A large, stylized handwritten signature in black ink, appearing to read 'Femke Voorn'.

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Integrating patient preferences and patient knowledge in an evidence-based guideline

A qualitative research regarding patient participation

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Preface

In 2011 on the 29th of October the sixth annual lichen sclerosus patient conference was held and I learned that a lichen sclerosus guideline was under development. During lunch I had an intriguing conversation with two of the speakers, professor Dr. Allen McLean (Medical University UK) and professor Dr. Sigrid Regaur (Medical University of Graz). We were discussing the potential effects and benefits of a national Dutch lichen sclerosus guideline. After reviewing the Health Economics, Policy and Law thesis topic list I came across a potential interesting subject regarding guideline development. I immediately contacted the board of the lichen sclerosus patient organisation and they were willing to give me full and unrestricted access to all their information. They also offered me to come along with them to all the meetings during this guideline development process.

The development of the lichen sclerosus guideline involved an intensive and extensive (nearly three years) process containing tight collaboration with all involved workgroup-members. The period of my research covered 1,5 years from November 2011 until May 2013. By observing firsthand I have had an unique opportunity to explore the interactions and intensive proceedings between workgroup-members including the patient organisation. In all I have attended more than twenty meetings all throughout the Netherlands and have separately interviewed nine important stakeholders. I am very grateful the lichen sclerosus patient organisation offered me this unique opportunity. Also the support received from all the other actors during this process was heart-warming. Physicians, patients, my supervisor and family: many thanks for your time and effort.

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Summary

Background/objective: Patients have an important and increasing role in public decision-making processes. In guideline development, patients are thought to contribute from a different perspective than those of health care providers, researchers and policy-makers. However, from the literature it is known that patient participation shows disappointing results in practice. It is difficult to explicitly incorporate the patient perspective in an evidence-based methodology of guideline development. Although patients are facilitated with participation possibilities this does not automatically mean that they can influence the decision-making process. In this research the development process of the lichen sclerosis (LS) guideline -in which the lichen sclerosis patient organisation (Stichting lichen sclerosis, SLS) participated- is explored in dept. The objective of this thesis is to explore how LS patients can contribute (with their experiential knowledge and preferences) on the content of the LS guideline, how LS patients can influence the decision-making processes and in what way the produced knowledge of the guideline is justified. The main research question addressed in this thesis is: *How and to what extent are patient preferences and patient knowledge incorporated in the lichen sclerosis guideline?*

Methods: In an intensive ethnographic research project beginning from November 2011 until May 2013 various qualitative methods are used to collect empirical data. Overall, more than twenty meetings have been attended and observed all throughout the Netherlands. Nine important stakeholders have been interviewed. Moreover, formal documents, minutes, reports and email exchanges from the start of the guideline development process (January 2009) have been analysed.

Results: The results of this research are structured according to three concepts; participation, representation of LS patients and experiential knowledge. The concept participation clarifies the methods of patient participation within the LS guideline development process. The representation of LS patients defines the ways in which the LS patient has been represented and all the work that the LS patient representative has done in order to strengthen his role and tasks. In what way the produced knowledge is justified in the LS guideline is clarified by the concept experiential knowledge. Furthermore, Sherry Arnstein's participation ladder is used to help explain the contribution of different methods of patient participation. The framework of Moreira about the four repertoires of evaluation is used to explore and understand the different judgements about knowledge.

Conclusion: It proves to be difficult to methodologically justify and incorporate patients' preferences and knowledge in an evidence-based methodology of guideline development. This is due to the fact that all four repertoires of evaluation have their influences on the development of the guideline. It turns out that the technical robustness of the produced knowledge within the guideline and the methodological adequacy of the proceedings in which this knowledge is established prevents to fully incorporate patients' preferences and knowledge.

The formulation of starting questions for the guideline is an important tool to explicitly introduce the perspective of the patient. But the fact that starting questions are not (explicitly) answered does not mean that patients do not contribute with their specific knowledge and preferences or that the patient perspective is not taken into account during the guideline proceedings.

The explicit referral and quantitative measurement of the input of patients within the guideline is not an adequate measure of the contribution that patients have with their knowledge and preferences. Hereby it should be mentioned that the incorporation of LS patients' preferences and knowledge are most visible within the annex 'the patient perspective' due to the chosen methodology of the whole LS guideline process. By considering 'the patient perspective' in the annex, experiential knowledge from patients has certainly been incorporated in the evidence-based guideline, although looking at the guideline per se one could not ascertain that. The input concerning this specific knowledge within the LS guideline is due to the fact that the patient representative has been actively participating within the whole process. All the (in)formal work that the patient representative has done contributed to the extensiveness in which the guideline is patient-centred.

1. Chapter 1 Introduction

1.1 General overview

At the 1st of January 2006 the Care Insurance Act (*Zorgverzekeringswet, ZvW*) has come into effect in the Netherlands. The assumption behind this health care reform is that it will lead to efficiency and cost-containment due to the creation of market-oriented incentives. Instead of rules and regulations the focus of the government is more on guidance and monitoring. Through different policy instruments the government deregulates certain responsibilities and creates opportunities for (local) stakeholders to participate within public policy-making and decision-making processes. While the government has to provide the constitutionally embedded social rights of accessibility, affordability and quality of care the government is hereby dependent on various decentralised stakeholders throughout various sectors to facilitate these provisions.

As a result of ‘regulated competition’ the triangle between the government, health care providers and health care payers (insurers) changed. Within this system the patient has obtained a prominent role, next to health care providers and health care insurers. The current Dutch health care system is distinctive for its interdependencies between public, private and professional stakeholders. Patients have an increasing role in public policy-making and decision-making processes since ‘regulated competition’ led to a switch from a supply side to a demand-driven health care system. The increasing role of patients is assumed to give the patient on the one hand more control on the care they receive and on the other more influence to demand the care they want. Therefore, the care provided is assumed to be responsive to the demanded needs and preferences of the patients (Van der Kraan and Van der Grinten 2004: 3).

Hereby, it is not surprising that modern health care is governed by the patient-centred medicine paradigm that is directed at patient participation in decision-making processes (Bensing 2000: 19). The overall assumption is that patients affect decision-making processes with their active participation (ibid.: 17). Patients can participate in health care policy- and decision-making processes in a number of ways and on a number of levels (individual/collective, micro/macro). On the individual level this is assumed to take place through a shared decision-making process between patients and physicians (Van de Bovenkamp and Trappenburg 2009: 198-199). The perspective of the patient should be taken into account whereby medical care should be tuned on patient preferences and needs (Bensing 2000: 17). One specific policy-making process that is becoming more patient-centred too is the development of guidelines. Patient participation on this level takes place via patient organisations whereby representatives represent the collective interests of (fellow) sufferers. Patient participation within a guideline development process is the focus of this thesis.

Participation can be related to the term involvement (Caron-Flinterman 2005: 17). A distinction can be made between active and passive involvement. Active involvement refers to the direct participation of the patient with the assumption that the patient owns sufficient knowledge to contribute to decision-making processes (ibid.: 17). Direct participation implies that participation activities cannot be delegated and that the patient is actually present. Moreover, the decision-making processes in which patients are facilitated to participate should make sure that the contributed knowledge is integrated in the outcome of the process (ibid.: 18). The aforementioned definition of active involvement is used in this thesis and is referred to as active (patient) participation. On the contrary, passive involvement means that the patient lacks sufficient competence and knowledge to adequately take part in decision-making processes (ibid.: 17). Passive (patient) participation is, in this thesis, defined as any (in)direct method in which patients participate in guideline development processes whereby patients lack the power to ensure that it is acted upon or whereby the contributed knowledge is not integrated in the outcome, the guideline.

With active patient participation in guideline development processes, patient representatives can express and introduce (collective) preferences of patients; the patient perspective. The assumption is that this knowledge can be used as an input in decision-making processes which enhances the quality

of the decisions (Van de Bovenkamp and Trappenburg 2009: 205). When talking about active patient participation the guideline development process is led by patients' preferences which are subsequently incorporated in the outcome, the guideline. This assumes that active patient participation in a guideline development process will lead to a patient-centred guideline, a guideline in which the patient perspective has been adequately used as input.

There is little research available about patient participation within guideline development processes and most research has focussed on whether or not patient participation is adequately effectuated in practice. From the existing literature we *do* know that the added values of patient participation shows disappointing results in practice, mainly due to all the difficulties it entails bringing patient participation in practice (Van de Bovenkamp et al. 2013: 7, Zuiderent-Jerak et al. 2011: 57). For instance, participating as guideline workgroup-member can bring along some complications. In the study done by Van de Bovenkamp et al. (2013: 10) patient representatives reported on not being acknowledged as equal partner since they were not able to conform to the evidence-based methodology of guideline development. Furthermore, it proves to be difficult to determine what happens to the input of patients within the guideline itself, since it is often not explicitly visible or referred back to patients (Zuiderent-Jerak et al. 2011: 57). It appears that the knowledge of patients is problematic to incorporate in an evidence-based methodology of guideline development (Van de Bovenkamp and Trappenburg 2009: 209, Zuiderent-Jerak et al. 2011: 57). The unique insight in the process of incorporating patients' preferences and knowledge into an evidence-based guideline and in what way the patients' perspective plays a role in the whole development process is the key focus of this research.

1.2 Case

The specific guideline development process that is researched is the chronic, inflammatory skin condition called lichen sclerosus (LS). The precise cause of this condition is unknown but heredity and auto-immune factors seem to play a role (NVDV 2012: 13). Affected cells lose their elasticity, making the skin very thin, tight and glossy. LS is therefore also known as the white spot disease.

LS can affect both men and women, adolescents and children. The female-male ratio varies between 10:1 to 1:1 (ibid.: 11). The estimated prevalence is between 1:60 and 1:1000 in adults (ibid.: 11). It can affect anywhere on the body but mainly occurs in the anogenital area. Due to loss of elasticity of the skin this condition is characterized by a sclerosis that causes itching and pain. Scarring leads to destruction and change of anogenital (skin) architecture. This may cause fusion of the labia, burying of the clitoris, narrowing of the vaginal opening and tightening of the foreskin (ibid.: 12). There is no curative treatment for LS and every treatment is concerned with reducing the symptoms, for instance relief of pain and itching. Most common treatment is the frequent use of corticosteroid ointment and the daily use of crèmes. While the current treatment may prevent further damage, any scarring that already has occurred will remain (Chi et al. 2011: 4). Moreover, the current treatment often causes adverse effects like corticosteroid-induced skin atrophy¹ (ibid.: 4).

LS has a significant negative impact on the quality of life and indicates significant sexual dysfunction and sexual distress (Van de Nieuwenhof et al. 2010: 281). Furthermore, the development of squamous cell carcinoma of the vulva during life is 4% to 5% for women with LS (MacLean et al. 2009: 115). From the limited scientific studies done the risk of squamous cell carcinoma of the penis is estimated on 9,3% (NVDV 2012: 18).

At the 27th of September 2006 the patient foundation lichen sclerosus (Stichting Lichen Sclerosus, SLS) has been established. The SLS has multiple goals mainly: equipping patients with information, supporting patients emotionally and getting more awareness for this chronic skin condition. The SLS initiated a secured internet forum where forum members (LS patients) are able to exchange knowledge and more importantly to seek peer support. This is framed by mutual experiences that enables patients to find ways of addressing their similar problems.

¹ Skin thinning.

In the year 2009 the SLS took the first steps in the development of a Dutch LS guideline. This development process is explored in depth and provides the empirical data for this research.

1.3 Objective and relevance

In existing research about patient participation less attention is paid on the questions how and to what extent patients can contribute (with their experiential knowledge and preferences) on the content of guidelines, how patients can influence the decision-making processes and in what ways (experiential, expert and evidence-based) knowledge is justified. Therefore, it is interesting to explore the process and outcome of a guideline development process in depth. The LS guideline is relevant in particular since the lichen sclerosus patient organisation is very active with (board) members (patient representatives) who initiated the guideline process themselves. In this case the extent of having an influence on the process is presumed to be larger than has been seen and explored so far.

The objectives of this research is to explore how LS patients can contribute (with their experiential knowledge and preferences) on the content of the LS guideline; how LS patients can influence the decision-making processes and in what way the produced knowledge of the guideline is justified.

In this intense research project of 1,5 years the different participatory methods in the LS guideline development process are explored. The experiences of professional workgroup-members with the participation of patients are thereby taken into account that broadens the picture of patient participation in practice. The activities and (inter)actions of the patient representative are observed in order to explore how (in)formal proceedings have an effect on the official guideline proceedings and the remaining tasks of the representative. Furthermore, the process of knowledge transfer between workgroup-members is examined that can help explain how the input of patients is justified within the guideline.

1.4 Research question

Taking the relevance and the objective into account the main research question addressed in this thesis is:

How and to what extent are patient preferences and patient knowledge incorporated in the lichen sclerosus guideline?

1.5 Outline thesis

In the above, an introduction is given about patient participation. Chapter 2 proceeds by outlining a theoretical perspective on patient participation within guideline development processes. At the end of that chapter three sub questions are provided which assist with answering the main research question. After an explanation of the research design in chapter 3 the different methods used to explore this research empirically are clarified. Chapter 4 outlines the most important and prominent research results. These results will be described on the basis of three concepts being (1) participation, (2) representation of LS patients and (3) experiential knowledge. The conclusions in chapter 5 connects the research results with the theoretical perspective to answer the three sub questions. Consequently, an answer will be given on the main research question. Hereafter the implications and limitations of this research are described in the discussion. Thereafter the references are listed after which the appendixes are used for further background information.

2. Chapter 2 Mind the patient

2.1 General overview

This theoretical chapter provides for an insight into the academic debates about the essential concepts that are used in this thesis. The next section gives an overview of how patient participation is facilitated through institutionalised reforms in the Dutch health care system. After that the added values of patient participation are clarified. In the fourth section the ways in which patient participation is currently shaped in practice are described. Unfortunately there are also disappointing research results about patient participation. That the main goals of patient participation can be jeopardized is explored in the fifth section. The subsequent section argues whether or not more participation is better. Going beyond the discussion of patient participation per se, the seventh section explores how every workgroup-member, including patients, draw upon different forms of judgement to construct knowledge and clinical guidance. In the eighth and last section three sub questions are formulated.

2.2 Facilitating patient participation

Patient organisations have an important and ever increasing role for active involvement in public decision-making processes because they are recognised as a legitimate third party by the Dutch government and other parties (Grit et al. 2008, Van Rossum et al. 2008, Van de Bovenkamp et al. 2008a: 9). By breaking through institutional barriers the government enables, creates and facilitates conditions for patient participation in a number of ways. The most dominant elements in the Netherlands will be outlined next.

Firstly, in the 1990s there has been a strong focus on introducing patient laws to strengthen the position of patients (Van der Kraan 2006: 117-124, Van de Bovenkamp et al. 2008a: 9). This is a legitimate basis for patient involvement. Especially with the health care reform in 2006 whereby the system of regulated competition with the *ZvW* came into effect in which: "the patient – the insured party – really occupies centre stage" (Ministry of Health, Welfare and Sport 2006: 3).

Secondly, through direct state funding as well as via indirect subsidisations patient organisations are able to professionalise themselves (Schipaanboord et al. 2011: 116). In 1996 the patient foundation (Stichting Patiëntenfonds) later known as foundation PGO (Fonds Patiënten- en Gehandicaptenorganisaties Ouderenbonden, PGO², Fonds PGO 2013) was established (TK 2002a). Structural subsidisation for activities as facilitating information, representation of patients and peer support are provided by PGO. Moreover, PGO tries to strengthen patient organisations through provision of subsidisations on a project basis (Van de Bovenkamp et al. 2008b: 15).

Thirdly, in 2000 the Health Council of the Netherlands³ (Gezondheidsraad, Gezondheidsraad 2013) has proposed active patient involvement in the development process of guidelines (Gezondheidsraad 2000: 45, Van de Bovenkamp and Trappenburg 2009: 199). From that moment on institutions who are burdened with the development of guidelines have actively tried to facilitate possibilities for patients to participate in this process (Van Veenendaal et al. 2004: 61-62, Schipaanboord et al. 2011: 114, Van de Bovenkamp and Trappenburg 2009: 199).

Fourthly, as from the 1st of January 2013 the Quality Institute⁴ (Kwaliteitsinstituut, CVZ 2012a) is established (TK 2012). The Kwaliteitsinstituut puts a strong focus and emphasis on the patient perspective. It has established six 'golden questions' to determine to what extent the patient perspective is introduced in quality standards and instruments (Delnoij 2012, CVZ 2012b). The notion on quality instruments is that when all health care parties are involved with designing, implementing and evaluating guidelines the quality of care will increase, health care expenses decrease and the care provided tuned on the demands and preferences of the patient (CVZ 2012c).

² Foundation PGO provides subsidies to nationwide patient organisations, foundations for disabled persons and associations for elderly people in mandate of the Ministry of Health, Welfare and Sport.

³ The Dutch Health Council is the independent scientific advisory board of the Ministry of Health, Welfare and Sport.

⁴ This Quality Institute facilitates uniformity among all actors in the Dutch health care system (health care insurers, health care providers and health care users) about quality measurements and instruments.

Patient participation is shaped through the above mentioned institutionalised reforms on a macro level. The main goals behind patient participation are outlined in the next section.

2.3 The added values of patient participation

Patients have an increasing participative role in public policy-making processes as a consequence of the aforementioned institutional reforms. As a direct result the involvement is materialized in the (active and) visible participation of patients at different policy tables. There is a growing consensus that patients' experiences are a valid source of knowledge (Van de Bovenkamp et al. 2008b: 33, De Wit 2008: 991, Abma 2005: 1311). Patients acquire experiential knowledge through empirical-based experiences. Physicians own experiential knowledge too though the experiential knowledge from patients is the focus of this thesis.

In guideline development patient participation will contribute on a different perspective besides those of health care providers, health care researchers, methodologists and policy-makers (Van de Bovenkamp et al. 2008a: 16, 34, Abma 2005: 1313). This different perspective is fundamental for the patient perspective. Experiential knowledge is of a different perspective due to the fact that patients exist in several social worlds (Barbot 2006: 539). The condition is just one aspect of the life of patients. Besides the condition a lot of other important domains like work and sexual functioning are determining the life of patients. Patients who are chronically ill can contribute with their own empirical-based experiences as sound source of knowledge to the decision-making process.

Experiential knowledge provides alternative views on conditions and treatments and therefore has a specific contribution to the decision-making process. The advantage of the required experiential knowledge from patients to establish better policy on care has been acknowledged by other parties (Van de Bovenkamp et al. 2008b: 33, Schipaanboord et al. 2011: 118, De Wit 2008: 991, Abma 2005: 1311). Patient participation is assumed to increase the quality of the guideline, the applicability of the guideline in practice and the quality of care (Van de Bovenkamp and Trappenburg 2009: 205, 211, Broerse et al. 2010: 25). The practical reasoning for patient participation is therefore an increase in effectiveness and quality of decisions.

Patients are along with physicians the most prominent users of and the ones directly affected by guidelines. Thus it seems fair that they should have a voice in the development process (Van de Bovenkamp and Trappenburg 2009: 205). Participation of patients increases the legitimacy of (the recommendations in) the guideline (ibid.: 205). The principle-based reasoning for patient participation is therefore that patient participation contribute to the democratisation of the policy- and decision-making process (Van de Bovenkamp et al. 2013: 1).

The goals of patient participation within guideline development are mainly two-folded and classified as resulting in better quality of guidelines and enhancing the democratic process in which decisions are made. Confronted with this participative task the individual patient is assisted by one's patient organisation that represent the collective interests. The representation of patients is an important task of patient organisations, next to facilitating information and peer support. Within guideline development patient organisations play an important role in participation on the collective level. Patient representatives represent the needs and preferences of their (fellow) sufferers. Moreover, they are assigned the third party role by the Dutch government (Van de Bovenkamp 2010: 16). This is increasingly put into practice and can take place in various ways as is discussed in the next section.

2.4 The ways in which patient participation is shaped in practice

Broerse et al. (2010: 18-23) have assessed different ways of patient participation. Patient participation can take place in different forms and in different stages of the guideline development process (ibid.: 18-23). Forms in which patient participation can take place are for instance participation in development workgroups where representatives, whether or not burdened with the condition themselves, are physically present among the workgroup-members. This is the most common used

method of patient participation within guideline development processes (Broerse et al. 2010: 18, Van de Bovenkamp and Trappenburg 2009: 207).

Focus groups can be used as sole participation method but is mainly used as complementary method (Broerse et al. 2010: 18-19). Through the use of focus groups an understanding of patient preferences can be obtained. This method is mostly seen among guideline development of chronic conditions since patients with chronic conditions are more organised in collaboration and are more committed to their patient organisation. These patients are therefore easier to approach and seem more willing to participate within guideline development processes (ibid.: 16).

Questionnaires are also a method to explore patient preferences. A generalization of preferences can support the representative in putting the patient perspective forward, especially when such information is not available within scientific evidence (ibid.: 7-8, 19). The search for (scientific) literature is also a method wherein patients can participate although in practice it is not common that patients do (Broerse et al. 2010: 7, Van de Bovenkamp and Trappenburg 2009: 207). Give feedback on draft versions of the guideline is often seen in practice as a method of participation (Broerse et al. 2010: 19).

In a guideline development process multiple stages can be distinguished: the start of the process; analysing the bottlenecks and formulation of the starting research questions; literature search and assessment; the judgement of results and formulation of recommendations; the draft version of the guideline and the authorisation of the guideline (ibid.: 20-22).

The start of a development process begins with the application of the guideline and the formation of the workgroup. When a patient organisation is the initiator of the process it automatically means that they are involved from this stage on. It also implies that the patient organisation is the one composing the (members of the) workgroup (ibid.: 20). Nevertheless, the analyses of bottlenecks is often the first phase and frequently the only phase, in which patients participate actively (ibid.: 20). Patients can introduce their own bottlenecks or reflect on bottlenecks that are already formulated by the workgroup. Focus groups are a method to assess bottlenecks from a patient perspective and to involve these in the starting research questions. The analyses of bottlenecks and the formulation of starting research questions are regarded as the most important methods to involve the perspective of patients within the guideline development methodology (Broerse et al. 2010: 57, Zuiderent-Jerak et al. 2011: 6).

The research done by Broerse et al. (2010: 20-21) revealed that patients are not involved in the literature phase in the majority of the investigated guideline processes. The literature search is never the only phase in which patients participate and is generally not considered as active participation activity (Broerse et al. 2010: 20-21, Van de Bovenkamp and Trappenburg 2009: 207). Since patient representatives are a member of the guideline workgroup they are often present during the phase in which results are judged and recommendations are formulated. Though, this does not mean that specific activities are set up to involve patients. Often the experiential knowledge is only used in the remainder (non evidence-based) recommendations (Broerse et al. 2010: 21, Van de Bovenkamp and Trappenburg 2009: 208).

Commenting on draft versions of the guideline is the other phase besides the analyses of bottlenecks in which patients most often participate actively (Broerse et al. 2010: 21). Patient (representatives) can give comments on the draft version of the guideline from a patient perspective. Authorisation is done by the involved professional associations to gain public support among the different professional associations who will use the guideline in practice.

The forms of participation and the stages in which patient participation takes place varies with every guideline. Various factors influence to what extent patient participation is present in guideline development processes. With multidisciplinary guidelines patient participation is more common in comparison to mono-disciplinary guidelines (Broerse et al. 2010: 16, Zuiderent-Jerak et al. 2011: 56). Mono-disciplinary guidelines are for instance the guidelines (standards) from the Dutch Association of General Practitioners (Nederlands Huisartsen Genootschap, NHG). These NHG-standards hardly make use of patient participation (Broerse et al. 2010: 16).

Other factors of influence are characteristics of the condition, characteristics of the patient organisation and boundary conditions. Patients with chronic conditions are more often organised in collaboration and therefore more often participating in guideline development processes in comparison with patients with acute conditions. Nevertheless, the severity of the condition can complicate active participation (Broerse et al. 2010: 16-17). Sometimes severe chronic conditions are so burdensome that participation is seriously hindered.

Among the characteristics of patient organisations belong the size of the organisation, the professionalization of the organisation and prioritization (ibid.: 17-18). As the size of the patient organisation becomes larger the more easier it becomes to fulfil participation activities. Features of patient organisations that are professionalized are the availability of knowledge, skills and resources. The availability of these features seems related to the size of the patient organisation; the larger the organisation the more (financial) resources are available, the more professionalized they can become. A close relationship with professionals is also related to professionalization of patient organisations. And of course, the more patient organisations prioritize participation possibilities in guideline development processes the more they are willing to make resources available. The availability of financial resources is an important boundary condition for patient participation since the formalization (of patient participation activities) is dependent on the amount of financial resources (ibid.: 17-18).

That patient participation varies among different guideline development processes is predominantly caused by the aforementioned factors that influence the extent in which patient participation is shaped.

Patient participation can take place in various ways and the experiential knowledge from patients can be used in many stages of the guideline development process. The Dutch Council for Quality of Healthcare⁵ (Regieraad Kwaliteit van Zorg, Regieraad, Regieraad Kwaliteit van Zorg 2013) has published the 'guideline for guidelines' (Regieraad Kwaliteit van zorg 2012). During the process of guideline development criteria have to be taken into account since guidelines have to meet certain requirements. The Regieraad has published the third edition of the guideline for guidelines in 2012.

The definition of a guideline has been changed in the second edition of 2011, based on the definition of the Institute of Medicine (IOM). This definition implies that the stated recommendations within a guideline, aimed at improving the quality of care, are based on a systematic review of scientific evidence and a consideration of the likely benefits and harms of different treatment options *supplemented* with the expertise and experiences from health care professionals and health care users (Regieraad Kwaliteit van zorg 2012: 5, Institute of Medicine 2011: 18). The latter being caregivers, (family of) patients and clients. So the fact that scientific evidence as main input for recommendations in guidelines has to be supplemented with expert *and* experiential knowledge was a formal and official fact in 2011. Moreover, one of the seven criteria of the preparation phase has been changed in 2012, in comparison with the text of 2011. This is the explicit mentioning that the patient perspective is part of the guideline (Regieraad Kwaliteit van zorg 2012: 8) instead of "health care users are involved with the development and maintenance of the guideline" (ibid.: 12). Though, the definition of this criteria is indistinct which leaves much room for own interpretation and execution in practice. All the above mentioned forms of patient participation are described as possible participation methods. The 'guideline for guidelines' mentions that patient participation is desirable at the start of the process with analysing the bottlenecks and formulation of the starting research questions; the determination of outcome measures; the formulation of recommendations and the comment phase (ibid.: 8).

Although the added values, the goals, of patient participation seems self-evident recent research discussed next also shows opposite results. These disappointing results are the focus of the following section.

⁵ The Dutch Council for Quality of Healthcare is established by the Ministry of Health, Welfare and Sport. This Institute brings out advisories about the Dutch health care system both uninvited as well as in request of the Ministry.

2.5 Disappointing research results about patient participation

As defined in section 2.2 patients are more recognized and involved as a legitimate third party in guideline development processes. The Dutch government facilitates patients with the opportunity to (actively) participate and consequently patients are expected to do so (Van de Bovenkamp 2010: 14-15) for reasons explained in section 2.3. This brings along both rights and obligations (ibid.: 18). Nevertheless, it can be questioned if patients are able and willing to perform this task.

There are certain skills (education level, the condition, age) on the basis of which can be assessed if patients (representatives) are adequately able to fulfil the participatory role (Broerse et al. 2010: 27-28). Since not every patient fit the ideal profile the danger is an unequal distribution in the quality of care favouring the most well-off people (Van de Bovenkamp 2010: 19). Furthermore, not all patients want to be involved in all stages of the guideline development process. Some patients want to have a voice when reviewing scientific evidence since they clearly understand this literature. Others are satisfied with their focus on the non evidence-based aspects of the guideline. An example will illustrate this situation.

The research of Broerse et al. (2010) was focussed on patient participation in five guideline development processes. During the guideline development process of cancer-induced pain⁶ the patient representative was not able to participate in medical discussions. Since the representative was not well grounded in scientific evidence and medical jargon she found this self-evident and not a problem at all (Broerse et al. 2010: 33). The fact that the representative was not involved with the literature search and medical discussions and that she was fine with that, does not eliminate the fact that she therefore could not assess the quality of this process and the outcome. This could affect the goal of improving the effectiveness and quality of the guideline.

Another important reason why patient participation can be disappointing is that due to their chronic conditions patients are not able to cope with the responsibilities (Van de Bovenkamp et al. 2013: 1-2). Not being able to voice hinders a democratic decision-making process and the reasons why patients were asked to participate for in the first place. In practice the goals of patient participation could therefore be jeopardized.

Let's assume that patients are willing to participate in the whole guideline development process and that their condition does not hinder them. Although the importance of patient participation is acknowledged by other parties it is often unclear what happens to the input of patients. It is difficult to assess whether or not the perspective of patients is used and involved in the guideline. The patient perspective can eventually be translated in the guideline in different ways. Most often it is seen in different sections of the guideline (education and communication) besides the main evidence-based medical recommendations (Broerse et al. 2010: 23). Sometimes an individual chapter is dedicated to the patient perspective but more often it is not explicitly referred to in the guideline (Van de Bovenkamp 2013: 8). The added values of patient participation are thus not easy to determine. That is why it is frequently questioned whether or not patients are able to participate actively and effectively.

The fact that the input of patients is not explicitly incorporated or mentioned in guidelines does not automatically mean that patient involvement was out of the question (Zuiderent-Jerak et al. 2011: 18, 57, Van de Bovenkamp et al. 2013: 4). Experiential knowledge could be incorporated in the guideline without a specific notification that it concerned the input of patients (Zuiderent-Jerak et al. 2011: 18, Van de Bovenkamp et al. 2013: 8).

That it is often unclear what happens to the input of patients is above all dependent on the specific knowledge and the methods that are focussed on to generate guidelines. Before going into the methodology of guideline development let's first start with the knowledge that is used to develop guidelines.

An important challenge with bringing patient participation into practice is the knowledge that is used to develop guidelines. There are three different sources of knowledge that can be distinguished in

⁶ Richtlijn pijn bij kanker.

guideline development processes. In hierarchical ordering these are scientific evidence, professional expert knowledge and patient experiences respectively (Broerse et al. 2010: 26).

Evidence-based medicine (EBM) can be defined as “the conscientious, explicit, and judicious use of current best evidence in making decisions about the care of individual patients” (Sackett et al. 1996: 71). Within EBM a hierarchy of studies exist in terms of the strength of evidence they provide (Dopson et al. 2003: 312). Randomized controlled trials (RCTs) are seen as the golden standard for determining effectiveness of treatments (Timmermans and Berg 2003: 27). Moreover, RCTs are focussed on determining the causes of conditions and they are the main input for evidence-based guidelines (Gezondheidsraad 2000: 31).

Physicians are autonomous experts. They bear professional expert knowledge. Due to years of education and medical expertise physicians can be relied upon to make individual choices and to act in the best interest of patients (Timmermans and Mauck 2005: 23). Physicians’ transfer of knowledge is primarily focussed on the biomedical model (focussed on diagnoses and treatments), which is the conventional way of doing medicine combined with individual clinical expertise (and experiential knowledge).

With having to live with a chronic condition patients build up experiential knowledge and become an expert in knowing their preferences. Moreover, the knowledge from patients consists of and is focussed on the symptoms of the condition in comparison with scientific evidence that is focussed on the causes of the conditions. Since preferences and needs are mostly not grounded on scientific evidence this is the least valued knowledge (Broerse et al. 2010: 26). Furthermore, preferences are very individual thus generalization to a whole patient group is difficult. The patient perspective is therefore easily put aside.

EBM has led to an evidence-based methodology of guideline development. The former consensus-based (Gezondheidsraad 2000: 18, 30, Turner et al. 2008: 46) developing method has underwent changes in so far that the focus has shifted away from the formation of consensus to the weighing of evidence (Zuiderent-Jerak et al. 2011: 39). The EBM methodology requires scientific articles (evidence) to be searched for, to be critically appraised and discussed whereby eventually the stated recommendations are a tool to support daily medical practice being clinical guidance. This systematic methodology to determine best practice is difficult to combine with the heterogeneity of preferences and needs from patients. This could lead to a clash between the different kinds of knowledge that is brought in between the actors of a guideline workgroup (Van de Bovenkamp et al. 2013: 9). Eventually the power to influence the decision- and policy-making process will decide which knowledge is used and which knowledge is put aside. Research show that in this situation patients are in a disadvantageous position (ibid.: 10). Although patients are facilitated with (legalised) participation possibilities this does not automatically mean that they can influence the decision-making process too. Whether or not patients should therefore participate more and more, is the focus of the next section.

2.6 More participation is better...or not

The disappointing research results about patient participation, as discussed in the previous section, has led to authors arguing that more effort should be put in patient participation possibilities (Arnstein 1969, Caron-Flinterman 2005, Broerse et al. 2010). After all ‘more (participation) is better’ as is argued by the ladder of (citizen) participation of Arnstein (1969). This participation ladder was initially meant to reflect the level of citizen involvement but this framework is also applied to distinguish different degrees of patient participation (Caron-Flinterman 2005: 18, Broerse et al. 2010: 7). Eight levels of participation can determine to what extent patient involvement leads to power in determining the end product (Arnstein 1969: 217). The higher on the ladder of participation the more influence of the patient is guaranteed and the more mechanisms for true power distribution.

Three different gradations of patient involvement (participation) can be distinguished: non-participation, pseudo-participation and citizen power (ibid.: 217). The two lowest rung of the ladder are manipulation and therapy (ibid.: 218). It reflects to non-participation and is therefore not addressed further. Information, consultation and placation are the fourth, fifth and sixth rung and they reflect pseudo-participation (Arnstein 1969: 219-221). In this level of passive participation patients have a

voice but they lack the power to ensure that it is acted upon. There is no redistribution of power and 'professionals' being for instance researchers and policy-makers decide whether or not the input from patients is included in the decision-making process (Caron-Flinterman 2005: 19). The methods of patient participation within this level are for instance questionnaires, interviews or focus groups among patients (Caron-Flinterman 2005: 19, Broerse et al. 2010: 7-8). Patients who are participating in the guideline workgroup but either cannot follow or interact in medical discussions or their voice is not acted upon also reflects pseudo-participation. The three highest rung of the ladder are partnership, delegated power and citizen control and they reflect to citizen power (Arnstein 1969: 221-223). Within these levels of participation power can be redistributed and decision-making responsibilities can be shared (ibid.: 221). Except for a few, there are hardly any examples of guideline development processes known in which patients were actively participating as partners (Broerse et al. 2010: 9).

Since participating is not the same as having an influence on the process and since patients are in a disadvantageous position they should climb up the rung of the participation ladder and participate more (Arnstein 1969, Caron-Flinterman 2005: 159). The notion of 'more (participation) is better' is further emphasized in the argument that patient organisations should professionalize themselves (more). Professionalization was even made a main task of patient organisations next to facilitating information, representation of patients and peer support on the basis of which subsidies were assigned (TK 2002b, TK 2007). But the focus on 'more is better' is within the existing literature also criticised.

Within guideline development patients can be trained to become full 'pseudo professional' members of the guideline workgroup. But this brings along a paradox: while more professional representatives could enhance the effectiveness of the guideline development process one can wonder if it enhances the outcome of the process too (Van de Bovenkamp 2010: 158). When patients engage in medical discussions and can follow the EBM guideline development process easily, they are taken more seriously by the workgroup-members. At the same time, it is argued that the tasks of representation and the input of experiential knowledge that should deliver better quality guidelines could be questioned (Van de Bovenkamp 2010: 158, Van de Bovenkamp et al. 2013: 11-12). While professional representatives can contribute to complicated discussions it alienates them from the representation of patients and the contribution with experiential knowledge (Van de Bovenkamp 2010: 158). Both goals of patient participation therefore do not necessarily go hand in hand with more (professionalized) participation as is argued by Van de Bovenkamp (2010: 158, Van de Bovenkamp et al. 2013: 15).

Let's go beyond the discussion about the levels of patient participation per se and the increased influence patients might have with participating more, and broaden the debate into what patients can contribute on the content of the guideline as a result of participation. Experiential knowledge is build up through experiences; experiences about having to live with a chronic condition; its symptoms and its treatments. Incorporation of this specific knowledge into guidelines creates guidelines that are better applicable to patients in practice; that creates more support for the users of the guideline and that let physicians understand what the condition implies. In sum and as discussed in section 2.3 patient participation increases the quality of the guideline. In this context, the discussion is not about participating 'more is better' per se and in this strict format. The different methods of patient participation, as discussed in section 2.4, each can have a different contribution. Participating as guideline workgroup-member is considered to be better, according to the participation ladder of Arnstein, since it is higher on the ladder in comparison with a questionnaire. But this need not be the case. An example will illustrate this situation.

Studies on client councils within different health care institutions show that representatives are increasingly expected to participate with adequate skills and expertise (Trappenburg 2008: 163). Representatives should have a say in every matter, both the common issues as well as policies. They should understand and discuss annual accounts too and can even be trained to do so. However, all the time spent talking about policies is time forgone discussing about common issues. And neglecting these common issues that representatives are supposed to deal with as well, is neglecting the things that matters most for clients, especially for residents of a nursing home (ibid.: 175). In that case a questionnaire could deliver an assessment of most important elements that should be taken into account instead of focussing on professional participation with adequate skills and expertise.

The incorporation of experiential knowledge is important and this can in turn play more or less an important role in the guideline development process and in the end product. From this it can be said that the added values of different patient participation possibilities depends on the setting that patients are asked to participate in.

The next section explores how workgroup-members, including patients, draw upon different forms of judgements to construct knowledge and clinical guidance.

2.7 The process and interactions within guideline development

When guidelines are developed by a multidisciplinary workgroup of researchers, policy-makers, methodologists, physicians and patients they form a group of stakeholders each with their own expertise and knowledge. The EBM guideline methodology therefore unite the activities, the expertise and the knowledge to produce recommendations that are evidence-based, scientifically valid, acceptable to users and feasible to implement (Pagliari and Grimshaw 2002: 145). Thus, recommendations not only have to be scientifically-proven. The workgroup should also assess and incorporate the consequences of the recommendations for daily medical practice. The definition of a guideline given by the Regieraad (and IOM), stated in section 2.4, explicitly mentions that scientific evidence has to be supplemented with the expertise and experiences from both physicians and patients (Regieraad Kwaliteit van zorg 2012: 5). Hereby, specific acknowledgement is given to the importance of experiential knowledge. Each individual actor of the workgroup has an important task contributing with his/her own specific perspective. Before exploring the process of justification of knowledge by each workgroup-member let's first go into the discussion why EBM guidelines have become so popular.

EBM is considered to have the alleged effects of controlling medical costs and improving quality of care due to a reduction of practice variation and uncertainty (Timmermans and Kolker 2004: 177, Timmermans and Oh 2010: S98). This is based on the underlying rational assumption of science, which is the main input for EBM guidelines, stating that integrating scientific evidence together will automatically lead to physicians adopting those recommendations in daily practice (Timmermans and Mauck 2005: 23, Timmermans and Oh 2010: S99). However, studies have shown that there is ambiguous evidence on the assumption that guidelines are an effective tool to change physicians' behaviour. This is in line with a study done by Grilli and Lomas (1994: 206) that found a compliance rate of 50% out of the 143 recommendations studied. A Dutch study done by Grol et al. (1998: 858) showed a compliance rate of 61% out of 47 different recommendations. This average dropped to 44% if the recommendations demanded a change in practice.

These disappointing effects of guidelines in practice is often an object of study. The so-called "implementation gap" is a consequence of the lack of congruence between guidelines (with the main input of scientific evidence) and daily medical practice (Dopson et al. 2003: 317). The complexity of individual patients cannot be captured fully in guidelines and this is one of the reasons why physicians question EBM that underpins guidelines (Timmermans and Oh 2010: S98). The biomedical model that underlies EBM results in setting up predictable and generalized diagnoses and treatments for patient groups with the same defined characteristics in clinical trials (Dopson et al. 2003: 324). RCTs and systematic reviews assume to provide with certainty what counts as best practice for all relevant patients (ibid.: 324). By encouraging to use guidelines that reduce clinical practice variation, medical practice can be characterized as cookbook medicine (ibid.: 312).

In daily medical practice physicians cannot follow a recipe when making clinical judgements (ibid.: 312). Patients are for instance confronted with co morbidity and multiple medical prescriptions. Some recommendations in guidelines could therefore have a conflicting or counter reacting and even a negative effect for an individual patient. Physicians are well aware of the uncertainty and complexity of clinical decision-making. This is also one of the reasons why a negative attitude from physicians towards guidelines can occur (Siriwardena 1995: 646, Timmermans and Oh 2010: S98). The heterogeneity of patients make extrapolation of scientific research to a wider population group difficult (Dopson et al. 2003: 321). Physicians therefore integrate their individual clinical expertise that they acquire in medical practice with the judgement of external evidence from scientific systematic

research and the experiential knowledge from themselves and from patients as well. The consideration of different types of knowledge from physicians in practice to support clinical guidance is one step in the direction to understanding the implementation gap (Moreira 2005: 1976).

The implementation gap per se is not the subject of this research although it is important to know the alleged effects of guidelines in practice since this affects the justification of the knowledge that is used within guideline development processes. As discussed in section 2.5, EBM provides for a hierarchy in the strength of scientific evidence. Incorporation of experiential knowledge will lead to better quality guidelines and in the assessment of scientific studies, experiential knowledge is used as an input in the discussions. But within the official guideline documents, experiential knowledge mostly disappears.

Evidence-based and scientifically valid recommendations in guidelines are not self-evident. The status of evidence is not only founded on the scientific articles which underpins the recommendations but also on the users and the interpreters from the evidence (Berkwits 1998: 1542). This means that the same recommendation can be interpreted and used differently in practice by two physicians since the evidence is judged differently by different actors. The workgroup-members who develop guidelines have to take into account that the users of the guideline in practice evaluate and judge the policy differently (Boltanski and Thévenot 2000: 216) and consequently act differently. But each actor of the workgroup connects the guideline and the world in a different way. This account for a plurality of judgements of the relationship between knowledge and the social action that this knowledge consequently leads to (Moreira 2005: 1977). Moreira (2005: 1976) argues that “clinical guidance comes to existence through the combination of repertoires of evaluation of knowledge”. There are four types of collective judgement on the basis of which multidisciplinary development groups corroborate clinical guidance (ibid.: 1976). These are (1) the technical robustness of knowledge; (2) its practical usability; (3) its political acceptability and (4) its methodological adequacy (ibid.: 1976).

The reliability of knowledge which is produced through science is judged by the technical robustness of the statements. The statements have to be supported with a certain amount and quality of evidence in order to reduce its possible resistance or critique from the outside world (ibid.: 1978). This is important because through the guideline the workgroup see themselves as accountable to the outside world (ibid.: 1977). The construction of a robust statement is a process in which the statement is evaluated in light of current medical practice. To support the strength of the statement and hence the reliability of the knowledge produced, it is important that enough data (scientific articles) are available with an adequate sample size (ibid.: 1979).

With the focus on science it is not surprising that this repertoire of evaluation is dominated by the researchers and policy-makers and to a lesser extent by physicians and patients. In this process researchers, policy-makers and methodologists enter into an exchange of cooperation. At the same time the discussions can be characterised by conflict whereby physicians can doubt the presented evidence due to their clinical expertise. According to Moreira (2005: 1979) patients would have a more passive role in this process and mostly listening. But as discussed before there are also patients who can (actively) participate in the discussions about scientific evidence; who have no difficulty with the medical jargon that is used and who are well informed about the latest scientific insights.

The usability of the produced knowledge is judged by its application in practice. The usefulness of a statement is evaluated in whether or not it proposes a change in patient-physician interactions and whether or not this change can be justified (ibid.: 1980). Discussions among group members would be characterised by the exchange of different types of knowledge. Knowledge about how to treat a condition in a particular case and how a condition is managed and experienced by patients.

One can argue that it is this repertoire of evaluation that patients can actively participate in with their experiential knowledge. For instance, scientific evidence shows that an intervention is effective but the practical usability is lacking for certain patients. Due to experiential knowledge it is known that in practice the intervention is not feasible for elderly. This is the specific added value of the experiences from patients. Yet, the fit with daily medical practice that the statement has to produce in order to be of practical usability is complex. Certainly when one takes into account the uniqueness of individual patients and the context in which the guideline is going to be used (Moreira 2005: 1980).

The useful practical knowledge should lead to ideal social action that is characterised by patient-physician interactions whereby the guideline should be one input of many in order to make clinical decisions (ibid.: 1980).

The political acceptability of statements is judged by its potential effect on power distributions in existing institutions (ibid.: 1981). The current distribution of inter-relational power and accountability is discussed and evaluated by whether or not the action of a certain statement imposes a change in the current situation. When a statement imposes a change in the current situation it should be evaluated whether or not this is political justified. The workgroup-members should take into account that the guideline does not impose a particular political standpoint favouring a collaboration with existing political actors or that it challenges existing political forces (ibid.: 1982). The workgroup as a whole has to come to an agreement about the political implications that the guideline in question can account for (ibid.: 1982). It is important to view these implications with the particular representation of different positions in the health care arena in the development workgroup. This is associated with the political history of the condition in question. In the end the guideline forms a political document whereby the underlying (ideal) actions and reactions that the statements are supposed to entail are taken into account (ibid.: 1982).

The methodological adequacy of the produced knowledge in the guideline is judged by the procedures, activities and assignments that the workgroup is supposed to accomplish (ibid.: 1978). The procedures that lead to the production of knowledge is judged by its adequacy. Thereby the final document, the outcome, is evaluated by the methodological activities, the process (ibid.: 1982).

This repertoire is constantly under discussion. Methodologists are focussed on setting up robust evidence-based statements and they are struggling with absent suitable methods to assess and incorporate the patient perspective in guidelines. It is difficult to methodologically justify and incorporate experiential knowledge.

When conditions are made visible under which statements are elaborated a link is established between the content of the guideline and the context in which those statements are formulated (ibid.: 1982-1983). The outside world can thus evaluate if the statements are disentangled from external influences, a criteria for the value of knowledge (ibid.: 1983). The disentanglement of the group from the outside world starts by announcing the links each actor has with external institutions (ibid.: 1983). This is important for the development of the workgroup as a collective agent, an institution in itself (ibid.: 1982). Consequently, the workgroup can evaluate and judge its own practices by their adequacy. Thereby an adequate form of representation seems crucial in patterns of interactions between workgroup-members.

Any imbalance of participation or suspected external influence can be addressed by workgroup-members who have had fewer participation possibilities within other repertoires of evaluation. Patients, overall less participating in the repertoire of science, can assess how the 'science' element of the guideline has been constructed and whether or not this has been done in acceptable ways and due processes (ibid.: 1983-1984). This in order to reassure that the knowledge that the guideline produces is not influenced by the connections with institutions that workgroup-members might have (ibid.: 1983). Patient representatives can use the initial starting research questions that are drawn to assess the adequacy of the produced knowledge within the guideline. These research questions should ideally lead the guideline development process in order to take the formulated bottlenecks into account. Answering all starting research questions is the assignment of the workgroup that they are supposed to accomplish.

In the section above is shown that different judgements about knowledge are taken into account when guidelines are developed. Previously it is argued that different kinds of knowledge that are brought in between the different actors of the workgroup could lead to a clash. Currently, the EBM methodology, with RCTs as the main input, is seen as the golden standard for determining effectiveness. Researchers, policy-makers, methodologists and physicians often have difficulty with patients who are interacting with a different level of knowledge. This brings more uncertainty to medical and scientific discussions since patient experiences are not well grounded on evidence-based research. To eventually produce recommendations that are evidence-based, scientifically valid, acceptable to users and feasible

to implement the development workgroup collectively judge knowledge through the combination of the four repertoires of evaluation.

2.8 Sub questions

This theoretical chapter has provided an insight into the academic discussions about the essential concepts used in this thesis. The following sub questions will assist with answering the main research question:

1. In which ways are the lichen sclerosus patients involved in the lichen sclerosus guideline development process?
2. What work has the lichen sclerosus patient representative done in order to be heard in the guideline (workgroup) proceedings?
3. In which way is the produced knowledge within the lichen sclerosus guideline justified in terms of the technical robustness, practical usability, political acceptability and methodological adequacy?

3. Chapter 3 The methods to collect empirical data

3.1 General overview

This first section describes the research design followed by a description of the specific case under study. Hereafter the methods that are used to collect empirical data are outlined. Each data collection method is explained with corresponding method of analysis. Section two clarifies the observations and document analysis. The third section is concerned with interviews. Subsequently reliability, validity and ethical considerations are outlined in the fourth, fifth and sixth section.

❖ Design

The focus of this research is patient participation within the development of the evidence-based LS guideline. This is further described in the next paragraph. Various methods to collect empirical data have been used. This can be referred to as data triangulation (Mortelmans 2011: 437). The exploration from different kinds of resources and angles enables an attempt to fully map the complexity of the case. Moreover, data triangulation can attribute to a full understanding of possible inconsistent or even conflicting findings (ibid.: 438) and thus provides a helicopter view.

❖ Case study

This research can be referred to as a case study (ibid.: 144) since the development of the LS guideline is a demarcated object of research. With the use of different data collection methods a full insight of the (inter)actions and knowledge transfer between various key stakeholders and their consequences has been explored. Additional activities that the SLS undertook (besides the development of the LS guideline) were attended to and observed from November 2011 until May 2013. This gave me the opportunity to get a full insight of the role and tasks of the LS patient organisation. Moreover, an examination could be made of how and to what extent this influenced the participation possibilities within the guideline development process. A total overview of data collection and additional activities during this research are provided in appendix 1.

3.2 Observations and document analysis

Patient participation of the SLS is among others explored via observations of two separate projects. One being the multidisciplinary workgroup (workgroup) consisting of 23 members concerned with the development of the evidence-based LS guideline. The other being the steering committee (steering committee) which was concerned with setting up 'the patient perspective'. Observations from both projects began from November 2011. At that moment most meetings of the workgroup had already taken place. Though the majority of the meetings of the steering committee could be attended to and observed. The meetings from both projects can be seen as formal and open forms of communications. This implicates that formal communication styles and meeting structures have been followed to fit customary rules. This leads to public communication in which it is difficult for outsiders, and me as a researcher, to ascertain underlying values, trust relations and beliefs. By attending the meetings I always joined up with the chairman of the SLS. I thereby followed the relevant actor focused on in this research. I always sat next to the patient representative. This allowed me to be an observatory participant (ibid.: 289).

By observing and experiencing first-hand, the (inter)actions in their natural setting were captured. The notes that were collected on the spot can be referred to as "jottings" (Bernard 1994: 181, 182) or "scratch notes" (Mortelmans 2011: 312). These short field notes have been reported in a "field diary". The notes from the field diary were written out into descriptive notes. Descriptive notes are the ones taken from two methods: listening and watching (Bernard 1994: 188). The details of the environment and the behaviour and (inter)actions from workgroup-members were given priority. These rich descriptions were electronically typed-up into thick descriptions. The thick descriptions from

observations refer both to describing and interpreting observational data within a context. They were a tool to clarify the context of the experiences of the involved participants both focussed on sufficient details as well as situation-specific meanings (Schwartz-Shea and Yanow 2009: 59). The thick descriptions were provided with corresponding codes whereby the theoretical concepts discussed in chapter 2 guided the process.

For analysing the data from the observations, additional sources of information were collected. Documents, minutes and reports from the start of both projects were collected and analysed. With a retrospective study of all previously held meetings an overall view of the whole process was obtained.

As private and confidential interactions are another way of knowledge sharing (Waring and Bishop 2010: 326) I asked permission from the patient representative to obtain the email exchanges between the SLS on the one hand and policy-makers (NVDV), physicians and SNH on the other. This way the more informal (and private) communication streams could be analysed to discover underlying values, trust and mutual understandings that as a researcher is not self-evident based on the formal communication streams while observing. Email exchanges from January 2009 until May 2013 were examined.

The thick descriptions, documents and email exchanges were analysed in which short descriptive codes categorised the data in shared themes. Interpretative analysis involved an ongoing cycle in which new acquired information was explored, confronted and related to existing knowledge and existing analyses. This led to a meaning, understanding and interpretation of the research findings (Corbin and Strauss 2008: 51, 52).

3.3 Interviews

Interviews were conducted to further explore the participatory role of the SLS. Six formal and three informal interviews with actors from both the workgroup and the steering committee were conducted (in the last trimester of 2012). Semi-structured interviews allowed for a higher standardisation among the discussed topics (Mortelmans 2011: 209, 217). A general topic list with key concepts functioned as a directory guide. Besides, there was still room and flexibility to ask other, different and follow-up questions. Furthermore, the interviewees could add own interpretations and thoughts. The topic list of the interviews is presented in appendix 2.

The interviews were tape-recorded and transcribed for further analysis. The transcriptions were thematically analysed with codes whereby the theoretical concepts discussed in chapter 2 guided the process. Then an ongoing cycle of interpretation and understanding began. Through data analysis it became possible to perceive patterns and eventually to gain arguable and understandable insights into the research questions addressed in this thesis.

3.4 Reliability

Reliability is an essential condition for valid results (ibid.: 433). Data have to be consistent and results verifiable and comprehensible. If one assumes reality as given than results can be replicated in a second research. As qualitative research assumes that reality is socially constructed the reproduction of results is not a target in itself (ibid.: 433). Coincidences as the context, the participants, the research methods and the researcher are factors that could influence the reliability. Since one cannot separate herself as person from the research and the analysis (Corbin and Strauss 2008: 4, 10) self reflection is a necessary method to create openness about the role as researcher (Mortelmans 2011: 435). In this research I employed multiple roles being an “emotionally involved” patient, a “professionally concerned” nurse and a “seriously hard working” HEPL student wanting to finish her degree. A transition between roles is strived after to widen up the angle and perspective of the data. Through self reflection it became possible to understand how I could have influenced the research process and how it could have influenced me as well (Corbin and Strauss 2008: 11).

3.5 Validity

Qualitative research presumes that reality is socially constructed (Mortelmans 2011: 440). The reality of the research findings was established through my interpretations of the research data. These interpretations build upon my prior knowledge and experiences (Corbin and Strauss 2008: 4, 10). And since multiple stories can be told from one set of data, multiple perspectives on the data were considered to consider and understand the point of view from the participants' perspective (Green and Thorogood 2004: 20, Corbin and Strauss 2008: 47, 48). The important issue is whether the interpretation from the data corresponds with the data collected (Green and Thorogood 2004: 243). This interpretation has to be credible and is in this research aimed for through different methods. First through triangulation the credibility of the results is assumed to increase (Mortelmans 2011: 437). The multiple perspectives that one perceives with multiple data collection methods contributes to the credibility of the data (ibid.: 436-438). The second method that enhances the credibility of the interpretations is the extensiveness (ibid.: 437) in which this research took place. Over a period of 1,5 years I attended more than twenty meetings. This gave me the opportunity to avail myself to the research setting and to ensure a credible reflection of the implications of the respondents.

3.6 Ethical considerations

All data from every data collection method were cleared from any personal information to protect confidentiality whereby I as researcher was the only one with access to those data. No stakeholder is referred to by his/her own personal name when a quote is formulated in the next chapters.

4. Chapter 4 The development of the LS guideline

4.1 General overview

The theoretical chapter has provided an insight into the academic discussions about the essential concepts used in this thesis being participation, representation and experiential knowledge. The results of this research are classified according to these three concepts. First, the next section provides a chronological description of the guideline development process. Here a distinction is made between the development of the evidence-based LS guideline on the one hand and the development of ‘the patient perspective’ on the other, since these two projects progressed separately. The third section is dedicated to the results regarding the concept of participation. All the different methods of patient participation within the guideline development process are described. The ways in which the patient representative has practiced his role and tasks as representative is clarified in the fourth section. The fifth section describes the research results concerning the concept of experiential knowledge.

4.2 The chronological LS guideline development process

❖ *The evidence-based LS guideline*

The lichen sclerosus patient organisation (SLS) has been established in 2006. From then on the SLS has applied for annual, structural subsidy from PGO that subsidizes patient initiatives. The SLS used its subsidy every year for activities as facilitating information, representation of patients and peer support. A substantial part of the subsidy from 2009 (25.000 Euros) was used to initiate the LS guideline and to facilitate this project with financial support. The Dutch Society of Dermatology and Venereology (Nederlandse Vereniging voor Dermatologie en Venereologie, NVDV), an association of the currently 580 dermatologists (April 2013) is executor of guidelines involving dermatologic conditions.

The first appointment between the SLS and the president of the NVDV was in December 2009. The start of the development of the LS guideline was initiated in this meeting. In the meetings that followed in January and February 2010 the application for the subsidy towards SKMS⁷ (Orde van Medisch Specialisten 2013a) was compiled; the draft of the starting research questions were formulated and the members and the chairman of the workgroup were selected by the NVDV and SLS. The workgroup consisted of dermatologists, gynaecologists, a pathologist, an oral and maxillofacial surgeon, a urologist, a sexologist, a paediatrician, a general practitioner (GP), a pelvic physiotherapist, a dental hygienist, an allergist, a gastrointestinal and liver physician, a nurse, policy-makers of the NVDV and the chairman of the SLS. This multidisciplinary group is representative of the professionals LS patients deal with in their care.

The NVDV is situated at the Domus Medica in Utrecht and this is the location where the development process has mainly taken place. In May 2010 a literature search and meta-analysis was conducted by the NVDV. The objective of the first official workgroup meeting, which was held in June 2010, was to agree upon the starting research questions. The months hereafter the NVDV compiled a draft guideline document based on the meta-analysis. The method used to compose the LS guideline is the “pressure cook method”⁸ in which in a two-day intensive weekend (13th and 14th of January 2011) the literature has been reviewed and assessed by the guideline workgroup on its strength of evidence, relevance and usefulness. After this session, a first draft of the LS guideline was provided by the policy-makers of the NVDV. Feedback on the draft was given by physicians, both external as well as internal to the workgroup. These reviews and adjustments were submitted to the workgroup-members by email twice at confirmation.

The objective of the next workgroup meeting (December 2011) was to agree upon the final formulation of the conclusions and the recommendations in the guideline. At the end of this meeting the final document was established. Hereafter all the involved professional associations were given the opportunity to give feedback on the guideline during three months.

⁷ Stichting Kwaliteitsgelden Medisch Specialisten is the foundation who subsidizes quality-projects of medical-specialist care.

⁸ Snelkookpanmethode.

The LS guideline was authorized in the annual NVDV society meeting at the 30th of March 2012.

At the 28th of February 2013 the NVDV provided a draft of the patient version of the LS guideline. SLS was asked to give feedback on this draft. The patient version of the LS guideline was final on the 4th of May 2013.

❖ *'The patient perspective'*

Initially the patient perspective was not incorporated in the LS guideline development process due to costs constraints. Therefore, in June 2010, the SLS and the NVDV applied for a project subsidy⁹. This subsidy of 27.000 Euros has been approved by PGO in December 2010. At January 2011 the NVDV has established a steering committee in which the SLS and the National Skin fund Foundation¹⁰ (Stichting Nationaal Huidfonds, SNH, Huidfonds 2013) cooperated together. The project design that was granted consisted of four elements being: a desk research; a focus group; a determination of a baseline measurement of patient reported outcome measures¹¹ (PROMs, Prinsen and De Korte 2011: 4) via a questionnaire and a second questionnaire one year after the implementation of the LS guideline. The desk research took place in April 2011 with a view to explore the evidence on PROMs of LS patients. The chosen generic PROMs were severity of the condition, quality of life (QoL) and treatment satisfaction. Since little evidence was known about the exact interpretation of PROMs of LS patients, SNH proposed to examine this themselves. The proposed study design¹² of SNH has been approved in August 2011 by the steering committee.

At the 20th of September 2011 SNH organised a focus group. The results from the focus group together with the results from the desk research have been used as input for the design of the study-specific questionnaire. This questionnaire has been conducted among all members of the SLS before the implementation of the LS guideline in March 2012. The results were published in a report in June 2012.

All the research results were translated into the final 'patient perspective' at the 10th of August 2012 that was given the status of an annex and displayed on the NVDV guideline website. A second but shortened questionnaire was executed in March 2013, a year after the authorisation of the LS guideline. In this questionnaire additional questions were added about the (implementation of the) LS guideline. The results from both questionnaires were analysed in order to ascertain a change in treatment satisfaction and whether or not this could be in consequence of the implementation of the LS guideline. These results are to be expected in June 2013.

A chronological overview of both the LS guideline development process as well as 'the patient perspective' is given in appendix 3.

4.3 Participation

This section clarifies the methods of patient participation within the LS guideline development process. The results are described in the sequential phases of the guideline developing process, though some phases are omitted in this section but described in the next sections since they better fit the other concepts that are used. The developing process of 'the patient perspective' is in total described in this section. Albeit the patient perspective affects the other two concepts too this procedure is chosen to prevent fragmentation of the story.

❖ *The initiation of the LS guideline*

The NVDV initiates the development of Dutch dermatological guidelines. With a restricted budget the NVDV is forced to prioritise alongside predetermined criteria, as orally clarified by the president of the NVDV (personal communication). These criteria being the prevalence, burden of disease,

⁹ Project "Versterking inbreng van de patiënt bij verbetering van de kwaliteit van zorg voor mensen met chronische huidaandoeningen lichen planus en lichen sclerosus".

¹⁰ National Skin fund Foundation is an independent research institute with the mission to increase quality of life and the quality of care for patients with chronic skin conditions.

¹¹ PROMs are comprised of the preferences and experiences of LS patients regarding relevant aspects of health related quality of life, treatment satisfaction and treatment preferences.

¹² "Onderzoek Behandelttevredenheid en Kwaliteit van Zorg bij Lichen Sclerosus".

relevance for society and the expectation that the guideline can enhance the quality of care are determined by the Regieraad. The criteria are further amplified and validated by the Order of Medical Specialists¹³ (Orde van Medisch Specialisten, Orde van Medisch Specialisten 2013b) and the Dutch Association of GPs (NHG). This led to a supplementary criteria being the need for a guideline both from the medical field as well as from patients. According to the president of the NVDV a LS guideline “is not unimportant but it does not stand in the top 10” [of most important guidelines, FV¹⁴] based on the aforementioned criteria from the Regieraad. The SLS indicated to the NVDV that as a patient organisation they would like to see a LS guideline being established. And like the president pointed out during the interview the SLS acknowledged the importance with “a very strong gesture”, being 25.000 Euros. As a dermatologic association this gesture from a patient organisation “was not easy to ignore”. The president of the NVDV does not exclude the possibility that there would not be a LS guideline today without this financial contribution and the fact that the SLS has been the initiator; “that money certainly made an important move” (president NVDV, personal communication). Three other interviewed workgroup-members have even put it more strongly indicating that when the SLS did not contribute financially there still would not be a LS guideline today.

❖ *Analysis of bottlenecks and formulation of starting research questions*

Since the cause of LS is yet unknown and compared with other conditions the prevalence is not very high, LS is not sufficiently being recognized by both patients and physicians. This often results in a misinterpretation and wrong diagnosis of the first symptom which is itching and therefore frequently associated with a Candida infection. This ‘diagnostic delay’ is still common practice. Furthermore, there is also a ‘patient delay’. As this skin condition is most often situated in the anogenital area, embarrassment prevents patients to see a physician or consult family and friends. Both diagnostic and patient delay needs to be decreased since there is a continuing rise in the incidence of vulvar cancer caused by aging. More awareness, proper diagnosis and interpretation of symptoms is assumed to be realised through the establishment of a LS guideline. An increase in quality of care is expected that expresses in less practice variation in treatment among patients.

The collective objectives of the establishment of a LS guideline can be summarized in decreasing the doctor and patient delay. The individual bottlenecks are very diverse. Most often heard bottlenecks are insufficient time per consultation; insufficient uniformity in diagnostics and treatments; insufficient follow-up; lack of awareness and inadequate guidance from secondary specialists. These bottlenecks are assessed and discussed between the SLS, the NVDV and the chairman of the workgroup. In the final guideline the bottlenecks from the perspective of LS patients are summarized and described as follow;

1. More awareness for the condition among general practitioners and specialists;
2. More time and attention during consultations of dermatologists, gynaecologists and other specialists;
3. Better guidance from for instance a registered psychologist/ -sexologist/ -pelvic physiotherapist;
4. More uniformity in diagnostics and treatment both between clinics as well as disciplines/specialism;
5. Adequate follow-up in connection with functional complaints and malignant degeneration.

The objective of the guideline development process is to agree upon these reported bottlenecks in practice; that the recommendations that arise from these bottlenecks are introduced in practice with the ultimate outcome an increase in the quality of LS care. Decreasing doctor and patient delay is assumed to be realised through more awareness (1), more uniformity in diagnostics and treatment (4) and adequate follow-up (3 and 5).

The experiences from LS patients and physicians from secondary care is that the doctor delay is often a consequence of the wrong interpretation of symptoms by GPs. Therefore patients are not diagnosed timely, treated with the wrong medications and not referred to secondary care in time. This

¹³ The Order of Medical Specialists is the professional association for and by medical specialists.

¹⁴ FV refers to Femke Voorn; the author of this thesis.

is considered and addressed in the guideline via referring to the cooperation agreement with the NHG. A further explanation of this cooperation agreement is described in the last paragraph of this section. Furthermore, since LS is a chronic condition that causes a significant reduction in the QoL it is preferable that LS is treated multidisciplinary (3). This is addressed within the formulation of a starting research question and the selection of the workgroup-members.

Due to the assessment of the bottlenecks in practice a first draft of the starting research questions was formulated by the chairman of the guideline workgroup and the patient representative in collaboration with the researchers and policy-makers of the NVDV. In the first meeting of the workgroup the starting research questions were finalized. A list of the final starting research questions (in Dutch) is presented in appendix 4. Input from the patient representative on the starting research questions were regarding epidemiology, prognoses, clinical view, non-medical treatments, medical treatments, quality of life, follow-up, consultation, referrals and education. The patient representative has put a lot of time and effort in the fact that these starting research questions were addressed and acted upon in the guideline via engaging in various (in)formal meetings and one-on-one dinners as will be clarified in section 4.4. The different methods that the patient representative undertook to address these research questions in the developing process are described in the next paragraphs and sections.

❖ *Literature search*

The search for literature was led by the starting research questions and conducted by the NVDV through a meta-analysis of scientific articles in PubMed and the Cochrane Library. The SLS contributed to this literature search by supplying one gigabyte of scientific articles that were already available on the website of the patient organisation. This made a great difference for the NVDV saving them a lot of time searching for literature and thereby shortening the time planned for this phase. The NVDV has asked the SLS to validate the reference list of the guideline.

In April 2010 the SLS delivers a list with the assessment of current treatment schemes used by 530 forum members (patients). This is directly related to the starting research questions concerning medical treatments being ‘which local treatment is preferred (+ order)?’ and ‘which local treatment is preferred by itch/pain?’ The judgment about defining these different treatment schemes in the LS guideline is described further in section 4.4.

❖ *Assessing the strength of scientific evidence*

Scientific literature was judged on its strength of evidence during the two-day pressure cook method. Initially the patient representative was divided in the subgroup of paramedical disciplines; sexual counsellor, pelvic physiotherapist and another board member of the SLS. This subgroup would judge the non evidence-based parts of the guideline (information, follow-up). It was unacceptable for the patient representative that he was classified in the subgroup that judged the non evidence-based part of the guideline only. The representative immediately demanded a change of subgroup and used the financial contribution from SLS as argument that he could have a say in the evidence-based parts of the guideline too. The representative judged the exclusion from assessing the strength of scientific evidence as imbalance in participation. Besides, the patient representative had come across a few new scientific articles that would shed another light on for instance heredity. These articles were evolved out of research that the SLS had financially supported. Therefore, the patient representative insisted on participating in the subgroups that assessed the evidence-based parts of the guideline. Without resistance from the president of the NVDV the representative was moved to a subgroup in which one dermatologist, one gynaecologist and one methodologist were classified.

Participating within a subgroup that assessed the evidence-based parts of the guideline, the patient representative introduced the scientific articles that he brought along. Due to these new insights the initial passage on heredity has been changed. The passage has been elaborated indicating that families with LS have a higher risk on the existence of squamous cell carcinoma in comparison with families without LS.

In assessing the articles on the prevalence of LS, the representative deliberately choose scientific articles that indicated a higher prevalence of LS. Through participation it became possible “to

negotiate directly with the physicians” (patient representative, personal communication). Therefore, the initial prevalence rate of 1:300 – 1:1000 has been changed into 1:60 till 1:300 – 1:1000. Hereby, the representative could give input on the starting research questions regarding epidemiology (‘what is the prevalence of LS?’) and prognoses (‘how often does LS turns into squamous cell carcinoma?’).

❖ *‘The patient perspective’*

The objective of the additional project with SNH was to integrate the LS patient perspective in the LS guideline. The intended result of ‘the patient perspective’ is enhancement of patient-centred LS care whereby a better informed choice in treatment can be made for an individual LS patient. Multiple research methods were designed and executed to determine the LS patient perspective. The first phase of this project comprised of a desk research on PROMs and a focus group among LS patients. The second phase consisted of the execution of two questionnaires.

The domains of the PROM treatment satisfaction that were selected after the desk research were: effectiveness, safety, user friendliness, information provision, physician-patient relation and organisation of the treatment. These generic domains with corresponding aspects (checklist) were used as an input for the focus group. Initially, the recruitment for participants took place by the SLS. Hereafter the screening of the pre-filled questionnaires and the focus groups invitations were sent by SNH.

The objective of the focus group was to determine aspects belonging to the domains of the PROM treatment satisfaction, that are relevant specifically for LS patients. Nine LS patients (eight women, one man) were invited to join the focus group. Despite reservations from SNH the patient representative was also present during the focus group. He wanted to observe the focus group himself so that he knew what information was shared with SNH and what information would be left over when SNH would translate these results into research reports. The reservations of SNH were regarding the fact that the representative (as being the chairman of the patient organisation) could hinder the process of consensus. According to the epidemiologists from SNH focus groups are preferably performed without board members to prevent any influence from the board on the process. “As chairman you have a certain status that possibly can form an obstacle for patients to speak out freely about their condition” (epidemiologists SNH, personal communication). The patient representative observed the process sitting aside from the table where the participants sat. Permission on the presence of the chairman of the patient organisation was asked and obtained from every participant.

Most patients knew each other, at least by name or by sight. Each patient was given the opportunity to share experiences with present and previous treatments. As being one of the participants I can tell from my experience that the atmosphere was open. Every participant was able to tell his or her full story. Participants were interested in each other: questions were asked and answered without paying attention to the time (the initial agenda for that meeting ran out).

All aspects that determined the extent of treatment satisfaction were written down and arranged in the six domains by a member of SNH. Then the checklist was presented to the participants. Each domain with corresponding aspects was discussed and assessed. The initial aspects were judged and accepted as suitable. Furthermore, the domains were supplemented by the aspects given by each patient individually at the beginning of the meeting.

Although the patient representative would only observe this session and not interfere, at a certain moment he did interfere. He had noticed that the experiences and preferences from male patients were underexposed and added information regarding this subject on behalf of the male LS patient. Albeit there was an agreement that the patient representative would not interrupt the group discussions, this knowledge was regarded as valuable. Since only one male LS patient was present during this session, indeed the experiences from male LS patients were underexposed.

The relative importance of the six domains was determined by giving each participant the opportunity to divide ten points among the domains, thereby giving the most points to the most important domain. The generalised scores showed that the domains effectiveness, physician-patient relation and safety were considered as the most important domains, though this did not necessarily reflect the preferences of individual patients. For example I gave the domain user friendliness eight

points indicating that this was the most important domain for me individually. But overall, this domain was valued as least important by the whole group.

❖ *(Results) questionnaire and cut-off values*

The desk research, together with the results from the focus group were used as input for the second phase of the project, the questionnaire. SNH developed the draft questionnaire whereby SLS provided feedback on the various versions via email. Smoking and heredity is common among LS patients as turned out from narratives (experiences) on the internet forum. SNH was not familiar with this fact and the questionnaire was extended with questions about whether or not patients smoke or whether or not LS occurs among family members.

The study-specific questionnaire among LS patients provided a baseline measurement of the three PROMs. An officially validated baseline value of QoL was obtained through adding the Skindex-29¹⁵ in the study-specific questionnaire (Nijland et al. 2012: 27). Norm scores were set to interpret the values of QoL and treatment satisfaction since the values of these PROMs are observations in itself without the attachment of cut-off values¹⁶ (Van Cranenburgh et al. 2011: 171). The percentage of 'satisfied' patients was cut-off at 67%. The percentage of 'dissatisfied' patients was cut-off at 5%. The measurement of QoL among LS patients contributed to the starting research question regarding quality of life ('what is the impact of lichen sclerosus on the quality of life?') since little scientific evidence is available on this subject.

The cut-off values have been determined by the steering committee in conjunction with experiences from the development of the Psoriasis guideline. In the workgroup from December 2011 the steering committee asked the workgroup for approval to use these cut-off values in order to create support. This has been obtained. Moreover, the patient representative wrote an article in the NVDV magazine of March 2012 in which he explained the importance of these cut-off values for the quality of LS care (Van Gestel 2012: 198). Furthermore, the representative (together with me) gave a lecture on the annual NVDV society meeting regarding the use and importance of the LS guideline and 'the patient perspective'. During this meeting the 300 dermatologists present were given the opportunity to vote on two theses concerning the cut-off values and the influence of the LS guideline on the quality of care. The results of these votes are presented in appendix 5 and display a wide consent about the existence of these cut-off values.

The research results concerning the LS patient perspective have been translated into various research reports. The results from the first phase were extensively and integrally defined in a report (Prinsen and De Korte 2011). The end research report, consisting of the two phases altogether, provides a full description of the results from the whole project (Nijland et al. 2012). It covers 86 pages. The domains of treatment satisfaction with corresponding aspects are in total translated in this report.

A last translation concerns the end product, 'the patient perspective' (Prinsen et al. 2012), which is official and publicly available on the website of the NVDV. Initially 'the patient perspective' covered merely 12 pages, all demographic and clinical data were left out. The patient representative insisted on inserting these data so one can see at a glance the demographic characteristics of an average LS patient. Still 'the patient perspective' consists of (only) 18 pages. That means that the total of research results are defined in a summarized way. For example, the six domains of treatment satisfactions are mentioned but not the aspects regarding each domain. The reader is referred to the extended end research report (of 86 pages), multiple times.

❖ *Authorisation phase*

The LS guideline was authorized during the annual NVDV society meeting in March 2012. 'The patient perspective' was not completed at that time. That led to the fact that 'the patient perspective' could not be authorized during that meeting. Therefore, 'the patient perspective' could not officially be

¹⁵ The Skindex-29 is a multi-dimensional, validated, dermatologic-specific quality of life questionnaire.

¹⁶ These cut-off values were already used within another target group being psoriasis.

incorporated in the main evidence-based LS guideline. Consequently, ‘the patient perspective’ received the status of annex but as annex it was never authorized.

There are different views among the workgroup-members on the fact that ‘the patient perspective’ received the status of annex. The president of the NVDV finds an appendix preferable when the preferences of patients as such do not appear *in* the guideline. If patients’ preferences correspond with the conclusions and recommendations of the guideline “*than* you can say it is part of the guideline” (president NVDV, personal communication). The document “with all their [LS patients, FV] wishes that really have a high desirable character and that are not one-on-one included in the guideline” is the reason why the deliberate choice is made to make the LS patient perspective an annex (president of the NVDV, personal communication). This is the choice that is made during the process.

‘The patient perspective’ is not authorized. According to the president of the NVDV it is more important whether or not the recommendations are used in daily medical practice. “But don’t expect too much of it, that is the truth!” is his answer on the question whether or not the physician in practice will read and use the guideline and the annex in practice.

The fact that ‘the patient perspective’ was not officially incorporated into the guideline is considered as an “unused chance” by the patient representative. On the one hand the representative wonders to what extent the current annex and its status has been worthwhile the subsidy of 27.000 Euros. On the other he considers the advantage of having insights into (the measurement of) the baseline PROMs as a huge gain and thinks that it enhances awareness for (the quality of life of) patients with LS. A dermatologists, from his perspective, believes that the measurement per se could be a tool to increase quality of care and life since “measuring is knowing”. Measuring makes it objective and makes it possible for individual physicians to question their practice when any progress fails to appear. Some physicians need to be confronted with objective measurements; “this way physicians are gently forced to gain insight” (dermatologist, personal communication). In order to gain this insight ‘the patient perspective’ must be read by physicians. “When the LS guideline and patient perspective is not read it simply becomes a piece of paper” (dermatologist, personal communication). But when physicians do read the guideline it enhances the quality of care, since “the patient perspective is the umbrella of quality of life” (dermatologist, personal communication).

❖ *The LS NHG-standard*

One of the starting research questions involved the deficiency in the referrals from GPs to secondary care. Therefore, a cooperation agreement between the NVDV and the NHG was established in September 2011. The NVDV has given insight to the NHG in all the gathered scientific literature, thus saving a large amount of research work for the NHG to establish a LS NHG-standard. Dermatological NHG-standards are very rare and the LS NHG-standard is one of five dermatology standards of the NHG.

The development of the LS NHG-standard was parallel to the LS guideline though they were two separate projects. This meant that the content of the two documents were not fully aligned except for the cooperation agreement. This is remarkable since a GP from the NHG and a gynaecologist, who were both workgroup-members, participated in the NHG-standard workgroup too. The president of the NVDV feels that it is typical for the NHG that this could happen since they have an own authorisation commission and they decide themselves what is described in the standard and what is left out.

In December 2011 the SLS was asked to give feedback on the draft version of the LS NHG-standard. Nearly a year later, in November 2012, the LS NHG-standard became into effect. In the intermediate period the SLS was not informed at all about the status of their feedback or the status of the LS standard. Only to see that the NHG had endorsed this document but the feedback of the SLS was not acted upon although SLS is mentioned as referent.

4.4 Representation of LS patients

This section describes the ways in which the LS patient is represented by the LS patient organisation and all the work that the LS patient representative has done in order to strengthen his role of representing the LS patient.

❖ *The LS patient representative*

The board of SLS consists of four members. These four board members each have their own tasks and duties. Within the division of tasks the chairman of the SLS does the external representation of LS patients. Other board members execute the tasks regarding treasury, secretary, internet forum, congress planning etcetera. During the whole LS guideline development process the LS patient was represented by the chairman of the SLS. He is an academic educated person, 40 years old, not burdened with LS himself and he practices a profession as auditor. Due to his profession the patient representative has had prior experiences in entering into processes in which different interests need to be mutually adjusted.

❖ *The creation of alliances*

The patient representative created mutual interests with different key stakeholders. After a close examination of email exchange and both formal (for instance conferences) and informal (for instance dinners) appointments the most important actors, as being the ones that have the most (in)formal contact with SLS, were identified.

The patient representative considers the foundation as a very small patient organisation with little formal infrastructural provisions like a medical commission. The representative is convinced that the SLS does not need a formal medical commission at the moment “since the SLS has very close connections with several physicians” (patient representative, personal communication). The “formula of success”, as the patient representative calls it, were the informal dinners. Becoming acquainted to one another led to SLS not being “one of many patient organisations” (patient representative, personal communication).

A gynaecologist acknowledges that he has a close relationship with SLS. From his perspective privileges arise due to close connections. Some patient organisations have officially established a medical commission. But the SLS is more “into informal contacts” (gynaecologist, personal communication). His close connection with the SLS contributes to the fact that the SLS does not have to establish such a formal provision. The gynaecologist points out that whenever the SLS needs assistance on a medical level they know where they can find him. He also performs a yearly “group-consult”, like he calls it, on the annual LS patient conference day. Entirely in his own time (he even blocks this day ahead for other appointments) and at his own expenses.

❖ *The underlying reason for the development of the LS guideline*

Different medical specialism can be concerned with the treatment of LS patients: gynaecologists, dermatologists, urologists. Dermatologists however, are confronted with a limited consultation time of five minutes whereas gynaecologists get a reimbursement for fifteen minutes from health care insurers. Five minutes of consultation time was considered to be insufficient to treat LS patients adequately, for both a dermatologist and the SLS.

Through personal narratives on the secured LS forum the SLS learned that a lot of LS patients complain about dermatologists not taking sufficient time for discussing other subjects besides the current state of LS and the (previous and future) treatments. The dermatologist on the other hand complained about not having sufficient time to address these subjects and he feels “to be in a hurry” to complete the consults and to reduce the waiting times of patients in the waiting room. Furthermore, he feels that patients should have the possibility to choose physicians with the best expertise but such physicians should be able to “charge a decent DBC!”¹⁷ (email exchange patient representative and dermatologist). Therefore, the SLS was asked by this dermatologist to express their concern to the health care insurer Centraal Beheer Achmea (Achmea) since this insurer is the biggest provider in Rotterdam area. This letter regarding the lack of consultation time was sent to Achmea in March 2009. Soon after this appeal a negative reply arrived stating that the chronic condition was not formally known to them since no medical guideline was available. “Actually a condition only matters to an insurer when a medical guideline is in place” (patient representative, personal communication). Both

¹⁷ Diagnose Behandel Combinatie, Diagnosis Related Groups.

SLS and the dermatologist realized the fact that the unavailability of a medical guideline was considered a criterion for Achmea to not enter into this discussion. In another email exchange the patient representative and the dermatologist agree about “drafting a cunning plan” during diner. The email exchange goes on indicating that a LS guideline is understood as *the* tool to persuade Achmea to let them reimburse properly. If that does not work than (peer) supporters, vulva clinics and the media should be mobilised in order to voice this issue.

To address the ineffectiveness of the five minutes consultation time for dermatologists a starting research question is dedicated to this issue being ‘how much time should a physician have for a first consultation with a LS patient and how much time for a check-up?’ Furthermore, this issue is mentioned as a bottleneck from the perspective of patients and this is in the final version of the LS guideline translated as “more *time* and attention during consultations of *dermatologists*, gynaecologists and other specialists”.

The changes in the LS guideline indicating a higher prevalence rate and indicating an increased risk on squamous cell carcinoma in families with LS were two issues that the patient representative “fought hard for” as he explains it. A higher prevalence rate and an increased risk on squamous cell carcinoma are two but very important indicators for the burden of disease. These indicators stating that LS causes a high burden of disease could thus be considered as tools when entering into the discussion with Achmea.

❖ *Selection of the members and the chairman of the workgroup*

In consultation with the NVDV the LS patient representative identified the primary medical specialism involved with the treatment of LS. Moreover, the representative decided which physician from each professional association was invited to join the guideline workgroup. “*Because* the SLS paid, the SLS had the following advantages: beforehand we could determine the composition of the workgroup; beforehand we could form the guideline as multidisciplinary as possible” (LS patient representative, personal communication). The workgroup-members included all the actors that the patient representative had a close relationship with. A few physicians that were pointed out to join the workgroup had given a lecture on previous annual LS patient conference days. In the years 2009-2010 there were also a few diners between the patient representative and some of the physicians of the selected workgroup-members. The fact that the patient representative composed most of the (members of the) workgroup was thought of as normal to the president of the NVDV: “they [the patient organisation, FV] should delegate their strongest people”. The chairman of the workgroup was chosen by the patient representative. This chairman was the same dermatologist that previously asked SLS to negotiate with and express their concern to Achmea about the difficulties the five minutes consultation time causes as clarified in the previous paragraph.

❖ *The absence of key stakeholders in the guideline workgroup*

The dermatologists of the University Medical Centre St. Radboud (St. Radboud) were not invited to join the guideline workgroup although the SLS has a close relationship with this department and the physicians working there. Some workgroup-members had even noticed their absence. The absence of the physicians from St. Radboud was a well-thought and deliberate choice since the SLS has commissioned the dermatologist from St. Radboud to thoroughly review the draft of the LS guideline. The dermatologist would be better able to objectively review the LS guideline as she was not involved in the development process. Moreover, due to time limitations the SLS has asked the dermatologist to review the draft version of the guideline on behalf of them. In return the dermatologist was rewarded 3.000 Euros. It turned out that the 3.000 Euros were meant for another project being the sponsoring of a symposium but the SLS was not allowed to support this project financially based on the demands and conditions from PGO. To reach consensus SLS asked the dermatologist to review the guideline which could be rewarded financially based on the conditions from PGO. That way the patient representative would not have to spend time on reviewing the guideline himself every time the NVDV asks to do this. The dermatologist on the other hand was financially supported with her project in which she had her own interests.

❖ *Financial support to additional projects*

Besides the financial support to the symposium described in the previous paragraph, the SLS financially supported other projects too. A dermatologist asked the SLS to support her financially with the execution of a scientific research regarding differential diagnoses. The research about differential diagnoses could contribute to the LS guideline since LS is not recognized efficiently or confused with other conditions. The consequent diagnostic delay is harmful for LS patients since LS can cause squamous cell carcinoma. SLS gave the dermatologist a financial contribution of 5.000 Euros to do research about the differential diagnoses. The only precondition that the SLS laid down was that this research would be completed in five months in order to be included in the LS guideline. Since the dermatologist would do research regarding the LS guideline the SLS could financially contribute to this project without problems concerning the conditions of PGO. The dermatologists completed the research (Chi et al. 2011) within five months and the results are one-on-one included in the LS guideline (NVDV 2012: 16). Due to this research (with implicit the financial contribution of SLS) the key starting research question of the guideline regarding clinical view ('what are the differential diagnoses of LS?') could be answered.

❖ *The role of the patient representative*

The patient representative was considered as an adequate representative of LS patients. He was always present, prepared and actively involved in the plenary (medical) discussions. Medical jargon did not hindered involvement in fundamental discussions. Medical jargon was used by the representative and used in the right context. He was able to participate in the judgement of research results and the formulation of the recommendations. On the one hand he was adequately informed about the latest scientific developments and on the other he was "willing to listen" according to the president of the NVDV. This is important in order to not be considered as a disturbing factor. The participation of the representative is considered as very positive and stimulating, "a motor" according to the president of the NVDV.

Through the creation of close connections it occurred that a few times decisions were made via email exchange without the necessity to organise a formal meeting. That way it became easier for the NVDV to address the whole process in a continues pace. Among the interviewees the creation and facilitation of conditions including the financial support is mentioned as the stimulating factor of the participation of SLS. A gynaecologist considered the SLS as binding factor among all parties since it is "immediately visible for whom you are doing this, since they sit at the table". Since the patient representative was actually and directly present during the whole process he could explain the development process in depth to LS patients. The LS guideline development process has been a subject of discussion twice during the annual LS patient conferences in 2011 and 2012. That way the members of SLS were totally up-to-date regarding the evolvement of the LS guideline.

4.5 Experiential knowledge

This section clarifies in what way the produced knowledge within the lichen sclerosis guideline is justified.

❖ *The recommendations*

The draft version of the LS guideline was discussed by the workgroup. Hereby the focus was to come to consensus about the exact formulation of the recommendations stated in the LS guideline. A few remarkable examples are explored in depth next.

At one point in the discussion there was disagreement about the exact formulation of the recommendations in the guideline. In the draft version of the guideline the recommendations are considered authoritarian and didactic. One physician, a gynaecologist, says: "authoritarian recommendations should stay away from the guidelines". He continues that words as 'must' and 'ought to' should be averted in order to avoid authoritarian recommendations. The whole workgroup

agrees on the fact that not so much the content of the recommendations should be changed but the way the recommendations are formulated.

First recommendation explored in depth concerns a biopsy. The workgroup discusses whether or not a biopsy is necessary in order to diagnose LS. The focus is placed whether inexperienced physicians and GPs are able to diagnose LS based on clinical inspection and with one's bare eyes. Every workgroup-member agree that they themselves are capable to see with bare eyes if a patient has LS but they are not convinced that every other physician, especially GPs, are competent to do the same. This argument that not every physician is considered competent is supplemented with experiences out of daily medical practice. A physician discloses an example in which a patient was referred to him with metastasis of squamous cell carcinoma of the vulva. He argues that it could have been prevented if the patient was diagnosed earlier.

The exact formulation of the draft and the final recommendations are stated below, among each other and translated in English. Hereby own accentuations are applied in order to observe the changes, the attachments and the detachments. For the draft recommendations words are underlined whereby the applied changes in the final recommendations are provided in bold. The original text of the following recommendations (in Dutch) are provided in appendix 6.

Draft recommendation concerning biopsy

With a classic presentation of anogenital lichen sclerosis the diagnosis can be determined on the sole basis of anamneses and physical examination.

A biopt is recommended by:

1. Diagnostic uncertainty
2. Suspicion of neoplasm
3. Insufficient result of current treatment

The biopt has to be taken by someone with clinical experience. In case of uncertainty regarding the location of the biopt and/or unfamiliarity the patient ought to be referred for this biopt.

The use of a potent corticosteroid by the patient can influence the histological circumstances. The use ought to be stated on the pathology form.

The clinical view must be leading in diagnoses and treatment if the histo-pathological result is a-specific.

Final recommendation concerning biopsy

With a classic presentation of anogenital lichen sclerosis anamneses and physical examination **is sufficient** to determine the diagnosis and taking a biopsy is not necessary.

A biopt is recommended by:

1. Diagnostic uncertainty
2. Suspicion of neoplasm

In case of uncertainty regarding the **preferred** location of the biopt and/or unfamiliarity with taking a biopsy the patient **should** be referred for this biopt to **someone trained in this field**.

The use of corticosteroids can influence the histological circumstances and **therefore should** be stated on the pathology form.

If the result of the histological report is inconclusive, than –after eventual consultation with the pathologist – the clinical view is leading. By suspicion of neoplasm the histological research should be repeated.

The content of the ‘authoritarian’ recommendation has not been changed although the formulation has. Words as ‘must’ and ‘ought to’ are mainly avoided in the final recommendations and when words as ‘should’ are used, arguments are given to explain why this should be done. The final recommendation provides for more individual freedom in clinical decision-making.

The second recommendation concerns educating patients. The draft recommendation is eight sentences. The workgroup agrees that eight sentences of recommendation is too long. They argue that if all recommendations are that long than the guideline would never be read and used. They opt for a short and clear sentence. The initial recommendation explains *why* patient education is important and *what* the physician needs to explain to patients. The workgroup agrees that every physician knows this. It is considered unnecessary, didactic and authoritarian.

Draft recommendation concerning patient education

Educating patients is important, also regarding self examination. He/she should know where to look for in case of a squamous cell carcinoma.

Every unusual changes/developments should be critically looked at. Patients with an increase or change in complaints should after telephonic contact be seen in very short notice, due to the high risk of squamous cell carcinoma.

Follow-up is advised by someone trained in this field by recurrence of complaints from patients, uncertainty regarding dVIN and with everyone with a history of squamous cell carcinoma or dVIN and also when LS occurs in the family.

Final recommendation concerning patient education

The patient **should be informed** regarding self examination. In case of an increase or change in complaints **he/she should contact the current physician**.

The final recommendation is two sentences, only stating *that* the patient needs to be informed and not *why* the patient needs to be informed and about *what*.

Next recommendation concerns the use of crèmes. The workgroup agrees that the use of crèmes is recommended. In the draft recommendation a few crèmes are mentioned by name, due to the assessment from SLS of current treatment schemes used by 530 LS patients. The workgroup-members do not want the guideline to recommend any crème in particular and various reasons are introduced for this. Firstly, the workgroup argues that there are so many different crèmes available and their experience is that patients have their own preferences regarding crèmes. Secondly, they do not want to favour certain crèmes and others not since the effectiveness is different among different patients. Thirdly, since crèmes only serve to make the skin stay flexible the workgroup does not regard crèmes as having medical efficacy contrarily to corticosteroid crèmes. Therefore, no crème is explicitly recommended.

Subsequently, the patient representative proposes to include a number on the prescription of crèmes to make it explicit how often crèmes should be used. He argues that overall, patients use crèmes twice a day and proposes to include that in the recommendation. This argument is not discussed endlessly like the discussion about the biopsy. The workgroup-members argue that there is no scientific evidence on the fact that the use of crèmes twice a day is the most effective treatment. Incorporation of an explicit figure cannot be linked to scientific research and articles and is therefore turned down. The workgroup-members agree that every physician in practice should prescribe the quantity of the use of crème tuned to each individual patient.

Draft recommendation concerning the use of crèmes

It is recommended to let patients use indifferent crèmes on the LS affected areas during treatment and during periods of low disease activity like cremor vaseline FNA, paraffine vaseline or lanettecrème II FNA.

Half a fingertip unit is sufficient for the vulva.

Final recommendation concerning the use of crèmes

The use of emollients is recommended.

The final recommendation indicate that the use of (any) emollients is recommended. It does not indicate *which* and *how much* emollient should be used.

The fourth recommendation concerns surgery. One physician, a gynaecologist, is strongly in favour to proceed to surgery when non-invasive treatment does not result in the desired outcome. However, every other workgroup-member does not perform surgery besides the introitus-plastic¹⁸ on occasion. They do not agree with the opinion of the gynaecologist and argue that non-invasive treatment is the standard treatment for LS. This disagreement is not discussed in depth since except for one member everyone agrees about this point of view.

Draft recommendation concerning surgery

Excision of the affected epithelium is an option with therapy-resistant lichen sclerosis.

With severe dyspareunia due to lichen sclerosis the possibility of surgical treatment can be considered.

In case of surgical treatment, treatment by a sexologist and pelvic physiotherapist ought to be considered.

Final recommendation concerning surgery

In case of severe dyspareunia due to narrowing of the introitus surgical treatment can be considered.

In case of severe phimosis surgical treatment can be considered. If the phimosis cannot be eliminated by treatment with local corticosteroids classification 3 of 4 and massage of the preputium circumcision is the preferred treatment.

In case of surgical treatment a preoperative consult with a registered sexologist and/or pelvic physiotherapist should be considered.

The workgroup in the end states that every other non-invasive treatment must be considered before surgery is an option. In the interview I held with the gynaecologist concerned he explained that he did not felt any support from the workgroup. He had the feeling that the physicians were very sceptical about operations. In the interview it became apparent that there was a wide misunderstanding about the reasons for surgery. The workgroup thought that only itching as symptom is the reason why the gynaecologist performs surgery. But the gynaecologist explained in the interview that surgery is also performed to improve sexual functioning. But he was not given the chance to introduce this argument to the workgroup. "I noticed on all sides hesitations, reservations. I have tried to indicate that in the workgroup but at one point I gave up" (gynaecologist, personal communication).

¹⁸ Dilation of the entrance of the vagina.

❖ *Starting research questions*

According to the president of the NVDV the starting research questions ensures the LS patient organisation that patient preferences are taken into account during the whole process. In the interview with the president of the NVDV he remembers that the ‘timely referral’ and the ‘consultation time of five minutes’ were the most important elements of the SLS. The fact that these elements were not explicitly mentioned in the guideline does not mean that they were not addressed. The president of the NVDV continues saying that in an evidence-based guideline the workgroup is inherently reserved to formulate recommendations that cannot be based on scientific evidence. When the evidence lacks, the recommendations cannot be judged as reliable, although the workgroup can agree about the statement. The patient representative believes that during the development *process* the workgroup-members have lost sight of the starting research questions especially due to time constraints. When the guideline was finished the workgroup did not return to the research questions, so the important five minutes consultation time was not explicitly addressed anymore in the *outcome*, the guideline. But the president of the NVDV is convinced that the starting research questions have led the process, although not all questions are explicitly traced back in the guideline.

The LS guideline development process was constrained by time lines as is normal with the development of guidelines. The process was bounded to end at March 2012. “We were not able to monitor that [the time line, FV] adequately” (president NVDV, personal communication). The guideline was finished right before the annual NVDV society meeting. In the last workgroup meeting no time was left to return to the starting research questions. But as is mentioned in section 4.3, the fact that the patient representative was considered as a motor within the whole process led to the LS guideline being so extensively described within the constrained timeline.

❖ *The final LS guideline and ‘the patient perspective’*

The multi-disciplinary evidence-based guideline is 76 pages long, predominantly filled with medical information from a scientific perspective. The guideline is a ‘guidance for daily medical practice’. Therefore, it is suitable for every physician concerned with the treatment of LS patients. The objective states that the guideline gives recommendations about the guidance and treatment of LS patients with the attention on psychosocial care and patient education. Besides the evidence-based parts, the LS guideline pays a lot of attention to the non evidence-based part of LS care too, especially psychosocial guidance and education.

The added value of the experiential knowledge from patients is acknowledged by all interviewees. “Without the participation of SLS the emphasis would have been put on the medical-technical part of the guideline” (gynaecologist, personal communication). A methodologist explained that the wealth of knowledge especially regarding the insights on treatments is valuable information that is unavailable for researchers from scientific articles or from experiences from physicians only. This is because the SLS is so close to the target group.

All the interviewed participants are of the opinion that the additional contribution of the SLS is ‘the patient perspective’ as annex. An epidemiologist from SNH feels that without the participation of the SLS (and the financial contribution) ‘the patient perspective’ would not have been addressed at all in the guideline since it was necessary to actually perform a research to ascertain the aspects concerning treatment satisfaction and the QoL of LS patients.

A patient version of the LS guideline has been drafted by the NVDV. SLS was asked to give feedback on the draft multiple times. The patient representative distributed this draft to a few workgroup-members including the chairman of the workgroup, to provide the draft with feedback. All the applied changes by the NVDV were made in consultation with the representative.

5. Chapter 5 Bringing research into practice and back again

5.1 General overview

This chapter brings research into practice and back again. I brought my research into practice and described the results in chapter four. This chapter brings it back again, connecting the research results with the applied theory in order to formulate the conclusions, implications and limitations. The next section is concerned with the conclusions. In separate paragraphs the three sub questions are individually answered. An answer on the main research question is provided in the third section. The discussion is described in the fourth section. Both the implications and limitations of this research are outlined in that section.

5.2 Conclusions

- ❖ *Sub question 1: In which ways are the lichen sclerosis patients involved in the lichen sclerosis guideline development process?*

The development of the LS guideline is initiated by the SLS and they contributed to this project with a substantial amount of money. The development of a Dutch LS guideline is accelerated due to this financial contribution.

From the literature it is known that patients mostly participate in the starting phase of guideline development with the assessment of bottlenecks and the draft of the starting research questions. It is frequently the only phase in which patients participate (Broerse et al. 2010: 20). The LS patient representative is actually present and direct participating in every phase of the LS guideline development process. The fact that the patient representative has been involved from the early beginning of the process and was the one composing the (members of the) workgroup is inherently related to the fact that the patient organisation was the one initiating the development process (ibid.: 20), which implies that this is a normal state of affairs and not an exception for the LS patient representative.

The starting research questions are considered as *the* method to involve the perspective of the patient within guidelines (ibid.: 57). These starting research questions should guide the development process in order to achieve the objectives of the guideline and to address the patient perspective within the guideline. With an assessment of bottlenecks, SLS formulated all the initial starting research questions that addressed the bottlenecks from a LS patient perspective. The patient representative as well as LS patients contributed significantly to this procedure within the guideline process. They were not only involved with formulating but also with answering one-fourth of the starting research questions.

There are examples of partnership (Arnstein 1969: 221) between the patient representative on the one hand and policy-makers, physicians and methodologists on the other. For instance, the patient representative brought along scientific articles that gave new insights on heredity and he initiated a change in the guideline text. Further, the SLS paid a dermatologist to execute a research regarding differential diagnoses. These results are one-on-one integrated in the LS guideline. Additionally, the patient representative participated actively in medical discussions; in judging the strength of scientific evidence and in the formulation of recommendations. The patient representative has been asked to provide feedback on draft guideline versions several times and all the applied changes were made in consultation with him.

The involvement of LS patients is evident in the annex ‘the patient perspective’ since this is developed with the use of a focus group and a questionnaire among LS patients. This is completely paid for via an additional project subsidy that has been requested by SLS. The preferences of LS patients regarding treatment satisfaction are ascertained through a focus group. The focus group, seen as a consultative, passive participation method (Arnstein 1969: 219, Broerse et al. 2010: 8) together with the desk research provided for the adequate input to develop the questionnaire. With the specific knowledge

belonging to SLS a study-specific questionnaire could be created. The questionnaire has been tailored for LS patients with the extension of questions about for instance smoking and heredity. A generalization of LS patients' preferences is obtained through consulting LS patients via the questionnaire.

The results of the focus group and the questionnaire (the baseline measurement) are used as input for the end research report. However, the knowledge and the preferences specific for LS patients are not mentioned in 'the patient perspective' since this annex only describes the results in a very summarized way. The domains regarding treatment satisfaction (effectiveness, safety, user friendliness, information provision, physician-patient relation and organisation of the treatment) are listed together with the results from the questionnaire. The relative importance of the domains is provided too. But for a clarification of the (generic) domains the reader is referred to the full research report. As the president of the NVDV pointed out that it is very unlikely that physicians actually read 'the patient perspective', it is thus even more unlikely that the reader searches for and reads the extensive full research report of 86 pages. Without a clarification of the aspects, the generic domains can be interpreted differently by every physician. And it is precisely the clarification of these aspects that LS patients were asked to join the focus group in the first place. As a result, the domains that are given the highest priority in 'the patient perspective' are not prioritized in the guideline.

The initiation of the LS NHG-standard is a result of one of the starting research questions of SLS. The same applies for the cooperation agreement between NVDV and NHG. The feedback of SLS on the draft version of the LS NHG-standard is not acted upon but SLS is mentioned as referent in the NHG-standard. This is an example of pseudo-participation (Arnstein 1969: 218). Nevertheless, the fact that the NHG did not act upon the seemingly passive participation of SLS is inherent to mono-disciplinary guidelines since they hardly make use of patient participation methods (Broerse et al. 2010:16).

In conclusion, the discussed participation methods can be seen as conditions for having an influence on the decision-making process. After all, one cannot have any influence when one does not participate at all. LS patients and the LS representative have been participating in every phase of the LS guideline development process. Moreover, all forms in which patient participation can take place, as discussed in section 2.4, have been used. The focus group and questionnaire are seen as passive and consultative methods of patient participation according to Sherry Arnstein's ladder of participation. However, they have contributed significantly to the development of 'the patient perspective', although the majority of this perspective has been lost during the translation of these results into the policy-document. The focus group and the questionnaire are apparently adequate methods to assess the perspective of patients but not strong methods to have influence on the process. The same applies for the provision of feedback on the LS NHG-standard, although this is not specifically inherent for this case. The patient representative on the other hand did have influence in decision-making processes. Due to partnership, the patient representative participated as equal workgroup-member. Moreover, decision-making responsibilities were shared and the representative initiated and decided on various procedures by himself.

❖ *Sub question 2: What work has the lichen sclerosis patient representative done in order to be heard in the guideline (workgroup) proceedings?*

There are factors that influence the extent of patient participation as is explained in section 2.4. The characteristics of the LS patient organisation do not fit the ideal situation. It is a small organisation with four board members. Among them only one person represents the collective interests. Though, SLS prioritised participation possibilities and they were therefore willing to invest the large majority of the obtained subsidy in the guideline development and coinciding projects. In turn, this strongly focussed approach created the conditions for an efficient process and constant pace in which the development of the guideline took place.

By entering into various tight relationships and alliances it became possible for the patient representative to align interests. Right from the beginning of the process interests were mutually adjusted with various workgroup-members. For instance, there was a shared interest for the initiation

of the LS guideline. This resulted from a joint concern between a dermatologist and the SLS. Through informal email exchange and a few dinners, interests were mutually adjusted between the two parties. The fact that SLS contacted the NVDV regarding the development of the LS guideline was no coincidence but rather a strategic move. They could have contacted the Dutch Society of Obstetrics and Gynaecology as well. It was also not a coincidence that *this* dermatologist was chosen as chairman of the workgroup.

Besides the alignment of interests, the patient representative also practised his role via creating and maintaining close relationships with several physicians. The representative and certain workgroup-members had an existing longstanding relationship with each other. In these previous interactions the representative gained trust and loyalty from these physicians. The chosen workgroup-members were not randomly selected. The representative deliberately chose physicians which he had close relationship with.

The patient representative used existing trust relationships with physicians to get involved in every phase of the process. Moreover, he used the financial contribution of the SLS in various occasions as argument to be physically present, for instance during the focus group and the assessment of scientific evidence. The fact that the representative was physically present during the whole project made it possible to represent the LS patient during every methodological phase of the process. That this really made a difference became apparent during the focus group in which the representative stood up for the preferences of the male LS patient.

The representation of LS patients is considered to be professionally performed and the participation of the representative is evaluated as a stimulator, a motor and a binding factor. This is mainly due to all the (in)formal work that the representative has done as described in previous paragraphs and in section 4.4. The binding factor of SLS led to the creation of support and trust among the most prominent and important actors (physicians) involved with LS care. This network of important actors was enabled to contribute in the development process one way or the other. Loyal physicians who did not join the workgroup as member were given the opportunity to contribute in another way as is explained in section 4.4.

Overall, the patient representative practised his role in a professional way. During the LS guideline development process he had an active attitude, was always present and prepared, independent and assertive. The representative used medical jargon and used these words in the right context. The workgroup-members could therefore judge that the representative knew what he was talking about. This all contributed to the workgroup-members considering the representative as a full and equal member of the guideline workgroup.

Within the literature it is argued that whenever patients participate as full and professional workgroup-members it delineates them from the initial tasks they were asked for; the input of experiential knowledge and the representation of patients (Van de Bovenkamp 2010: 158, Van de Bovenkamp et al. 2013: 11, 12, 16). So, patient representatives who become ‘pseudo professional’ workgroup-members bring along a paradox: in order to be taken serious *as* patient, patient representatives have to be *less* of a patient. This need not be a problem whenever they still can contribute the initial assignments they were asked to participate for in the first place. This research shows that, by utilizing different instruments and mechanisms, the LS patient representative becomes a ‘pseudo professional’ workgroup-member who at the same time is able to represent the LS patient and to put the LS patient perspective forward.

In conclusion, through private negotiations, strategic moves and the creation of tight relationships, the LS representative aligned interests and bound with important actors. All these (inter)actions turned out to be very important in order to create partnership and to establish legitimacy as patient representative. Herewith, several (in)formal procedures within the development process were negotiated outside the official workgroup proceedings. Though the development process was bounded by stringent deadlines, the work that the patient representative has done certainly contributed to the extensiveness and multidisciplinary approach in which the guideline is described within the fixed time schedule. Moreover, not only has the representative become a professional guideline workgroup-member. He has also maintained his initial tasks as patient representative.

- ❖ *Sub question 3: In which way is the produced knowledge within the lichen sclerosus guideline justified in terms of the technical robustness, practical usability, political acceptability and methodological adequacy?*

The process of justification and coming to shared consensus about knowledge was most prominent during the workgroup meeting in which the exact formulation of the recommendations were discussed. All four types of collective judgements were considered during this specific workgroup meeting.

When formulating the recommendations the workgroup judged whether these were technical robust. This to let the guideline produce reliable knowledge through scientific evidence and to reduce the possible resistance from the outside world (Moreira 2005: 1978). This led to the exclusion of some recommendations. For example, the explicit mentioning of using crèmes twice a day, as the patient representative proposed, could not be based on scientific evidence and was therefore omitted.

Surgery as treatment for LS patients was evaluated from the point of view of current best practice and the available evidence. Since LS patients are mostly treated by dermatologists and not by gynaecologists, surgery is not so often performed. There are few scientific studies available regarding the effects of surgery. Thus, there is not much evidence about the likely benefits and harms of different options for surgery. The lack of evidence consequently led to the recommendation being based on experiences from the workgroup-members. Since the majority of the workgroup-member were dermatologists they agreed that non-invasive treatment is the current best practice for LS patients and the final recommendation state that surgery can be an option when all other (non-invasive) treatments are considered.

The recommendations were also judged in light of their usability in practice. The usefulness of the draft recommendation about patient education was not judged applicable to practice. The workgroup-members did not want to impose an authoritarian and didactic opinion stating that other physicians are unaware about the importance of why patients should be educated and about what.

The fact that the patient representative wanted to include an explicit number in the recommendation concerning the use of crèmes is found to be unfit with daily medical practice in order to be of practical usability. The workgroup agreed that after all, patients have their own preferences regarding the use of crèmes. In the final recommendation the discussion about the use of crèmes (which one and the amount of use) is redirected towards individual patient-physician interaction. This is also the ideal social action that a recommendation should lead to, the guideline is just one input in order to make clinical decisions (ibid.: 1980).

The political implications of the formulated recommendations were considered carefully. The workgroup agreed that a biopsy is not always necessary in order to diagnose LS. However, GPs are considered to be incapable to diagnose LS based on clinical inspection only. It is furthermore questioned by the workgroup whether GPs are able to adequately perform a biopsy. But the cooperation agreement between the NVDV and the NHG and the political implications of this agreement were also taken into account. This meant that the workgroup-members did not want to offend GPs by stating that GPs are incapable to perform biopsies. Though, the workgroup-members did want a change in the current situation and they also agreed on the justification for it since diagnostic delay is still common practice. Several arguments are introduced in the discussion to amplify their opinion like own experiences and examples out of daily medical practice. In the end the final recommendation does not impose a standpoint in which physicians are favoured to perform biopsies and GPs are brought in a disadvantageous position.

The workgroup as a whole represent the position in which the guideline is developed, namely the association of dermatologists (and not gynaecologists). Overall, dermatologists believe in non-invasive interventions and therefore do not want to favour surgery. Evidently, it does matter what specialism is a member of the workgroup as they decide on the content of the recommendations.

Patients, overall less participating in the repertoire of science, can address any imbalance of participation through assessing the methodological adequacy of the produced knowledge (ibid.: 1983-1984). Any imbalance in participation that possibly could have happened within the repertoire of science (or other repertoires) is tackled by the patient representative due to his visible participation

among the workgroup-members discussing the evidence-based part of the guideline (and any other phase of the development process). The representative could therefore not only participate but he could also assess the methodological adequacy of the process. From that, he could evaluate the construction of the science part of the guideline himself and the processes in which it took place.

The starting research questions can be used as a procedure to assess the methodological adequacy of the produced knowledge within the guideline. These research questions should ideally lead the guideline development process in order to take the formulated bottlenecks into account. Answering all starting research questions, which have been formulated by SLS, was the assignment of the workgroup that they were supposed to accomplish. The fact that not all starting research questions are explicitly answered is judged as a deficiency by the patient representative. The president of the NVDV believes that the research questions have led the guideline development process. The fact that indeed not all research questions are dealt with is due to the fact that not every research question could be based on scientific evidence and therefore the workgroup was reserved to formulate recommendations. Moreover, the development process was constrained by a stringent time line that was not monitored adequately.

The patient representative could assess the methodological adequacy of the development process of 'the patient perspective' due to his visible presence during the execution of the focus group (and any other phase of that development process). 'The patient perspective' is not included *in* the evidence-based guideline since this project went totally isolated and separate from the evidence-based guideline development process. The timelines of the two projects were not adjusted to each other and there was no cooperation between researchers, methodologists and policy-makers. The adequacy of the methodological process of 'the patient perspective' is questioned by the president of the NVDV. The argument brought forward is that the preferences of patients do not match the conclusions and recommendations in the evidence-based guideline. *Therefore* the annex is unauthorized. This implies that the NVDV does not formally acknowledge the content of 'the patient perspective'. 'The patient perspective' therefore does not have a coercive character and it misses a sign of support and justification from the NVDV too.

In conclusion, the produced knowledge of the guideline is justified in light of all four types of evaluation. The justification of the technical robustness and methodological adequacy of the produced knowledge prevented the incorporation of experiential knowledge in the LS guideline the most. Justifying the technical robustness of the produced knowledge is the repertoire that brought along short but the most firm and resolute discussions. The technical robustness of science prevailed over experiential knowledge. Even a physician is overruled by his own colleagues as evidence for surgery as treatment lacked. The methodological adequacy of the produced knowledge is a prominent repertoire of evaluation within this guideline process. The stringent methodology of the evidence-based LS guideline development process prevented any incorporation of patients' preferences as mentioned in 'the patient perspective'. And as some starting research questions could not be based on scientific evidence, they were not answered; the lack of scientific evidence is hereby viewed as a valid justification and excuse for methodologists and policy-makers.

The justification of the practical usability and political acceptability of the produced knowledge allowed for other considerations besides (the strength of) scientific evidence and the fixed guideline development methodology. The patient representative participated within discussions justifying the practical applicability of knowledge. The fact that his input was not acted upon was due to the fact that the workgroup took the uniqueness of individual patients into account redirecting decisions to daily medical practice. The political acceptability of the produced knowledge is considered carefully with in depth discussions and the introduction of own experiences by the workgroup-members to eventually reach consensus.

5.3 How and to what extent are patient preferences and patient knowledge incorporated in the LS guideline?

The first sub question discussed all the methods of patient participation within the LS guideline development process. The methods of patient participation set the conditions for having an influence on the process although it did not automatically mean that it was always acted upon. The second sub

question clarified all the (in)formal work that the LS patient representative has done in order to strengthen his voice and empower his role towards researchers, policy-makers, methodologists and workgroup-members involved in the process. The third sub question provided for an explanation of the process in which knowledge was evaluated and justified according to the four repertoires of evaluation.

‘The patient perspective’ is an explicit and visible reference to the incorporation of LS patients’ preferences and knowledge. The LS patient representative and LS patients participated through various methods that eventually led to the development of the LS patients’ perspective. It took a whole research of three years in which various consultative and partnering methods were used to collect data. Arnstein’s participation ladder (1969) is used to help explain the contribution of the different methods of patient participation. The composition of a focus group together with a questionnaire formed an adequate methodology for defining, assessing and measuring the preferences of LS patients. The input from the focus group was essential to ascertain an insight in the domains of treatment satisfaction and to develop a study-specific questionnaire. The questionnaire, in turn, was an adequate instrument to consult an illustrative group and to ascertain a generalised perspective of the LS patient.

Since specific information is omitted in the outcome one could assume, based on ‘the patient perspective’ per se, that the incorporation of LS patients’ preferences and knowledge was non-existent. But the fact is that ‘the patient perspective’ could not be developed at all without the participation and the involvement of the knowledge from LS patients. The patient representative provided specific contributions like the representation of the male LS patient during the focus group and the additional knowledge that led to the composition of a study-specific questionnaire.

The justification of the technical robustness and methodological adequacy of the produced knowledge and the stringent methodology of the LS guideline development process prevented any incorporation of patients’ preferences in the evidence-based LS guideline, as mentioned in ‘the patient perspective’. Although ‘the patient perspective’ has become an unauthorized annex this does not mean that LS patients did not have any influence on the content of the evidence-based LS guideline; that LS patients’ preferences and knowledge were not taken into account during the guideline proceedings or that LS patients’ knowledge has not been incorporated in the LS guideline. In this, the patient representative was the binding factor among all parties in which he adequately represented the LS patient.

The patient representative has been actively participating within the whole development process and he had influence in decision-making processes. This was reinforced by all the (backstage) work and thereby gaining the trust and legitimacy of important actors to consequently be taken seriously and to be heard in the guideline workgroup proceedings. He even could be accorded a full workgroup-member and participating as partner. In accordance with the literature (Van de Bovenkamp 2010: 158) this indeed enhanced the effectiveness of the decision-making processes. But while it is argued that professionalization hinders the input of experiential knowledge and the task of representation (Van de Bovenkamp 2010: 158, Van de Bovenkamp et al. 2013: 11, 12, 16), this was not the situation within this development process. Precisely because of the active and visible participation of the representative he could introduce knowledge from the specific perspective of LS patients in every methodological phase. He could also represent the LS patient adequately throughout every methodological step of the process.

The formulation of (and answer on) starting research questions is regarded as *the* most important method to involve the perspective of patients (Broerse et al. 2010: 57, Zuiderent-Jerak et al. 2011: 6). However, that knowledge is judged by four different repertoires of evaluation prevented the explicit answering of all starting research questions. Through a combination of the four repertoires of evaluation the workgroup collectively judged all evidence and knowledge to eventually produce recommendations that are technically robust, practically useful, politically acceptable and methodologically adequate. Producing reliable technical robust knowledge that at the same time is justified according to methodological adequate proceedings prevented the incorporation of experiential knowledge the most. In this, the technical robustness of science prevails over experiential knowledge.

Justifying the practical usability and political acceptability of the produced knowledge allowed for an incorporation of the knowledge that the patient representative brought to the (policy-) table. For example, when it concerned the starting research questions; the provision of scientific articles that gave new insights on heredity; the incorporation of differential diagnoses; the extensiveness in which psychosocial guidance and patient education has been addressed and the cooperation agreement with the NHG that is stated within the guideline.

Consequently, and in light of the main research question addressed in this thesis, a few concluding remarks can be drawn from this research. It proves to be difficult to methodologically justify and incorporate patients' preferences and knowledge in an evidence-based methodology of guideline development. This is due to the fact that all four repertoires of evaluation are considered and combined and have their influences on the development of the guideline. It turns out that the technical robustness of the produced knowledge within the guideline and the methodological adequacy of the proceedings in which this knowledge is established prevent to fully incorporate patients' preferences and knowledge.

The formulation of starting research is an important tool to explicitly introduce the perspective of the patient. But the fact that starting research questions are not (explicitly) answered does not mean that patients do not contribute with their specific knowledge and preferences or that the patient perspective is not taken into account during the guideline proceedings.

The explicit referral to the input of patients within the guideline is not an adequate measure of the contribution that patients have with their knowledge and preferences. Hereby should be mentioned that the incorporation of LS patients' preferences and knowledge are most visible within the annex 'the patient perspective' due to the chosen methodology of the whole LS guideline process.

By considering 'the patient perspective' as annex as a given fact, experiential knowledge from patients has certainly been incorporated in the evidence-based guideline, although looking at the guideline per se one could not ascertain that. The input concerning the knowledge specific for LS patients within the LS guideline is due to the fact that the patient representative has been actively participating within the whole process. All the (in)formal work that the patient representative has done contributed to the extensiveness in which the guideline is patient-centred approached.

5.4 Discussion

Current methods of patient participation possibilities within guideline development processes require a critical reflection. The patient is assumed to be 'the third party' and 'the guideline for guidelines' explicitly mentions that experiential knowledge from patients should be incorporated into guidelines. However, in this research it has become apparent that researchers, policy-makers and methodologists lack sufficient methods to adequately involve patients' preferences and knowledge within an evidence-based methodology of guideline development. This gap needs to be bridged if patient participation in the future must have an adequate continuation. Moreover, the stringent conditions of PGO on which subsidies are provided do not permit individual patient organisations to spend its financial resources to any other destination besides facilitating information and peer support (TK 2011). The tasks of professionalization and representation of patients has ceased to meet the conditions of PGO as from 2012. However, this research has demonstrated that the LS patient organisation was in the lead mainly because of the large amount of financial resources it has contributed to the whole process and additional projects. In order to ensure that patient organisations are in the lead, they themselves should be able to manage the financial resources and PGO should loosen its stringent conditions in order to avoid that patient organisations are forced to creative accounting to achieve their goals.

Active patient participation is assumed to lead to patient-centred guidelines. After all, experiential knowledge from patients provides for an additional and unique perspective on (the symptoms of) conditions and the experiences with different treatments. However, research on patient participation shows disappointing results in practice (Van de Bovenkamp et al. 2013: 7, Zuiderent-Jerak et al. 2011: 57). Although there are qualitative studies performed, like the study of Broerse et al. (2010) the contributions of patients participating within guideline development processes has, in previous studies, also been examined via quantitative measurements (Van de Bovenkamp et al. 2013, Zuiderent-Jerak et

al. 2011). But the same studies also conclude that the input of patients is frequently not explicitly incorporated in guidelines. Therefore, it is difficult to assess and determine what happens to the input of patients based on the text of the guideline only and what the added values of patient participation are. This research has shown that a qualitative study on patient participation yields interesting insights.

The explicit referral and quantitative measurement of the input of patients within guidelines is not an adequate measure of patients' contribution with their knowledge and preferences, as this research evidently demonstrates. It can therefore be assumed that research on patient participation via a quantitative measurement results in an understated measure for participation, especially if based on the text of guidelines themselves. Future research about (the added values and true measure of) patient participation within guideline proceedings should be done qualitatively in order to explore *how* patients can contribute with their specific perspective, *how* this influences the content of the guideline, whether *or not* 'more participation is better' and whether *or not* it is a problem at all when certain (patient-centred) aspects are omitted in the guideline.

If patients should participate at all, with what methods and in which phases of guideline development processes depends among others on the added value participation has on the care patients receive in daily medical practice. It is not yet known whether patient participation leads to an increase in the quality of care individual patients receive. Further research can explore to what extent a (patient-centred) guideline influences the care patients receive. For example, this can be done with the cut-off values that were mentioned in section 4.3. Measurements of PROMs can be performed before and after the implementation of a guideline. Via repeated measurements of various PROMs it becomes possible to perceive a trend (before and after the implementation of the guideline) and can therefore be used as a tool to measure and monitor the quality of care. The cut-off values provides for an insight in whether or not PROMs should be enhanced and can therefore be used as impulse for the further improvement of the provided care.

The participation ladder of Arnstein, that is frequently applied to research the added values of patient participation, supports the argument of 'more (participation) is better'. Using the initial manner of the participation ladder it would be argued that for instance a focus group and questionnaire are not preferred in comparison with participation as member of a workgroup since the latter is accorded as more participation and *therefore* better. Using the participation ladder is presuming that patient participation should take place in a specific format, namely only participating more and more.

I argue that the discussion about patient participation should be more turned towards the specific contribution that patients can bring forward with the different methods of participation. In this specific research the patient representative was able to adequately perform his tasks: representing the LS patient; bringing forward the specific experiential knowledge from patients; participating as professional workgroup-member. But one can wonder whether other patient representatives are capable (and willing) to do the same since they for example cannot follow medical discussions or are not able to invest so much effort since they are burdened with the condition themselves. In light of this, it cannot be said that participation as workgroup-member is better than the focus group as for example from this research, the unique perspective of the LS patient could not be assessed and determined without the execution of a focus group.

Still, this does not eliminate the fact that when patients *do* participate, they still remain in a dependent position (Van de Bovenkamp et al. 2013: 10-11) concerning the extent in which the knowledge is utilized. There are barriers why patients' experiential knowledge does not end up in guidelines. Patients have to be provided with adequate participation possibilities to ascertain the patient perspective. Focus groups and questionnaires are adequate methods to understand and generalise this perspective as is evident from this research. The knowledge obtained from these participation possibilities supply the additional perspective of patients that can increase the quality of the decision-making processes and the guideline itself. However, patients participating in separate projects without a cooperation of the guideline development group, makes it virtually impossible to include this perspective within an evidence-based guideline development methodology. The involvement of patients with whatever method cannot take place without a cooperation of guideline workgroup-members since they decide on the content of the guideline.

The conclusion of this research implies that the process of guideline development is very dynamic and incremental. Various parties (in this research twenty-seven) with multiple interests have to reach consensus about best practice regarding a certain patient population. Through guideline development a redefinition of knowledge takes place. During the entire guideline development process (experiential, expert and evidence-based) knowledge is mobilised and transferred. The development of guidelines as *process* is a tool to mobilise and transfer knowledge as the *outcome* (the guideline) in itself is as well. This all happens within the context of the guideline and has its effects. This research shows a number of these spin-off effects. During the LS guideline development process unresolved questions have led scientists and professionals to initiate further research based on the evolved insights regarding LS. As a consequence multiple studies have been directly derived and conducted from this guideline development process. This is shown via various scientific articles that have been published (Van de Nieuwenhof et al. 2010, Burger 2011, Chi et al. 2011, Van Gestel 2012, Maassen et al. 2012, Lansdorp et al. 2013, Mevius 2013). Furthermore, the LS guideline has been incorporated in the permanent educational programme of GPs and dermatologists. This has led to even more research and publications. This methodological observation about the importance of the *process* underpins the fact that research is being put to practice and back again.

❖ *Limitations*

As with any other research this research has his limitations too. This research is carried out within a specific context (the development of a dermatological guideline with a multidisciplinary workgroup), with a specific patient population (LS patients) and patient representative (academic educated person, male, 40 years old, not burdened with the condition himself). The LS patient organisation prioritised participation possibilities very strong; they initiated the guideline process themselves and they were willing to invest the large majority of the obtained subsidy in the guideline development process. Through this, one could have expected that LS patients had a lot of influence on (decision-making) processes.

The transferability of these research data to another context or population can be more difficult due to the specific context, patient population and patient representative. Given that every research is unique (Mortelmans 2011: 442) perfect generalisation is impossible. But then again, the reproduction of research results within qualitative studies is not a target in itself. Still, insight is gained into this specific context, patient population and the (evidently invisible workings of the) patient representative in order to initiate further research and to explore unresolved questions and gaps. What *is* a target in itself within qualitative studies, is producing the most credible data as possible to ensure that a reliable and valid research has been conducted.

The reliability and validity of the research results could have been influenced by the researcher. I could have influenced the research process since I translated the participants' words and actions from the observations and other data collection methods. And as my interpretations were not exactly replications of the data but rather my impression of it, I could have misinterpreted opinions and experiences of participants. The methods that are used to prevent and to minimize misinterpretations are self-reflection, data triangulation, the extensiveness of the research and interviews.

The advantage of being a patient and a nurse myself is that I could follow every (medical and scientific) discussion perfectly. Through the different data collection methods I perceived different perspectives of the participants and their words and actions. The informal communication schemes contributed to the interpretations of the data collected from observations. Especially the strategic (inter)actions and private negotiations between the patient representative and several physicians could therefore be discovered and understood. The interviews gave an additional advantage in that it was a way to verify the results from the observations and to check my interpretations with the participants individually. The combination of these methods resulted in an interpretation of the research results which eventually led to the most credible data as possible.

Despite its limitations this research contributes to existing knowledge about patient participation, since limited research on this subject has been conducted so far. Thanks to the intensiveness of this research project, the positive experiences of professional workgroup-members with patient participation are taken into account that broadens the current picture of patient participation in practice. Having

explored a guideline development process in depth and over a substantial period of time, private, confidential and sensitive information and proceedings shed an interesting light on all the backstage occurrences that influenced the official front stage (inter)actions. A clear insight is obtained in the reasons why certain (patient-centred) aspects were included and omitted in the evidence-based guideline. Were it not for the extensiveness and intensiveness in which this research is carried out, it would not have delivered this unique insight about patient participation within a guideline development process.

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Appendix 1 Overview data collection

	Date	Location	Type activity	Objective	Relevant actors involved
1	29-10-2011	Houten	Annual LS patient conference		SLS, LS patients
2	21-11-2011	AMC, Amsterdam	Steering committee meeting	Observation; feedback study proposal questionnaire quality satisfaction	SLS, SNH
3	01-12-2011	Domus Medica, Utrecht	Workgroup meeting	Observation ; introduction of norm scores to multidisciplinary workgroup members. Review recommendations in LS guideline.	SLS, multidisciplinary workgroup members, policy-makers NVDV
4	12-01-2012	Domus Medica, Utrecht	Steering committee meeting	Observation; determining definitive questionnaire.	SLS, SNH
5	24-01-2012	Domus Medica, Utrecht	Steering committee meeting	Observation; determining the operationalization of questionnaire and division of tasks for the execution of the questionnaire.	SLS, SNH
6	30-03-2012	Congress centre Papendal, Arnhem	Annual NVDV society meeting	Training on content of LS guideline and authorisation LS guideline.	Dermatologists, NVDV, SLS, speakers.
7	19-04-2012	Erasmus Medical Centre, Rotterdam	Symposium; patient-centred care	What is patient-centred care?	Informal interview; chronic patient with immunodeficiency
8	22-04-2012	Houten		Observation; board meeting LS patient organisation	SLS
9	01-05-2012	Domus Medica, Utrecht	Steering committee meeting	Observation; results questionnaire.	SLS, SNH
10	13-09-2012	National Cancer Institute, Antoni van Leeuwenhoek Hospital,	European College for the study of vulval disease	Interdisciplinary forum for exchange and discussion dealing with many aspects of vulval	

	Date	Location	Type activity	Objective	Relevant actors involved
		Amsterdam		conditions by gynaecologists, dermatologists, sexologists and pathologists.	
11	16-10-2012	Delft			Informal interview workgroup-member; gynaecologist
12	25-10-2012	Beatrix theatre, Utrecht	Jaarbeursessie 2012 PG-organisaties	Meeting of the Ministry of VWS for PG-organisations. Objective is to explore in what way and to what extent patient experiences and the patient perspective can gain more influence in the provision of care. Focus is on the activities of the Kwaliteitsinstituut. A few PGO introduces their own examples in practice.	Informal interview; Daily executive Kwaliteitsinstituut
13	27-10-2012	Houten	Annual LS patient conference		SLS, LS patients
14	30-10-2012	Rotterdam		Interview	Workgroup member; pelvic physiotherapist
15	31-10-2012	Havenziekenhuis, Rotterdam		Interview	Chairman workgroup; dermatologist
16	06-11-2012	Domus Medica, Utrecht	Steering committee meeting	Observation; what is the state of affairs and what about the second questionnaire?	SLS, SNH
17	08-11-2012	Amsterdam Medical Centre, Amsterdam		Interview	Workgroup member; gynaecologist
18	20-11-2012	Rotterdam		Interview	Chairman SLS
19	21-11-2012	Domus Medica, Utrecht		Interview	Managing director NVDV

	Date	Location	Type activity	Objective	Relevant actors involved
20	18-12-2012	Domus Medica, Utrecht		Interview	Policy-maker SNH
21	05-02-2013	Domus Medica, Utrecht	Steering committee meeting	Observation; determining the final content and operationalization of the second questionnaire and division of tasks for the execution of the questionnaire.	SLS, SNH
22	04-05-2013	Houten		Observation; board meeting LS patient organisation	SLS
23	22-05-2013	Domus Medica, Utrecht	Steering committee meeting	Observation; Review preliminary results second questionnaire.	SLS, SNH

Appendix 2 Topic list interviews

❖ INTRODUCTION

Background & objective research and interview
Agreement on recording

❖ MAIN PART

Experiences guideline development processes in general
Experiences LS guideline development process
Experiences patient participation within LS guideline development process
Daily medical practice with regard to guidelines
Daily medical practice with regard to knowledge
LS patients
'The patient perspective'
Externalities/NVDV day

❖ CONCLUSION

Remaining issues not addressed so far
Request for contact via email in case of further questions/explanations
Tips about other stakeholders as participant for an interview

Appendix 3 Chronological overview LS guideline development process

❖ LS guideline

Date	Type activity
03-04-2009	Meeting between chairman of the SLS and dermatologist to talk about Achmea.
10-12-2009	Initial meeting with the president of the NVDV and the chairman of the SLS.
26-12-2009	First payment of 5.000 Euros to a dermatologist for the development of a process flowchart for differential diagnoses.
18-01-2010	First meeting with the president of the NVDV whereby the chairman and the members of the workgroup are selected.
11-02-2010	NVDV fills for a SKMS grand.
09-03-2010	Joint intention NHG and NVDV to cooperate together on LS guideline and LS-standard.
10-03-2010	Draft of starting research questions.
24-04-2010	SLS provides an assessment of current treatment schemes as input for the guideline.
01-05-2010	The NVDV performs a literature search and meta-analysis.
30-05-2010	The dermatologist has finished her research (Chi et al. 2011) and delivered the final version of the process flowchart for differential diagnoses.
14-06-2010	Defining the starting research questions for the NHG-standard.
15-06-2010	First meeting workgroup.
14-07-2010	SLS has provided scientific articles from their website to the NVDV to use as input for the meta-analysis.
20-07-2010	NVDV asks the SLS to validate the reference list used as input for the guideline.
01-10-2010	SLS has found articles on PubMed about heredity and provides this to NVDV and workgroup-members as input for the guideline.
01-10-2010	The patient representative talks about the LS guideline in Liverpool in reaction to the British LS guideline which was the fundament for the Dutch LS guideline.
14-10-2010	A gynaecologist external to the workgroup approves to review the LS guideline. In return the SLS sponsors a symposium for 3000 Euros.
13-01-2011	Two-day meeting of the workgroup, the pressure cook method.
21-06-2011	Revised guideline is proposed to the workgroup.
27-07-2011	SLS receives the reviewed LS guideline from the gynaecologist. This serves as an input for the feedback that the SLS gives on the guideline to the NVDV.
25-08-2011	Revised guideline is proposed to the workgroup.
14-09-2011	Draft cooperation agreement between NVDV and NHG.
01-12-2011	Meeting workgroup to determine the final conclusions and recommendations of the LS guideline and introduction of norm scores to multidisciplinary workgroup members.
10-12-2011	Draft NHG-standard is available for comments.
20-01-2012	Every involved professional association is given the time (2 months) to review the definitive LS guideline.
20-03-2012	Approval of the LS guideline by multidisciplinary workgroup.
27-03-2012	Final version of the treatment cooperation agreement between NVDV and NHG.
30-03-2012	Authorisation of the LS guideline on the annual NVDV society meeting.
05-11-2012	Final NHG-standard.
28-02-2013	The NVDV provides a draft of the patient version of the LS guideline.
05-03-2013	SLS provides feedback on the draft of the patient version of the LS guideline.
04-05-2013	Final patient version of the LS guideline.

❖ Patient perspective

Date	Type activity
17-06-2010	Draft application for project subsidy patient perspective.
29-06-2010	Final application for project subsidy patient perspective.
29-12-2010	PGO approves project subsidy.
01-01-2011	Steering committee was formed.
18-01-2011	First meeting steering committee.
01-04-2011	Desk research.
10-05-2011	Second meeting steering committee.
22-06-2011	Third meeting steering committee.
05-09-2011	Invitations for focus group.
20-09-2011	Focus group.
17-10-2011	Steering committee.
29-10-2011	Annual LS patient conference.
21-11-2011	Results of the focus group presented in a report by SNH.
21-11-2011	Steering committee; feedback study proposal questionnaire.
29-11-2011	Joint meeting guideline workgroup and steering committee.
12-01-2012	Steering committee; determining definitive questionnaire.
24-01-2012	Steering committee; determining the operationalization of questionnaire and division of tasks for the execution of the questionnaire.
01-03-2012	Questionnaire among LS patients.
01-05-2012	Steering committee; results questionnaire.
10-07-2012	Results from desk research and questionnaires are translated into a research report.
10-08-2012	Final patient perspective.
27-10-2012	Annual LS patient conference.
06-11-2012	Steering committee; what is the state of affairs and what about the second questionnaire?
05-02-2013	Steering committee; determining the final content and operationalization of the second questionnaire and division of tasks for the execution of the questionnaire.
01-03-2013	Second questionnaire among LS patients.
22-05-2013	Steering committee; review preliminary results second questionnaire

Appendix 4 Starting research questions LS guideline in Dutch

Definitie

- Wat is de definitie van lichen sclerosus?

Epidemiologie

- Hoe vaak komt lichen sclerosus voor (in algemene populatie, eerste en tweede lijn, vulvapoliklinieken)?
- Bij welke leeftijdsgroep (-en) komt lichen sclerosus het meest voor?
- Bij welke bevolkingsgroepen komt lichen sclerosus het meest voor?
- Bij welke geslacht komt lichen sclerosus het meest voor?

Etiologie, pathofysiologie

- Wat is de oorzaak?
- Wat zijn risicofactoren?

Prognose

- Hoe vaak leidt lichen sclerosus tot een maligniteit?
- Wat is het natuurlijk beloop van lichen sclerosus?

Klinisch beeld

- Wat is het klinisch beeld?
- Welke andere aandoeningen lijken hierop (wat is de differentiaal diagnose)?

Diagnostiek

- Welke vragen moeten in de anamnese worden gesteld?
- Welk lichamelijk onderzoek dient plaats te vinden? (m.n. speculumonderzoek)
- Welk aanvullend onderzoek dient plaats te vinden? (m.n. biopsie, kweek)
- Wat is de diagnostische meerwaarde van PA-onderzoek t.o.v. het klinisch onderzoek?
- Wat is de waarde van teledermatologie en fotografische vastlegging voor diagnostiek en follow-up?
- Welke veranderingen wijzen mogelijk op het ontstaan van een maligniteit en moeten vandaar gebiopteerd worden?

Behandeling

Niet-medicamenteus

- Welke niet-medicamenteuze adviezen zijn zinvol (vermijden van bijv. detergentia, synthetisch ondergoed en lubricatiemiddelen; bekkenbodempfysotherapie)?
- Wat is de plaats van operatieve ingrepen?

Medicamenteus

- Welke locale behandeling heeft de voorkeur (+volgorde)?
- Welke factoren zijn verantwoordelijk voor het falen locale behandeling (bijv. secundaire infectie)?
- Welke locale behandeling heeft de voorkeur bij pijn/jeuk?
- Wat is de waarde van een onderhoudsbehandeling?
- Wat is de plaats van systemische behandeling?

Kwaliteit van leven

- Welke symptomen zorgen voor de meeste ziektelast?
- Wat is de impact van lichen sclerosus op kwaliteit van leven en op seksualiteit?

Follow-up

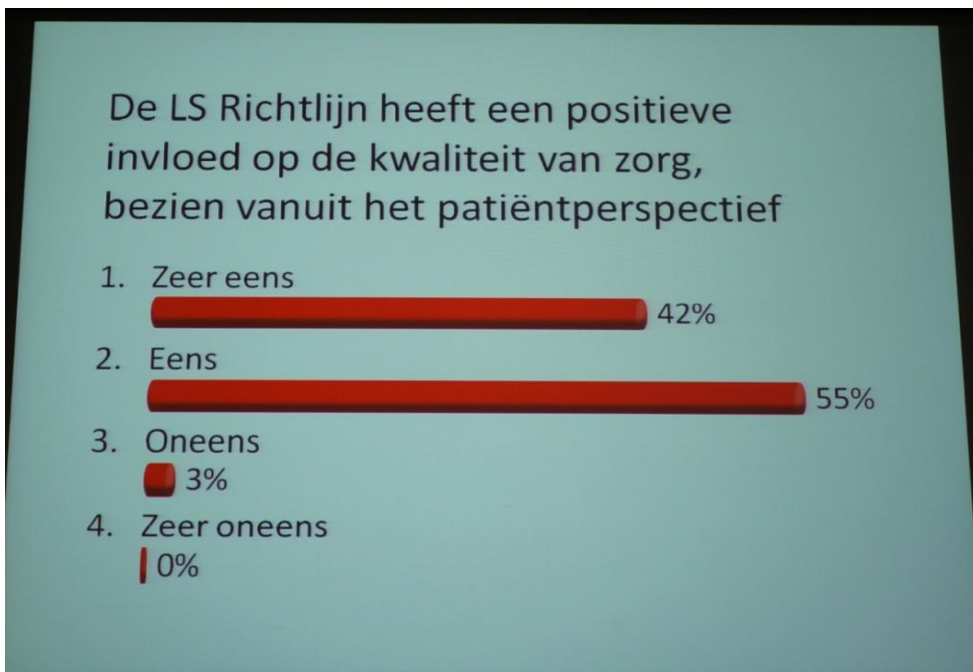
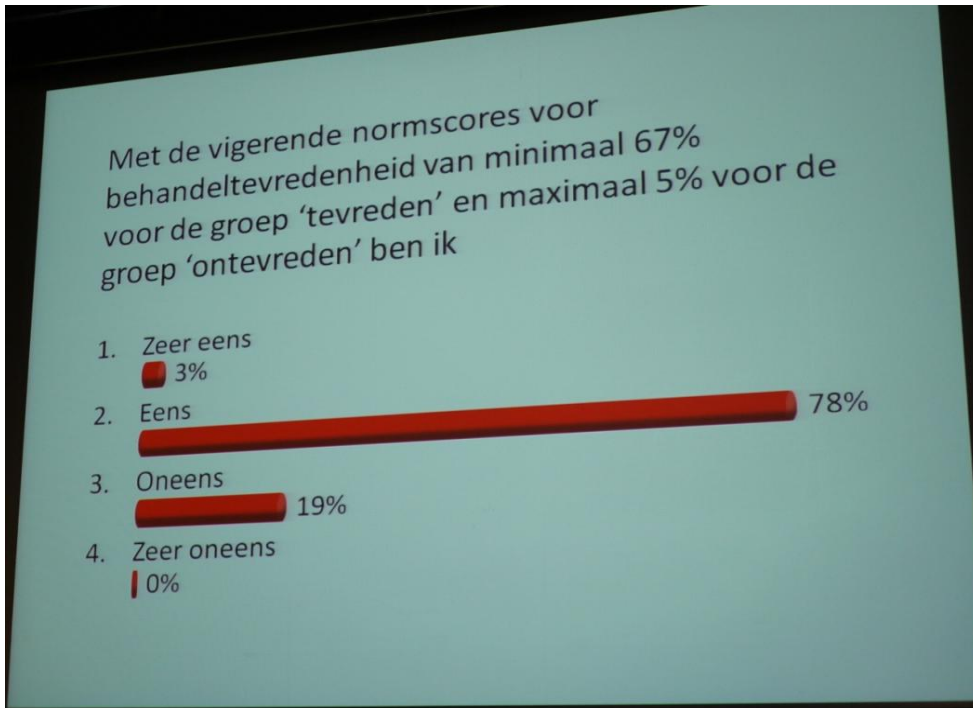
- Hoe vaak is controle nodig of zinvol en door wie?

Consultatie en verwijzing

- Hebben praktijkondersteuners een toegevoegde waarde?
- Is een multidisciplinaire aanpak gewenst?
- Wanneer en naar wie moet een patiënt met lichen sclerosus door de huisarts worden verwezen (dermatoloog, gynaecoloog, uroloog, seksuoloog, kinderarts)?
- Hoeveel tijd dient de specialist beschikbaar te hebben voor een eerste consult en een controle afspraak van een patiënt met lichen sclerosus?

Voorlichting

- Hoe voorkom je ‘*patient*’ en ‘*doctor delay*’?
- Wat kan de richtlijn bijdragen aan goede voorlichting?



¹⁹ Results on my two theses presented among 300 dermatologists. Source: pictures are taken by myself during the annual NVDV society meeting, 30-03-2012.

Appendix 6 Recommendations LS guideline in Dutch

Draft recommendation concerning biopsy

Bij klassieke presentatie van anogenitale lichen sclerosus kan de diagnose op basis van anamnese en lichamelijk onderzoek worden gesteld.

Een pons biopt wordt aanbevolen bij:

1. Diagnostische onzekerheid
2. Verdenking op neoplasie
3. Onvoldoende resultaat van ingestelde therapie

Het biopt moet genomen worden door iemand met klinische deskundigheid. Bij onzekerheid over de locatie van biopt en/of onbekendheid dient patiënt voor biopt te worden doorverwezen.

Indien een potent corticosteroïd wordt gebruikt dan kan het histologisch beeld beïnvloed worden. Het gebruik dient te worden vermeld op het pathologie formulier.

Het klinisch beeld moet leidend zijn in diagnose en behandeling als de uitslag van het histopathologisch uitslag specifiek is.

Final recommendation concerning biopsy

Bij klassieke presentatie van anogenitale lichen sclerosus **volstaan** anamnese en lichamelijk onderzoek voor het stellen van de diagnose en is het nemen van een stansbipt niet noodzakelijk.

Een stansbipt wordt aanbevolen bij:

1. Diagnostische onzekerheid
2. Verdenking op neoplasie

Bij onzekerheid over de meest **optimale** locatie van stansbipt en/of onbekendheid met biopteren **dient** patiënt voor stansbipt te worden doorverwezen naar een **ter zake deskundige**.

Het gebruik van corticosteroïden kan het histologisch beeld beïnvloeden, en **dient daarom** te worden vermeld op het pathologie-formulier.

Als de uitslag van het histologisch onderzoek niet conclusief is, **dan is - na eventueel overleg met de patholoog - het klinisch beeld leidend**. Bij verdenking op neoplasie **dient** het histologisch onderzoek herhaald te worden.

Draft recommendation concerning patient education

Patiënten educatie is **belangrijk**, ook met betrekking tot zelfonderzoek . Hij/zij moet weten waar in het kader van een plaveiselcelcarcinoom op gelet moet worden.

Alle ongewone veranderingen/ontwikkelingen moeten kritisch bekeken worden. Patiënten met toename of verandering van klachten of moeten na telefonische melding op korte termijn gezien worden, gezien het risico op plaveiselcelcarcinoom.

Follow-up wordt geadviseerd door een deskundig specialist bij aanhoudende klachten van patiënt, onzekerheid over dVIN en bij iedereen die al een plaveiselcelcarcinoom of dVIN in de voorgeschiedenis alsmede familiale LS.

Final recommendation concerning patient education

De patiënt **dient geïnformeerd te worden** over zelfonderzoek. Bij toename van de klachten of verandering van de aard ervan **contact opnemen met behandelend arts**.

Draft recommendation concerning the use of crèmes

Het verdient aanbeveling om patiënten tussendoor of in een fase dat er vrijwel geen ziekteactiviteit is de LS-plekken te laten insmeren met een indifferente crème, zoals cremor vaseline FNA, paraffine vaseline in gelijke delen of lanettecrème II FNA.

Een halve vingertipunit is voldoende om de vulva te voorzien.

Final recommendation concerning the use of crèmes

Het gebruik van een emolliens wordt aanbevolen.

Draft recommendation concerning surgery

Excisie van het aangedane epitheel is een optie bij therapie-resistente lichen sclerosus.

Bij ernstige dyspareunie ten gevolge van lichen sclerosus kan de mogelijkheid van chirurgisch handelen worden overwogen.

Bij chirurgisch handelen moet medebehandeling door sekuoloog en bekkenfysiotherapeut worden overwogen.

Final recommendation concerning surgery

Bij ernstige dyspareunie ten gevolge van **vernauwing van de introïtus kan** chirurgie worden overwogen.

Bij ernstige phimosis **kan** chirurgie worden overwogen. **Indien de phimosis niet wordt opgeheven door lokale behandeling met corticosteroiden klasse 3 of 4 en masseren van het preputium is circumcisie aangewezen.**

Bij chirurgisch ingrijpen **dient** een **preoperatief consult** bij een **geregistreerd seksuoloog en/of geregistreerd bekkenfysiotherapeut** te worden overwogen.