of patient participation in
research by providing subsidies for the execution and evaluation of transition experiments (ZonMW and VSBfonds 2009). These subsidies address a strong need for thorough evaluation of experiments with patient participation in health research to gain insight into experienced barriers, solutions, enabling factors, effective methods and evidence of impact and benefits (Beresford 2007; Broerse, Zweekhorst et al. 2010). When transition experiments are evaluated, lessons can be made explicit and experiments can be improved and adapted, leading to best practices. Gaining insight into the impact and benefits of patient involvement in health research can remove an important barrier for researchers. It can lead to convincing proof-of-principle concerning the added value of patient participation in research. Besides evaluating current experiments and approaches, there is a strong need for making an inventory of best practices and developing new approaches for patient participation in health research. New approaches are needed because currently there is a lack of knowledge on how to put patient participation into practice throughout the research cycle. For some steps of the research cycle (e.g. agenda-setting), effective approaches are being developed. For other steps, new social experiments should be set up to test appropriate new approaches. New practices can be tested and improved in transition experiments, leading to evaluated best practices to envisage change in the system (Broerse, Elberse et al. 2010).

Secondly, not all research projects are suitable for patient participation, and a balance needs to be found between the involvement of patients and the benefits for the research project. However, clear criteria are lacking to identify suitable research projects (De Wit, Elberse et al. submitted). Also, there are various forms of participation, and some are more suitable than others in certain research projects. The intrinsic and potentially utilitarian value of basic health research should not be underestimated. And therefore, the question of whether all types and phases of health research should be subjected to the influence of patients is legitimate. Experiments are needed to clarify this issue. The involvement of patients in health research is not a plea for putting a patient next to every researcher. It addresses the question of how patients can be effectively involved, leading to a more needs-oriented system.

Thirdly, it is important to focus on the experiences and expectations of researchers and other actors, because there is limited insight into how they feel about patient participation. Researchers have hardly had a say in how transition experiments were shaped or executed. This was mostly decided upon by funding agencies or patients and their representatives. It is also important to keep track of how attitudes and expectations change over time and how the expectations of the different actors involved can be aligned.

Fourthly, another focal point should be the sustainability of collaboration between the different actors. If active involvement stops after a project is finished – as is now often the case – neither researchers nor patients have the opportunity to evolve. Patients will develop more confidence
over time and will be able to provide more useful input and get more used to the practices within health research (Shea, Santesso et al. 2005; Kirwan, Newman et al. 2009). Researchers need more time to get familiar with the involvement of patients and how to handle their input based on experiential knowledge. Collaboration often only continues if the different actors involved have an intrinsic motivation (Jansen, De Vries et al. 2008). Therefore, it is important to understand why people want to become involved in an experiment. Actors need to be motivated to continue collaboration in order to create new routines.

Broadening

Broadening entails the notions of repeating experiments in different contexts (Raven 2005; Geels and Raven 2006; Rotmans and Loorbak 2006) and linking experiments to other functions. Experiments which are considered successful can be repeated in a different setting while applying the lessons learned and adapting the experiment to the new context. Guiding principles can be established based on previous experiments to provide direction and support for new experiments (Raven, Van der Bosch et al. 2010).

Most initiatives with patient involvement are ad hoc and single events (Jordan, Dowswell et al. 1998; Stevens, Wilde et al. 2003; Caron-Flinterman, Broerse et al. 2005). However, the repetition of transition experiments in different settings is essential in the development towards a needs-oriented health research system. For example, the involvement of patients in research agenda-setting was repeated by different actors, including funding agencies and the Health Council of the Netherlands (RGO 2005; RGO 2006; Gezondheidsraad 2006; Gezondheidsraad 2010; Gezondheidsraad 2011; Elberse, Pittens et al. 2012). This led to a ‘best practice’ and guiding principles which actors can follow (Abma and Broerse 2010). Experiments with a patient advisory board within funding organisations are also being copied.

Initiatives with patient participation in health care, health policy and health education are also taking place. The different actors involved in these experiments can help stimulate and inspire each other. The fact that experiments are occurring in these different domains encourages the development of knowledge and skills for patient participation. An enabling element is that most actors are related to different areas. For example, the NAF is also experimenting with patient participation in health care and care improvement. Some researchers are care providers, for example clinicians. This may lead to synergy.

The involvement of more actors in current niche developments will help to create momentum in order to influence the regime and stimulate a system innovation. Therefore, the approach used by ZonMW, focusing on connecting people, is essential. Networking is also beneficial for creating awareness and connecting different transition experiments; actors from different experiments can stimulate and learn from each other. It is important to gain insight into the different approaches,
underlying visions, methodological steps, effects and impacts on society and patients as well as science and health research. When these insights are well documented, interested stakeholders can experiment and reflect upon the approach and make possible adaptations. Also, discussions can lead to new ideas and visions, important to stimulate the system innovation even further. The national and international lobbying for more patient participation by several frontrunners is important to make people aware of possibilities and create a larger network.

To generate interest in different actors to become involved in activities, they need to be motivated to take risks. Intrinsic motivation can be enhanced by making the benefits of involving patients clearly visible. In order to change the dominant way of acting and thinking in research, researchers should firstly become aware of the effectiveness and usefulness of involving patients. Currently, experiments are often not published nor shared in other ways (Crawford, Robotham et al. 2011). For example, many scientific journals are not interested in articles on patient participation. As a result, knowledge obtained during experiments with patient participation is not easily accessible, and researchers rarely encounter examples of patient participation within ‘their’ international scientific literature. Visibility can be created by publishing the results of patient involvement in research in journals read by health and biomedical scientists, or by presenting work at conferences in their field.

**Scaling up**

Scaling up refers to activities to embed the new culture, structure and practices at the regime level (Geels and Raven 2006). When scaling up, a shift in thinking, doing and organizing is taking place whereby the dominant regime changes fundamentally. This steering notion is the most challenging one. Much resistance can be encountered, and institutional barriers have to be overcome. It is of major importance to involve key figures who have the willingness to change as well as access to resources and support (Van der Bosch 2010). Also, it is important that landscape developments support this shift. In the case of patient participation in health research, the landscape developments like increasing patient empowerment, democratization of science and public accountability can be considered supportive for a shift towards a more needs-oriented research system.

Although more supportive structures seem to be coming into existence, e.g. funding agencies requesting lay summaries or statements about how researchers will realize patient participation in their research, most funding agencies are hesitant to reject research proposals because they insufficiently addressed this criterion. Care should also be taken that such incentives do not lead to tokenism. Tokenism is the involvement of patients without giving them a real influence, creating a false appearance of the involvement of patients in health research (Beresford 2003; Pronk 2007). Researchers indicate that the risk of tokenism will be present when they are obliged by financers to realize patient participation without receiving further support, additional money or
Concluding remarks

Allocating an active role to patients within health research seems to be quite a radical change. To realize such a change, a system innovation would be needed affecting institutional structures, funding structures, and established ways of working change. It is expected that such a drastic change will take time and effort and will need to overcome various barriers. However, considering the current developments, a system innovation from a supply-driven health research system towards a needs-oriented health research system by means of patient participation may be possible. Currently, there seems to be commitment from a growing number of organizations in the system to effectuate a change towards a more needs-oriented health research system. Front runners like the Netherlands Asthma Foundation, ZonMW and the Dutch Arthritis Patients’ League can be identified. They stimulate different ‘followers’ in these developments, who have also started to experiment with different approaches of patient participation. The current initiatives are still fragile, however. Involving patients in health research implies a rather radical change in the dominant culture, structure and practices. Such changes are complicated, given the generally observed resistance of networks and regimes to change.

The current change agents with respect to patient participation have expressed a particular need for more appropriate methods and increased competences at the niche level and adjusted procedures at the regime level. In order to enhance this shift, the effectiveness and usefulness of the different experiments should be analyzed more thoroughly and made more visible to the different potential change agents so people can learn from each other. More importantly, the impact of patient participation on health research, patients and other actors should be made more visible for the research network to induce change in the dominant way of organizing, thinking and acting. Specific attention should be paid to the interaction between the niche and regime level.

Despite the obstacles of enhancing a system innovation towards a more needs-oriented health research system, different initiatives do show that the involvement of patients could induce this shift, like involving patients in setting a research agenda. The contribution of the experiential knowledge of patients to health research processes may lead to closing the gap between needs and what is researched. Related to the different phases described in the transition theory, there are indications that the system innovation towards a more needs-oriented health research system
is in the take-off phase. During this phase, the development of change gains momentum, resulting in structural changes. The current health research regime is losing support for its practices and structures on some fronts. Niches are gaining support, and more approaches are arising. There is a growing interest in patient participation in health research from different actors like patient organizations, government-related organizations like ZonMW and RGO, and charity foundations. Funding agencies are involved in different approaches, are taking actions and setting up different experiments. Since many charity foundations have close relations to both a patient community and a research community, they might be able to influence the parties and bridge cultural and structural gaps effectively. The concept of patient participation is also slowly becoming visible in scientific papers and congresses within the system and in formal policy documents. These points indicate that this might be the beginning of a shift towards a more needs-oriented health research system in the Netherlands.
Part 3: Setting priorities
Patient participation in priority setting

In the introduction, a needs-oriented health research system is defined as a system addressing the ‘needs’ of patients regarding what is researched as well as how research is conducted. In patient participation in priority setting, the focus is on the role patients can have in defining ‘what’ should be researched. While in the described case studies the term research agenda setting is used, in literature, also the term priority setting for health research is used (Clavisi, Bragge et al.; Viergever, Olifson et al. 2010). Based on a workshop discussing priority setting in health research, Nasser developed the following definition of research priority setting:

“Research priority setting is part of a research cycle starting from mapping and identifying the stakeholders, understanding the context, identifying questions, prioritizing them, disseminate the results, and finally implement, evaluate them and providing an opportunity to appeal the results. It has an explicit objective to identify priorities but might have additional implicit objectives e.g. involving certain stakeholders in the work of the research organization. Priority setting processes includes (a) context (b) skills (c) methods. A priority setting process is always happening in a specific context, setting and population.” (Nasser 2012).

One of the first steps in a research process is identifying research topics. A list of relevant, high prioritized research topics in a specific field is called a ‘research agenda’. Since governments, funding agencies, research institutes and departments and charity funds do not have an unlimited amount of resources to spend on health research, it is important to make choices about which research topics are most important; a research agenda can help to make efficient use of limited resources, to steer funding and lobby (Fleurence and Torgerson 2004; Viergever, Olifson et al. 2010). Research agendas can be set using a formal or informal approach, with or without a political mandate. Most of the time, topics on research agendas are defined by (scientific) experts and/or the scientific advisory board of a funding agency or government (Caron-Flinterman, Broerse et al. 2007; Tong, Sainsbury et al. 2008; Broerse, Elberse et al. 2010; Gold, Whitlock et al. 2011; Petit-Zeman 2012). Therefore research agendas can be considered supply driven: experts decide which research topics are on the agenda. From their professional position, they consider which developments in the research field are important to connect to and stimulate and which research topics seem most promising. Often, topics are chosen which are promising for publications and scientific relevance. And most topics on research agendas are disciplinary focused.

Despite the fact that experts often make accurate decisions on what should be on the research agenda, and the health research system realized much improvement in health and quality, there are gaps in health research as already described in part two of this thesis. Research topics
considered important by patients are neglected or not known (Grant-Pearce, Miles et al. 1998; Buckley, Grant et al. 2011; Stewart, Caird et al. 2011). Research topics are often disciplinary focused while patients experience multidisciplinary or more holistic problems. Including patients in the research agenda-setting process could address biases in research agendas by indicating new research topics, or broadening or shifting the focus of the agenda (Goodare and Smith 1995; Popay and Williams 1996; Entwistle, Renfrew et al. 1998). Patients have experiential knowledge which can complement the scientific knowledge of experts (Boote, Telford et al. 2002; Caron-Flinterman, Broerse et al. 2005). Different scholars argue that active involvement of patients in research agenda setting will lead to more needs-oriented research (Blume and Catshoek 2001; Telford, Beverley et al. 2002; Oliver, Clarke-Jones et al. 2004; Caron-Flinterman 2005; Broerse, Elberse et al. 2010). For inducing change towards a more needs-oriented health research system, it is important to obtain insight in patients’ priorities for health research. Within different organizations, including academia, funding agencies and patient organizations a variety of experiments have stimulated the development of patient participation in establishing research agendas (Abma and Broerse 2010).

**International developments**

Patients are increasingly involved in setting research priorities (Gold, Whitlock et al. 2011; Stewart and Oliver 2011; Stewart, Caird et al. 2011), especially in the UK, Canada and the Netherlands. Examples are known from different disease areas, cancer (Wright, Corner et al. 2006; Corner, Wright et al. 2007; Tyndale-Biscoe, Malcolm et al. 2012), chronic kidney diseases (Tong, Sainsbury et al. 2008; Nierse, Schipper et al. 2011) mental health diseases (James, Aitken et al. 2002; Gold, Whitlock et al. 2011; Lloyd and White 2011) and respiratory diseases (AsthmaUK 2004; AsthmaUK 2007; Sheikh, Major et al. 2008). Different approaches and methods are described in literature, although still limited, which are used to elucidate patients’ priorities for health research. Several examples are known whereby a Delphi method is used to gain insight in patients’ research priorities (James, Aitken et al. 2002). Owens (2008) describes that four different stakeholder groups were involved simultaneously in four separate Delphi surveys, aiming to identify each group’s priorities for research on mental health and learning disabilities (Owens, Ley et al. 2008). McIntyre (2010) set patients priorities for cerebral palsy by means of the Delphi method (McIntyre, Novak et al. 2010) as well as by Malcolm (2009) who used a three round Delphi technique to identify and prioritize the research topics for children’s hospice care in Scotland (Malcolm, Knighting et al. 2009). In this latter study all stakeholder groups were included in one survey. Also the so-called ‘Listening model’ is used to involve different stakeholders including patients in setting research priorities (Lomas 2003).

In the UK, the James Lind Alliance (JLA, www.lindalliance.org) aims to encourage patients, carers and clinicians to work together in Priority Setting Partnerships in order to identify and prioritize
uncertainties about the effects of treatments (Firkins 2008; Cowan 2010; Petit-Zeman, Firkins et al. 2010; Stewart, Caird et al. 2011). The JLA facilitates and supports these Priority Setting Partnerships. The process always consist of at least one patient organization and one clinical organization. The process is adapted to the stakeholder groups involved. However, to guarantee a certain consistency and optimal learning, the JLA formulated different principles: transparency of the process, balanced inclusion of interests and perspectives of patient and clinician, exclusion of non-clinician interests and perspectives, exclusion of non-clinician researchers for voting purposes (though they may be involved in all other aspects of the process), exclusion of groups/organizations that have significant competing interests (for example pharmaceutical companies), audit trial of original submitted uncertainties to the final prioritized list, and recognition that priority decisions do not create new knowledge, but review existing evidence of uncertainty.

The outcomes of the Priority Setting Partnerships are used to influence and inform publicly funded research. Several different Priority Setting Partnerships have been established so far. JLA stimulated partners within the partnership to publish in related disciplinary journals (e.g. asthma (Elwyn, Crowe et al. 2010), urinary incontinence (Buckley, Grant et al. 2010; Buckley, Grant et al. 2011), eczema (Batchelor, Ridd et al. 2012) and schizophrenia (Lloyd and White 2011) to create more visibility of the priority setting outcomes and to raise more awareness about the benefits of involving different stakeholders in priority setting. The JLA works closely with NHS Evidence (part of National Institute of Health and Clinical Excellence) which hosts the UK Database of Uncertainties about the Effects of Treatments (UK DUETs, http://www.library.nhs.uk/duets). UK DUETs has been established to publish uncertainties about the effects of treatments. These uncertainties can currently not be resolved by reliable up-to-date systematic reviews of existing research evidence.

Despite the above described international examples, there are still few validated methods to involve patients meaningfully in setting research priorities. Some patient organisations did not follow any method, but decided to directly influence medical research by raising funding for research they consider a priority (Blume and Catshoek 2001; Flinterman, Teclemariam-Mesbah et al. 2001; Kent 2002; Rabeharisoa and Callon 2002). Since the money can be spent according the wishes of the patient organisation, they can steer research in every direction they feel is important.

Till the beginning of the last decade, hardly anything was known about how to realize patient involvement in research agenda setting. However, in the Netherlands there was a growing interest of patient organizations and funding agencies to let patients become heard in establishing a research agenda. Therefore, the challenge of realizing patient participation in health research agenda setting was taken up here. The Dialogue Model was developed which operationalizes the
partnership concept of a ‘participation ladder’ described by Arnstein (Arnstein 1969) (Caron-Flinterman 2005; Abma, Nierse et al. 2006; Abma and Broerse 2007; Abma and Broerse 2010). The Dialogue Model is inter alia based on participatory and interactive methodologies and the Interactive Learning and Action approach (Broerse 1998; Broerse and Bunders 2000; Caron-Flinterman 2005; Roelofsen, Broerse et al. 2008). Despite the repeated use of the Dialogue Model, still much can be learned on how to optimize patient participation in health research agenda setting. In the next three chapters, three case studies are described whereby the Dialogue Model is used to set a research agenda including the patient perspective. The three case studies differed from each other in aim and scope, providing the opportunity to experiment with and learn from patient participation in health research agenda setting in order to gain insight in how to stimulate embedding of patient participation.