

SUMMARY

PATIENT PARTICIPATION IN RHEUMATOLOGY RESEARCH – A FOUR LEVEL RESPONSIVE EVALUATION

This summary is written for family, friends and patient research partners who are interested in the research that I have undertaken over the last four years.

In this thesis, I have studied a new role of patients with a rheumatic condition in the context of scientific research. This new role is different from the traditional patient role as passive subject of research. The first chapter describes how patients can perform this new role in different ways of which I have examined four: The patient role as a research partner, as a participant at a scientific conference, as a scientific committee member and as a patient researcher.

What these roles have in common, is the equality between patients and researchers when collaborating in research practices. Establishing such relationships is not easy. It is the challenge to create openness for the everyday perspective of patients in a power-free dialogue. Making space for patients' experiential knowledge is important because the patient perspective is often lost in the current system of research. That has to do with the dominant medical discourse in which curing the disease and treatment are central instead of the person with the disease. The focus on medical aspects of the disease means that other aspects are excluded. These are often existential, social and societal questions that arise when people are confronted with a (chronic) disease. These questions go beyond 'does a cure exist' or 'is there a therapy'. These are questions that are important from the perspective of patients, but they often disappear inadvertently and unintentionally from the sight of researchers. I call them 'slow questions' because the solution is often not obvious and scientific research takes time.

Patient participation offers an opportunity to restore the balance between the focus of the research system and the experiences of patients. If patients become involved as equal partners in formulating research questions and are encouraged to think about the conduct of research, important themes of patients and patient organizations will be added to the research agenda. Especially in modern, democratic societies many experiments with patient participation are ongoing. Here participation is seen as a democratic and fundamental right of patients. On the basis of four case studies, I have tried to answer the question: What is the added value of patient participation and what does it take to achieve that?

The case studies take place at four levels within rheumatology research: Local, National, European and International. By interacting directly with all participants (responsive evaluation) I hope to be able to answer the research question. Below I will summarize the findings of this thesis.

Dutch network of patient research partners (Dutch Arthritis Association)

The Dutch Arthritis Association took the initiative in 2007 to build a network of research partners. A patient research partner is a person with a chronic condition who has an interest in scientific research and is willing to bring in the patient perspective into a research project on a voluntary basis. They collaborate with researchers on an equal basis. Within three years, 27 patients were recruited who followed a two-day course and then entered a kind of

apprenticeship at one of the participating academic centers for rheumatology. In the second year of this project, I started, together with Janneke Elberse, studying the success and failure factors of this network. Our evaluation lasted eighteen months. We actively monitored the project and advised the 27 research partners and 16 researchers. We used the FIRST model which has been developed in Bristol (UK) and offers practical guidance for researchers to engage with research partners. The letters stand for **F**acilitate, **I**dentification, **R**espect, **S**upport and **T**raining. Data were collected through interviews, focus groups, (instruction) meetings, email and telephone. The results of this study are presented in chapters 2 and 3.

The research of the Dutch network has shown that the FIRST model is a useful guide for building a network of research partners. In this model we have clarified the distinction between 'facilitate' and 'support'. Facilitation is aimed at creating conditions for patient participation by removing barriers in the research system and motivating and guiding researchers. Facilitation is a responsibility of the research management or the principal investigator. Support is focused on creating a safe environment in which research partners can provide meaningful contributions to research. This is possible by preparing research partners at the start of a project and to give patients the opportunity to tell their story without immediately translating their story in scientific terms. In other words: To engage with research partners in a power-free dialogue and give space to their experiential knowledge. Providing support is a responsibility of the actual researcher who directly communicates with the research partners.

Our research revealed that the FIRST model is not only helpful to guide patient research partners, but also applies to the guidance of researchers. Many researchers were not able to work together with research partners on an equal basis. They indicated that they found it difficult to build a sustainable relationship with research partners. Sometimes because of barriers in the research system and sometimes because they felt insufficiently prepared. They often received insufficient support from colleagues and superiors who, for example, do not accept experiential knowledge as a valid source of scientific research.

European recommendations for patient participation (EULAR)

At the initiative of the European arthritis patient organizations, the European League Against Rheumatism (EULAR) has developed recommendations for involving research partners in scientific research. I facilitated this process as a patient researcher. Part of my responsibility was to create conditions for an open and equal dialogue between seven experienced research partners and seven researchers. This dialogue has led to broad consensus on eight recommendations for patient participation. These are presented in Chapter 4.

Research partners should always be involved in clinical research and in the development of recommendations. In other research, researchers are strongly advised to consider patient

participation. If researchers want to collaborate with research partners, this should start as early as possible, not only once, but preferably during all stages of the research project. It is also desirable that research partners are never on their own in a committee, but always at least with two people. This has many advantages. The recruitment of research partners is facilitated with a clear description of the expected contribution. This helps to clarify mutual expectations. In the recruitment process of research partners communication skills, motivation and a positive attitude are important. Researchers should also provide appropriate support to research partners and take their physical limitations into account. They should ensure proper information and training where necessary. Finally, it is important that the contributions of research partners are sufficiently acknowledged.

Outcome measures in rheumatology (OMERACT)

In 2002 I was invited by the Dutch Arthritis Foundation to participate in a conference on outcome measures in rheumatology (Outcome Measures in Rheumatology, in short: OMERACT) as a patient representative. This event made a great impression on me and has paved the way for my current role as patient researcher. During the conference I was part of a group of 11 patients with rheumatoid arthritis (RA) who were asked to review the existing outcome measures often used in RA clinical trials. This set of RA-outcomes was developed in 1992 during the first OMERACT conference. The purpose was to motivate researchers to measure the same outcomes in clinical trials. There was a great need for more uniformity in the number of outcome measures, the measurement instruments and the reporting on outcomes. With regard to the latter a wide variation existed often resulting in researchers only presenting outcome measures that were found to be promising. The so-called RA core set changed this. Due to the success of the conference and the consensus on the core set, the initiators of OMERACT decided to repeat the conference every second year. After all, for other rheumatic diseases core sets would be of similar importance.

After ten years uneasiness with the RA core set occurred among a small group of researchers. When they discussed the question how big a clinical improvement should be before calling it a 'relevant' improvement, it was suggested that only patients could answer that question in a meaningful way. The organizers had already been thinking about inviting patient representatives, but saw practical objections. In 2000 the discussion reached its tipping point and it was decided to invite patient representatives for the next conference in 2002.

In chapter 5 to 8 I reported the findings of a responsive evaluation on ten years of patient participation in OMERACT. Responsive evaluation means that an initiative or event is evaluated from the perspective of all participants (stakeholders) through a genuine dialogue in which everybody has equal opportunities to speak up. In this case, my research consisted of

an analysis of documents, interviews with participants and observing patient research partners during meetings. Interviews took place with twelve researchers, two representatives of the pharmaceutical industry and international regulators, two staff members, eight experienced and eight new research partners. Of the latter group, I interviewed three participants three times to get a better understanding of how new research partners experience their participation in the conference at different times.

In my role as patient researcher I did not take on the position of an independent outsider. I did not look from a helicopter view at the people and events, but I stepped *into* that event. I talked to people on the basis of equality and tried to obtain more insights in the perspectives of all participants. The objective was to facilitate mutual understanding in order to prepare all participants involved for the consensus finding process. As a researcher, I choose for a multiple partiality. This means that I am equally open for the views of researchers, research partners and other participants. By doing so, I prevented one perspective prevailing over the other, for instance in the case that I would identify myself too much with one particular group. Although I have a strong commitment to the patient group, it is my responsibility to ensure that all participants have equal opportunities to express their opinions in the dialogue. Because in practice, research partners have often more difficulties than researchers to make their voices heard, I helped them to do so. Using this approach, I studied both the process and outcome of patient participation in OMERACT.

Chapter 5 describes the effect of patient participation on the research agenda of OMERACT and how OMERACT has become a shining example for other scientific conferences. The most striking result is the attention that has arisen for the theme of 'fatigue' since 2002. That was, apart from 'well-being' and 'sleep disturbances', a major problem that was missed in the RA core-set by the patients. Almost all interviewees confirmed that fatigue would not have given this attention without the input of patients at the congress of 2002. The topic of fatigue was previously put forward by researchers as an important symptom, but was not picked up by researchers. It was mainly due to the persistence of patients that a stream of publications emerged that also inspired other researchers to study fatigue in other (rheumatic) diseases.

Our study within OMERACT also generated more knowledge about the success and failure factors of patient participation in practice. Chapter 6 describes five success factors described. A long-term commitment of the leadership is indispensable. If the leadership does not endorse the importance of patient participation, it is difficult to start the cooperation between patients and researchers. It is also important that the design of the conference is adapted to the needs and capacities of research partners. This can be done by choosing appropriate time schedules and accessible building for patients, but also by allowing research partners to participate in the

governance of the organization. The contribution of research partners grows when patient participation is embedded in the vision, structure and procedures of an organization. Another success factor is an adequate selection of partners. Within OMERACT researchers are responsible for identifying patients. This worked over the last decade because they know exactly what is expected of the research partners and whether their candidate is competent to work together with professionals. They should not only speak and read the English language, but also have the physical, mental and communicative capacities to endure a five-day conference. In addition, researchers feel responsible for proper guidance and support of their research partner.

From the interviews it became clear that the moderation style of group leaders is decisive for research partners having the opportunity to participate in a discussion or whether their role is reduced to passive observers. An empathic facilitator who has communication skills is necessary to create an open dialogue in which all participants feel safe to participate and to contribute their experiential knowledge. Finally, also individual support of research partners proved to be of importance.

In Chapter 6 I presented three barriers such as the heavy conference program, the limited representativeness of the patient group and resistance to change. This resistance exists among a small group of researchers who are not always convinced of the added value of patient participation. They sometimes feel that OMERACT promotes patient participation for strategic reasons, for example, to be politically correct. Others believe that patient participation takes time without significant benefits. And others still find that the potential value of patient participation is not fully utilized with a serious risk of tokenism.

In chapter 7 we focus on the expectations, experiences and contributions of patients who participated for the first time at the OMERACT conference in 2010. Their stories have taught us much about the dual nature of the learning process that many research partners go through. I described this as a process of socialization in which they learn how they can contribute their experiential knowledge to scientific research in a meaningful way. That is not always what happens in reality. The value of experiential knowledge is often still not recognized, resulting regularly in frustration and disappointment. Research partners do not feel valued for their contributions and may become uncertain. The research system forces them to adapt, but they fear that the value of their experiential knowledge will get lost.

The combined findings of ten years of patient participation in OMERACT are integrated in chapter 8, resulting in a number of recommendations.

Reflections on the role as patient researcher

In chapter 9 I described my experiences as a patient researcher in which the methodology of

responsive research plays an important role. I chose this methodology because dialogue and mutual learning processes are seen as essential concepts of collaboration. During my research it became clear that responsive evaluation is a suitable method to study patient participation in scientific research. Moreover, I have learned that the role of a responsive researcher fits very well with the position of a patient researcher. In both cases the responsibility for creating optimal conditions for dialogue is crucial and the aim is to stimulate relational empowerment: The situation in which both patients and researchers learn from each other and, as a result of the cooperation (synergy), become stronger. To achieve this, as I have mentioned before, a multiple partiality is required: The ability to empathize with the interests, priorities and concerns of both researchers and patients. An open attitude, and knowledge that is obtained from this, enables the patient researcher to become a bridge between both worlds.

This chapter elaborates on some examples where it was not always easy to meet the condition of a multiple partiality. There is always the risk of over-identification, for example when the patient researcher shows too much understanding for the world of peers and the balance between closeness and distance. Or when the patient researcher empathizes too little with the perspective of researchers and tries to convince them of the importance of participation. The different responsibilities of a patient researcher sometimes conflict with each other. As facilitator you want to change behavior, ideas or a research practice. That intention is sometimes at odds with wanting to understand the motives of all stakeholders. To preserve the right balance between understanding and changing the patient researcher should consider continuous self-reflection on their own actions and approach.

Reflection on the findings of this thesis

In the last chapter I have tried to analyze the four case studies from a critical perspective. For this I used the theory of communicative action of the German philosopher Jürgen Habermas. How can patient participation contribute to bridging the gap between the modern world of medical research and the everyday world of people with rheumatic diseases? In other words: What conditions make researchers and patients to give up their traditional positions to enter an equal conversation regarding the direction and content of scientific research?

In practice, it is difficult to achieve such a constraint-free dialogue. In the case studies I have seen examples where researchers actually were able to transform hierarchical relationships into sustainable and successful partnerships. Patient involvement in OMERACT has led to a greater focus on issues that are important from a patient perspective and that derive directly from their everyday life. I have also seen examples where researchers were unable to start a constructive dialogue with research partners; Sometimes caused by restrictions imposed by the research system, and sometimes due to a lack of knowledge, practical difficulties or common

misunderstandings regarding patient participation.

With this thesis, I hope to have given some insights into the possibilities and limitations of patient participation in arthritis research. In addition, I hope that the presented cases will not only inspire researchers and research partners to continue on the path of dialogue and mutual learning, but also provide some practical tools and guidance to make participation a successful and enjoyable endeavor.