Summary

The health research system is responsible for generating knowledge through ‘scientific inquiry’ focusing on people’s health and well-being. Health research spans the entire range from (bio)medical research, clinical research, public health research, epidemiological research to care research. The current system has developed over time, building upon established relationships between actors, stabilized ways of interaction, set procedures, standardized routines, and shared values and views on research. Its incumbent culture, structure and practices are difficult to change.

Despite the fact that the system has generated an enormous amount of knowledge and innovation that has allowed many to live longer, fuller lives, there are also concerns. The health research system is rather supply-driven, in the sense that research is driven by the ideas and interests of researchers. The system’s supply-driven nature could lead to mismatches in knowledge development, since topics selected by researchers and experts do not necessarily correspond to patients’ needs; nor is it always pursued in ways preferred by patients.

Active patient involvement could improve the quality and relevance of health research as the experiential knowledge of patients – acquired through living with an illness, sickness and/or disease – can complement the scientific knowledge of researchers. By involving patients, research can become more responsive to their needs. Acknowledging the role of patients as the end-users and primary stakeholders of health research would increase both the political legitimacy and improve the quality of the decisions that are being made. Furthermore, it can lead to higher levels of social support for, and acceptance of, research. However, 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. A system innovation is needed to change from a supply-driven system towards a patients’ needs-oriented system.

The involvement of patients in health research has grown over the past two decades, with patients and patient groups demanding that their voices be heard. Health researchers and health funding agencies increasingly consult patients; policy-makers in health research-related organizations increasingly consider patients as stakeholders and a source of experiential knowledge. The voice of patients is gaining weight within health research. However, 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. A change in the current culture,
structure and practices of health research is needed to effectively and structurally involve patients. The goal of this thesis is to contribute to an increased understanding of how a system innovation towards a more patients’ needs-oriented health research system can be enhanced by means of active patient involvement in health research. Three main objectives have been formulated:

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?*

**Methodology**

The thesis presents an inventory study and four case studies (transition experiments) which form the main core of this thesis. The inventory study focused on developments taking place in the Netherlands and the activities of two frontrunners in relation to patient participation in health research. It scrutinized which barriers are encountered that are related to the incumbent health research system and how the steering notions ‘deepening’, ‘broadening’ and ‘scaling up’ can be applied to enhance a system innovation towards a needs-oriented system.

Three transition experiments were developed, executed and analysed based on the Dialogue Model to gain insight into patient participation in health research agenda-setting and its potential to enhance a system innovation towards a needs-oriented health research system. 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 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 questionable whether they have a profound impact to enhance a system innovation. Therefore, a fourth transition experiment was undertaken that aimed for structural patient participation in the conduct of health research projects by means of partnerships between patients and research. The ‘Network Patient Research Partners’ initiated and coordinated by the Dutch League of Arthritis Patient Associations has been monitored and evaluated to investigate 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 the conduct of health research may induce a shift towards research which is conducted more in line with the perspectives of patients.

**Inventory**

Patient participation in health research is slowly increasing and progress clearly can be witnessed. Currently, there seems to be a commitment from a growing number of organizations in the Netherlands to effectuate a change towards a more needs-oriented health research system. Front runners can be identified like the Netherlands Asthma Foundation, ZonMW (The Netherlands Organization for Health Research and Development) and the Dutch Arthritis Patients’ League. They stimulate ‘followers’ in these developments, particularly other funding agencies and patient organizations. Patients are increasingly involved in research agenda-setting, in the appraisal procedures of funding agencies, in clinical trials and in the execution of a broad range of research projects. This indicates that the possibilities for patient participation in health research are wide-ranging. However, in the Netherlands, many approaches are still in an ‘experimental phase’; patient participation in health research is still contested, and barriers are experienced. Barriers are related to characteristics of the academic research community (decision-making dominated by experts in research, strong specialization of the research, and the high valuation of scientific knowledge and excellence), the industry (strict rules and regulations related to contact with patients) and the patient community (not always effectively organized, balancing other priorities, lack of resources and reluctance to become involved). Also, there is still no sense of urgency to change the incumbent system among different actors, particularly academic health researchers, since the health research system is functioning rather satisfactorily. To stimulate a system innovation, additional attention has to be paid to thorough evaluation of initiatives, the development of new approaches and best practices, finding a balance between patient involvement and the benefits for health research and increased sustainability of collaboration between patients and researchers. Furthermore, the repetition of experiments is essential, as well as connecting to experiments in related fields like health care and health education. Also the involvement of more actors can create momentum for a system innovation. Making examples and experiences visible and accessible via different routes is crucial.

**Research agenda-setting**

The Dialogue Model for patient participation in health research agenda setting is adopted in three different transition experiments. This model is flexible so it can be adapted to specific circumstances, needs and wishes of the stakeholders involved and to requirements from commissioners. Also there is a strong focus on social conditions which enables patients to become involved in the process and share their ideas and perspectives.
It is one thing to strive for equal collaboration between patient and professionals, but another to operationalize this. There is no history of equal partnership between patients and professionals to build upon. Instead the relationship between experts and patients can be described as asymmetrical due to the traditional difference in social status, as well as to the value assigned to the knowledge of both groups. To optimize the dialogue between patients and professionals in research agenda setting, it is important to be aware of exclusion mechanisms and to apply inclusion strategies. ‘Exclusion’ is defined as a process in which members of a certain stakeholder group – in this case patients or patient representatives – or their perspectives are not incorporated in the decision-making process, because of actions taken by members of other stakeholder groups or the process facilitator. Applying inclusion strategies to overcome exclusion mechanisms can help to stimulate genuine dialogue between the different stakeholders involved.

Three categories of inclusion strategies can be distinguished; ‘circumstances’, ‘behavior’ and ‘verbal interaction’. When it comes to creating ‘circumstances’ as a basic requirement for a genuine dialogue, the precautions are in the field of preparation and organization. To stimulate inclusion in relation to ‘behavior’ and ‘verbal interaction’, strong facilitation is essential for recognizing and correcting exclusion. However, people are often unaware that they exclude others through their use of verbal and non-verbal communication. This is a subtle process; in the case described in Chapter 5, patients and informal carers perceived their participation in the dialogue as inclusive, although some of their specific input was sidelined, which became evident in the resulting research agenda. It is suggested that exclusion in this case study was based on embedded assumptions concerning the lower value of experiential knowledge compared to scientific knowledge, and the idea that experts are best suited to define the research agenda. This view was not only observed among professionals, but also among patients and informal carers, who seemed to have a high degree of trust in the experts.

When an approach becomes validated, like the Dialogue Model, it is important to test if the approach can become more time and cost-effective, to make it more suitable for embedding in current structures and practices. In Chapter 6, a condensed agenda setting experiment is described. The developed method proved to be successful in the articulation of patients’ and researchers’ needs for health research, resulting in a state-of-the-art research agenda reflecting both the needs of patients and researchers. There was much overlap between the priorities of professionals and patients on broader themes, although they differed in details and brought different and challenging perspectives and issues to the table. A professional patient was involved as a member of the research team to ensure that the input of patients was visible in the integrated research agenda (to avoid tokenism). Ideally, even in a condensed approach, a dialogue should have taken place between professionals and patients to discuss differences and to increase mutual understanding. If topics are only considered important by patients, not by researchers, it entails the risk that researchers will not submit research proposals on these topics.
In the case of the research agenda on medical products, (Chapter 7) the focus was on one specific area of research – the development of medical products – but included 15 different disease domains. The commissioner set strict criteria for the patients’ input as was requested by the Minister of Health. Even though the focus of the research agenda was highly specific and pre-framed, patients were still capable of articulating their needs in a facilitated process. The process was designed in such a way that, while maintaining the focus and criteria set by the commissioner, additional attention was paid to broader issues that were highly valued by patients. During the focus groups, the context of the patients’ daily life was taken as the starting point to articulate needs. Also, issues outside the scope of the agenda were acknowledged in the discussions. It was important for the patients that the commissioner acknowledged their broader input by valuing these issues for providing context to the advice on medical products. A balance was found between keeping with a predefined focus, criteria, and a mandate for the commissioner, while patients are able to provide valuable input from their own perspective.

**Patient research partners in the conduct of research**

To realize patient participation as a partnership between patients and researchers, establishing a network to organize, coordinate and support this partnership provides an interesting opportunity. A network can, besides providing support and coordination, create the feeling of being part of a group. It is important that such a network creates possibilities to meet each other, to exchange experiences and to develop new ideas and visions. Also, the network can incorporate being a neutral party to facilitate the collaboration between researchers and patients. The FIRST model (Facilitate, Identify, Respect, Support, Training) offered guidance in setting up such a network, but adaptations to and elaborations on the model were necessary. The model appeared to be rather static, while setting up successful partnerships is a dynamic process, and regular reflection on the process is needed. The key is to stimulate learning among the different stakeholders about how they would like to shape participation, to make the added value of involvement explicit and to provide feedback on how they experience collaboration and how experiential knowledge can be integrated in research projects. Furthermore, in the current FIRST model, little attention was paid to supporting researchers in this new endeavor while this was very necessary.

The partnerships can be seen as protected spaces in which people (researchers and patients) can experiment with new practices, structures and cultures of conducting health research. The presence of facilitators like availability of resources, training and support for patients and researchers and the presence of change agents committed to the cause was key. Sharing lessons and making the added value explicit helped researchers and patients to stay motivated and increased their understanding of effective collaboration. Partnerships where facilitators were amply present could shield themselves sufficiently against pressure from the incumbent practice, structures and culture and develop new practices, structures and culture through a joint learning process. Barriers were experienced in all partnerships due to the pressure of the incumbent
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system. Due to the lack of initiative and knowhow by both partners and researchers and little interaction within the partnerships, collaboration was suboptimal. Furthermore, the network operated in a rather isolated way and offered insufficient support and few incentives to the partnerships. Due to a combination of the above-mentioned barriers, most partnerships did not develop new routines and values to involve partners more structurally in research. If few facilitators were in place, the protected space for collaboration did not develop or collapsed, resulting in business-as-usual.

Strategies to enhance partnerships between patients and researchers should focus on training in necessary competences, which include increased awareness of what patient participation entails, a more positive attitude, skills and knowledge, and building up a strong structure for the network. Creating clarity on expectations and agreements about contact, tasks and responsibilities help to realize a successful partnership. However, in this early phase of experimenting with structural patient participation, it is important that partnerships receive tailor-made advice and support. Nevertheless, such profound change cannot be realized immediately; time is needed to develop trust, insight in effective collaboration and self-confidence. Also, a shared vision is needed because it provides direction, alignment and can inspire followers.

Embedding patient participation in health research

Embedding patient participation in health research means that patient participation becomes included in the research structure, culture and practices, anchored in the system, the ‘normal thing to do’. It is important that it takes place in different phases of the research process and involves sustained collaboration between patients and researchers. To embed patient participation, several strategies are formulated. Firstly, embedding clearly requires a learning process. Through experimentation, reflection and learning, more insight will be gained into suitable approaches, projects and tasks. A regular and genuine dialogue between stakeholders will help patients to understand the principles of research better and will help researchers to understand the patients’ story better as well as its value for research. It is important that support is offered to all parties involved to strengthen the competences of all parties to collaborate effectively. Secondly, change agents and followers need to be facilitated by providing appropriate methods, tools and competences. Change agents are people or organizations motivated to induce change and willing to invest resources. In addition, followers are also important; followers can repeat experiments, copy new practices, or develop new values based on what they see done by the frontrunners. If more people follow in the development of change, embedding of patient participation is stimulated. Thirdly, alignment of expectations of parties involved helps to create commitment to the outcomes and clarity on responsibilities and activities. Neutral facilitation could help in the alignment of expectations and in clarifying agreements. Fourthly, in case of structural patient participation, the presence of organizational elements, like a network, can help to embed patient participation. Fifthly, it is important that the benefits are made explicit, visible
and accessible. Making the benefits of engaging patients more explicit on the level of a research project creates a more positive attitude and more open-mindedness towards involvement and motivates researchers to involve patients. Making the added value explicit on a larger scale could serve as an incentive to new actors to start to experiment with patient participation as well.

A system innovation towards a more needs-oriented health research system
Based on the developments described in this thesis, it may be concluded that a shift towards a health research system in which patients have an important role at the stage of ‘take-off’. Changes become visible and an increased number of people and organizations are experiencing a positive effect. But does patient participation result in a more needs-oriented health research system? Agenda-setting is useful and suitable to involve patients and gain insights in their needs. Patients are capable of articulating their needs for health research in a facilitated process, covering a broad spectrum of topics related to their life with a disease. Patients and their representatives identified and prioritized new research topics, complementing those considered important by experts. Patient participation in health research agenda setting has developed quickly in the last years and is still accelerating. An increasing number of patient organizations, funding organizations and governmental organizations established a research agenda including the patient perspective or are currently working on it. This niche seems to be scaling up since involving patients in setting the research agenda becomes more embedded in the current structures and is increasingly considered the ‘normal’ thing to do. Therefore, patient involvement in health research agenda-setting has great potential to stimulate a system innovation towards a more needs-oriented system, moving away from a supply-driven system where professionals set the research agenda. However, there are two drawbacks. First, research topics identified and prioritized by patients are not automatically implemented or picked up by researchers. Having topics put on a research agenda does not guarantee that they will indeed be addressed. Researchers tend to continue formulating project proposals on topics they are familiar with and rarely follow up on the identified ‘gaps’ in scientific knowledge that have been identified by patients. Second, research projects are usually formulated and executed according to ‘business-asusual’. It does not yet lead to a change in how research is conducted. Many barriers are experienced in the partnerships between patients and researchers. This niche is still small in the Netherlands and has had little impact on the regime so far. Several Dutch patient organizations are interested in setting up similar networks, but seem reluctant since there are very few good examples.

Future research
A system innovation towards a more needs-oriented research system is at point of take off. It is still no guarantee that a complete system innovation will take place, but changes become visible within health research and different stakeholders are experimenting with different approaches.
This research contributed to the body of knowledge of patient participation in health research, but still many questions are open. Especially related to structural involvement of patients in different phases of health research, still many issues are unclear. For future research I suggest three main directions: obtaining structural insight in the impact of patient participation, developing new approaches and best practices and developing strategies to enhance a system innovation.

To conclude, to let patient participation become successful, an open mind is needed of all actors involved.