

**General discussion**

**9**

## MAIN FINDINGS

This thesis focused on the illness and care experiences of people with neuromuscular diseases (NMDs), their spouses, and health care professionals. Several innovative care interventions were responsively evaluated from these multiple stakeholder perspectives: physical training or cognitive behavioural therapy in the FACTS-2-NMD study and having a case manager in a trial for ALS patients. In this final chapter the main findings will be discussed and a sociological reflection will be given on how these findings can contribute to health care and health care innovation in the field of rehabilitation care for NMDs. This will be followed by a description of the lessons that were learned by executing this qualitative study, including methodological reflections. This chapter will finish with practical implications and recommendations for future research. The thesis consisted of two parts; Part A about the illness experiences, and Part B about the care experiences. The main findings of both these themes are discussed separately.

### **Illness experiences**

Several chapters concerned the illness experiences of people with NMDs (Chapters 2, 3, 4 and 5). The focus of most of these chapters was on the illness experiences of people with FSHD and PPS, since our systematic review showed that, compared to ALS, relatively little attention has been paid to the illness experiences of these two NMDs. Reflecting on the findings of these illness experiences, more general insights were made. First of all, it is notable that the results of Chapters 2, 3, 4 and 5 showed that the NMDs have a great impact on the broad spectrum of people's lives. In previous studies, attention was often paid to specific physical consequences (symptoms) and how people handle these consequences (1). The results of our studies showed, however, that NMDs have a much wider impact; they change identity, self-fulfilment, expectations regarding the future, and body experiences. Our findings showed that not only the physical aspect of the disease, but also these (psycho)social aspects greatly affects people's lives and the lives of their families.

The broader impact of the disease on the lives of people with NMDs can be seen by looking at the disablement process. The disablement process, shown in a disablement model by Verbrugge et al., makes a distinction between different aspects of having an illness or impairment (2). First, there is the pathological part, the biochemical and physiological abnormalities. These abnormalities lead to dysfunctions in the body, the impairments. For example, in ALS the motor neurones are affected (pathology), leading to loss of muscle strength in different parts of the body (impairment). The impairments lead to functional limitations (for example, being unable to climb stairs), and in the last step these activity limitations can result in a disability (for example, being unable to work as a builder). Our findings show that the degree to which a person experiences disabilities

is influenced by intra- and extra-individual factors that affect the functional limitations. Intra-individual factors originate from or operate *within* the person. Extra-individual factors concern aspects introduced from *outside* the person (3). Verbrugge et al. use terminology that closely resembles that of the ICF framework, discussed in Chapter 1. The ICF, for example, uses ‘activity limitations’ and ‘participation restrictions’ compared to ‘functional limitations’ and ‘disability’. Both models focus on the consequences of a disease in the broader context of people’s lives and are therefore in line with each other (4).

The disablement model is shown in Figure 1, showing the steps from pathology towards disability. The intra- and extra-individual factors shown in this figure are a summary of the empirical results of our study, and therefore specific to people with NMDs. In Chapter 3, the model was specified for people with FSHD, but more specific factors were added when also taking PPS and ALS (Chapters 2 and 5) into account. Aspects such as coping with increasing dependency and end-of-life decisions (in ALS) are considered intra-individual factors that relate to a person’s disability (Chapter 2). Adjustments to the physical environment can also be distinguished among the extra-individual factors. One may think of adjusting one’s house in the case of ALS, or the use of special devices. The physical environment and social context can also result in disabilities, for example when people have social contacts that expect too much of them, or when living or working in inaccessible places (Chapter 2 and 4). Finally, different experiences of the body can lead to different care needs (Chapter 5).

What stands out is that health care was, in the context of illness experiences, only indirectly mentioned by the participants as an important theme in their lives (Chapter 2 to 5). This is remarkable, as the model of disablement, and other studies as well, describes care as an important factor influencing the quality of life (2; 5). One of the reasons for the lack of attention to care might be that NMDs are incurable and have no specific treatment. Many respondents (especially with FSHD and PPS) mentioned in their interview that they had not seen a doctor for their condition in a long time, which implies that care is not seen as a very important factor. Another explanation, however, seems more plausible; care was addressed in the interviews in a very specific way, as related to the interventions people followed. The participants in our studies were recruited from the FACTS-2-NMD study, which offered physical training or cognitive behavioural therapy, and from the trial for ALS patients which offered a case manager. By talking extensively about this specific sort of care intervention, it is possible that other important issues related to health care in general were overlooked. As care was indirectly mentioned (for example as regards the process of being diagnosed, in Chapter 2), it is included in Figure 1.

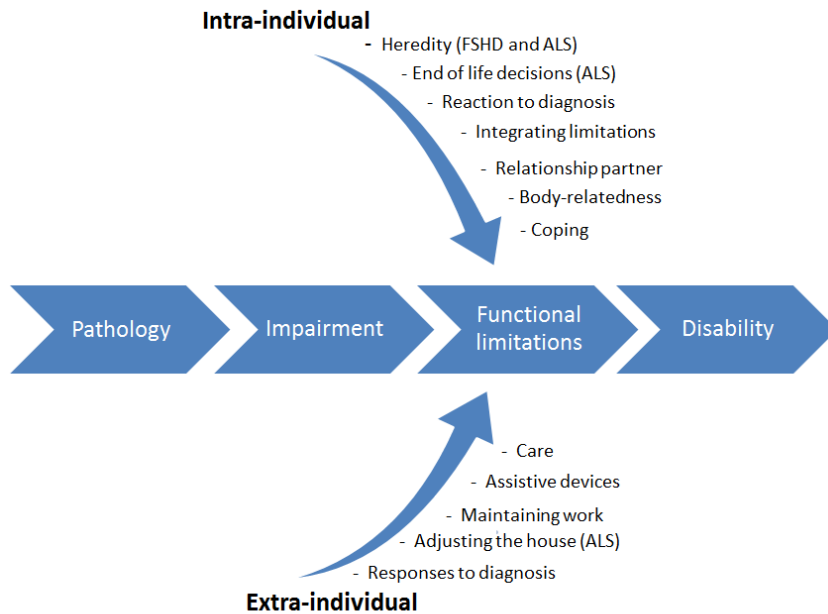


Figure 1: The disablement model for NMDs

Figure 1 gives insight into the disablement process of people with NMDs and which factors play an important role in this process. The strength of this model is that it includes a very broad scope of living with a certain condition, by giving insights into the *interaction* between the intra- and extra-individual factors that influence quality of life and participation (such as the responses to the diagnosis and the coping strategies of patients). The ICF model also takes the individual and the environment into account, and both these models show that quality of life is determined by much more than just the severity of the disease. This is in concert with our findings that show that people worry much more about decreased independence and the use of assistive devices, rather than about the decline of muscle strength itself (Chapters 2 and 3). Our model, which is based on the empirical findings of our studies, stresses the interaction between the intra- and extra-individual factors, which can help health care practitioners to take this interaction into account when treating people with NMDs.

Reflection on how this model can help to innovate health care practice will follow later in this chapter. For now it suffices to stress that it is important to realise that health care practice can only be improved if the model is personalised (rather than used as a 'one size fits all' model) (6), as people may handle the same symptoms in very different ways (Chapter 4), and their experiences are influenced by their personal circumstances and needs (7; 8).

## Care experiences

Some general conclusions can be drawn from the care experiences of people with NMDs. A first conclusion based on the findings of our studies is that people with NMDs are often not only in need of physical therapy, but also of emotional support (Chapters 6 and 7). This finding is well in line with the findings of the illness experiences as described above; patients describe the impact of the disease on an emotional, social and physical level, which implies that all these aspects are important to take into account in the treatment of people with NMDs. In the FACTS-2-NMD study, a programme consisting of three RCTs, the emotional as well as physical aspects were taken into account. In the FACTS-2-NMD study, these were more or less separate elements, as the exercise therapy (ET) focused on the physical element and the cognitive behaviour therapy (CBT) was mainly invested in the cognitive or emotional elements. Some participants and their ET therapists said explicitly that a combination of both therapies would have done more justice to both interventions (Chapter 6). Although the CBT did address physical activity if needed by the participant, the combined effect of both CBT and ET was not studied in the FACTS-2-NMD study, because this would have required a four-arm study design which was not considered feasible. In studying a physical as well as a cognitive intervention, however, the FACTS-2-NMD study was innovative compared to earlier studies that often focused merely on physical or pathological aspects. The ALS case management study was even more in depth in the psychological aspects, as it intended to lighten the burden of the patient and their informal caregiver (spouse), and support the patient in making care-related decisions.

A second conclusion that can be drawn from the studies is that the specific situation and context of the patient influences their individual care needs (Chapter 6 and 7). The need for formal care, for example, was dependent on the progression of the disease, the coping style of the patient and the extent of their social network and associated informal care (Chapter 7). It also turned out to be crucial that patients were able to fit the therapy into their daily routine; if for instance exercise training programmes are too intense, this can result in disappointment and sometimes drop outs (Chapter 6). Both findings advocate for a good balance between care supply and demand. This finding fits with the more general transition towards shared decision making and patient-centred care that has taken place over the last decade. Shared decision making is a process in which practitioners and patients collaborate to access relevant information in order to enable patient-centred health care (9). This can result in patient-centred care, meaning that healthcare providers take patient differences in values, preferences and needs into account, resulting in well-coordinated care (10; 11). This definition fits the findings of our results well, and has some implications for clinical practice. As described by Lusk et al., the identification of need for help is a complex task, as it relies on delicate communication between the patient and health professional (10). However, only by clear communication can the patient clarify their care needs and can the healthcare

professional explain the treatment options (12). Listening to everyday stories of patients can allow insight into the care needs of patients (Chapter 5).

Shared decision making is not only important in the context of clinical reasoning, but also in the context of research (Chapter 8). Without agreement on the wording of questionnaires, these questionnaires can be interpreted as confrontational, painful and even shameful by patients. Making a trial more attuned to the needs and wishes of patients can be achieved by including patient research partners in the research team. By including patients as equal team members, research becomes more patient-centred since fellow sufferers in general have a good insight into the needs, wishes and thoughts of other patients (13; 14).

## SOCIOLOGICAL REFLECTION ON THE MAIN FINDINGS

The research question of this thesis, which consisted of two elements, was *‘what characterises the illness and care experiences of people with neuromuscular diseases and how can these experiences contribute to health care innovation?’* The main findings provide several general insights into the characteristics of the illness and care experiences of people with NMDs. Firstly, these illness experiences affect many aspects of the lives of patients, quality of life is influenced by the interaction between intra- and extra-individual factors, experiences (and care needs) are context-bound, and care innovations require patient participation and dialogue with other stakeholders. We will reflect upon these main findings below, from a sociological perspective.

### **Different models to understanding illness and disability**

In medical practice, the medical model is used to interpret bodily problems. This model originated in the Enlightenment. In this period, medical knowledge started to develop and over the centuries people became more aware of the pathology and physiology of the body. Within Europe (and beyond), there was a so-called ‘secularisation of illness’; the power of knowledge moved from the church towards science and technology (15). This pushed the moral model – in which illness was seen as a punishment of God – further into the background and the medical model became the new dominant model. This model is characterised by the metaphor of the body as a machine; if a part is “broken”, the doctor needs to fix it. This implies a dissociated body-relatedness, where the body is seen as a separate ‘thing’, apart from the mind (mind-body dualism) (16). In this medical model, an illness or disability is seen as an individual problem. A doctor’s primary task is to fix the patient’s body as far as possible. Another characteristic of this model is that its main focus has been on diagnosis and physical aspects; with the

right diagnosis, doctors are better able to fix a problem and every diagnosis has its own characteristics. With the decrease of acute diseases and the increase of chronic diseases, this model has become more problematic, as doctors are more often unable to fully 'fix' the broken body.

Patients with neuromuscular diseases are treated by medical rehabilitation teams, which have a broader focus than just the physical aspects, and work along the lines of the ICF model. The ICF resembles the social more than the medical model, and fits better with our findings than the traditional medical model. In the social model the disability (resulting from an impairment) is no longer a personal feature, but rather a disability that should be considered a relationship between a person and their environment. The ICF-model focuses more on impairments, activities and participation, and less on the personal and contextual/environmental factors (4). The social model, as presented by us, can therefore be seen as complementary to the ICF-model since it emphasises the *interaction* between a person and their environment. From the perspective of this model, sitting in a wheelchair, for example, is not a disability in itself; the fact that not all buildings are accessible for wheelchairs *makes* sitting in a wheelchair a disability. In other words, people are not born disabled, but become disabled in a certain environment. From this perspective, the focus on the diagnosis is less important, as the environment rather than the diagnosis leads to the disability. This means that context plays an important role, which makes it possible to reflect upon the impact of this context and see how it affects a person's experiences. In contrast to the medical model, in the social model the person is seen as a holistic entity, where body and mind both play a pivotal role and where body-mind dualism does not exist (16). As shown in various chapters, too narrow a focus on the physical aspects of the disease may result in overlooking the illness and care experiences of people with NMDs.

Focussing only on the social model would, however, be too unilateral. If we look at the disablement process as shown in Figure 1, a combination of the different models can be recognised; the disability does have a medical basis (pathology), which should not be overlooked, but at the same time, the 'solution' of the disability should not be solely looked for in the medical system. As we have seen in this thesis a lot of patients with NMDs use devices, technology, adjust their houses, and have informal care in order to participate in society. The interaction between intra- and extra-individual factors does better justice to this broader scope of disablement; it is one's personal, medical, social and physical situation that leads to certain disabilities (2). These factors should thus all be taken into account. This 'combined' model, with attention to the personal, medical and social situation, is what we will call the disability studies perspective; it sees disability as a complex biopsychosocial phenomenon (15). It seems that this inclusive approach is a suitable model with which to interpret the main findings of this thesis, and to generate health care innovations.

### **Key elements of the disability studies perspective**

Our findings also indicate that a disability studies (DS) perspective might be useful to gain a deeper understanding of the impact of NMDs on the lives of people. A DS perspective emphasises that a disability is 'life-long' and affects many different aspects of people's lives. The NMDs included in this thesis are not 'lifelong' in the same manner as for people who are born with a disability, but from the moment of onset, these illnesses become 'lifelong'; they are chronic and people will have to live with the consequences of the illness for the rest of their lives once the illness has manifested itself. In addition to the 'lifelong' aspect, the disability studies perspective is also characterised by the fact that it recognises that the illness broadly affects the patient's life; the illness impacts all domains of life, including relationships with partners, family life, work, leisure, school etc. The findings of this thesis show this as well (Chapter 2, 3, 4 and 5).

As described above, within the social model and also from the DS perspective, there is much less focus on the diagnosis than in the medical model. From the DS perspective, the diagnosis (or impairment) does not determine the disabilities that people experience. A 'cross-disability perspective' can be valuable, meaning that patients are not grouped based on their diagnosis, but on shared experiences across patients with different diagnoses. The cross-disability perspective originates from patient initiatives, as specific patient groups often cannot achieve as much as one large patient group. In the Netherlands, we see similar movements, for example, the 'Prinses Beatrix Spierfonds' covers all muscle diseases. The patient organisation 'Vereniging Spierziekten Nederland' (Dutch Association for Muscle Diseases) is an umbrella organisation for people with different types of muscle diseases. One of the main benefits of a cross-disability perspective is that it decreases the risk of stigmatisation (as it does not focus on specific illness characteristics) and increases insights into the common elements of different diseases (15). Cross-disability research shows that with different diagnosis, illness or care experiences (or factors influencing these experiences) are often the same among different patient groups. In this thesis, we used a cross-disability perspective, by including different NMDs in our study. The findings show that for people with ALS, PPS and FSHD, illness experiences are affected by the context and impact the broad spectrum of people's lives (physically, emotionally, mentally and socially). This is not specific to one diagnosis, but is true for all three these groups (and probably also for other NMDs).

Although research and patients can benefit from a cross-disability perspective, it is also important to be cautious with it. Researchers or policy makers can more easily overlook the differences that still exist between different groups of patients (15). In this thesis, we have tried to stay sensitive towards the individual differences among the different diagnosis. For example, the fast progressive character of ALS, often leading to death within a couple of years, affects the illness experiences of these patients



(Chapter 2). This was shown in Figure 1 (where intra- and extra-individual aspects are mentioned specifically for people with ALS). Still, the other themes, although relevant for all diagnoses, can also differ. For example 'coping', as mentioned in Figure 1, means something different for people with PPS (who are known for their 'fighter spirit') than for people with FSHD (who demonstrate different coping strategies). The benefit of a cross-disability perspective (a decrease of stigmatisation) can thus at the same time be a risk of overly broad 'non-information' that does not give anyone detailed information anymore. As a researcher it is always important, as we have tried to do in this thesis, to look for the right balance between both extremes.

A last important characteristic of the disability studies perspective is its emancipatory character. This can be explained by the way in which disability studies originally started, as an activist movement led by people with handicaps. The most famous slogan of the disability studies perspective is '*Nothing about us, without us!*', which reflects this emancipatory character very well. This slogan and the principle of emancipation of people with a disability is no longer just propagated by activists; DS research has incorporated it as well. DS research aims at strengthening and improving the position of patients in society and increasing possibilities for participation, in order to improve the quality of life (and maybe even the quality of existence) of people with disabilities (15).

We strove for patient participation in this thesis, for example by giving patients a voice in our qualitative study, and in the RCTs. We included people with NMDs and their spouses by listening to their illness and care experiences via in-depths interviews, and we consulted them and validated their stories via focus groups. We saw that amongst other things, this led to increased self-esteem and confidence, empowerment, and a sense of ownership in research. The patient stories were also shared with health care professionals to raise their awareness of such patient stories and to adjust their care and treatment. Chapter 8 focused on patient experiences with the trial itself, which enabled the researchers to make adjustments to the questionnaires. Here, the voice of the patients was again explicitly taken into account. Chapter 6 also gives insight into how patients (and also professionals) experienced the specific therapies (17). This is still a rather unique perspective within RCTs. It shows, that listening and understanding 'lived experiences' from the perspective of insiders (versus reducing these experiences to a causal model) is a form of emancipatory research, as it gives patients and their families a voice and a say. We will reflect upon the value of this voice and perspective in the next section.

In this section we have argued that the social model further developed in this thesis is complementary to the ICF model, as it emphasises the interaction between the intra-individual and extra-individual, and that the DS perspective (which builds on this social model) is suitable for gaining a profound understanding of the experiences of

people with NMDs. By including the perspectives of patients, spouses and therapists regarding their experiences with illness and care, valuable new insights emerged. In this sense, patient participation can be seen as a goal in itself (emancipatory) and as a 'tool' to improve rehabilitation and health care practice in general, and to create innovations. In this process, dialogue is essential; perspectives are exchanged and generate a shared construction (18). We have seen the value of a dialogue among stakeholders (e.g. in Chapter 7), where patients, spouses and health care professionals exchanged ideas, perspectives and experiences in a heterogeneous focus group about the multidisciplinary ALS care. Here, health care professionals expressed their gained understanding of the urgency of some of the health care innovations mentioned by the patients.

## LESSONS LEARNED AND METHODOLOGICAL REFLECTIONS

This section will be used to discuss some of the lessons that we learned during the execution of the studies and to critically reflect upon these lessons. As described in Chapter 1, this study emerged from collaboration between the VU University Medical Centre and the Academic Medical Centre Amsterdam. We were approached for both the FACTS-2-NMD study and the ALS case manager trial by the coordinator of the trial and asked (although at a different stage for each study) to execute a qualitative (sub-) study considering the experiences of people with NMDs. For the ALS study this question of a qualitative study emerged from a lack of evidence found in the quantitative study (which surprised the researchers and therapists involved, as patients seemed relatively satisfied with the case manager). For the FACTS-2-NMD programme, we were asked from the start to participate in the three trials as a sub-study. In both cases, this meant that the coordinators of the programmes were open to our contribution to the interventions and acknowledged that a qualitative study could generate valuable additional insights. This implied a certain appreciation for qualitative and participatory approaches and made it possible to collaborate.

The FACTS-2-NMD study started with an introductory meeting to which all sub-study researchers were invited and asked to explain the aims and methods of each specific study. In this meeting we were confronted with the different scientific perspectives (ontologies, epistemologies) which underlie qualitative and quantitative research methods. In qualitative research methods, researchers work from a constructivist approach, meaning that they assume that the reality we perceive is constructed by one's social, historical and individual context. Knowledge is, therefore, intersubjective and context-related. Engagement is seen as necessary to gain an understanding of the

lived experiences from an insider's perspective. Within quantitative research, a more 'positivist' approach is used, meaning that researchers (often) work from the assumption that there is a "reality" outside the perspective of the knower, which they are trying to discover (19). For these researchers, knowledge is, and should be, objective and neutral. Although our collaboration in the FACTS-2-NMD study had already shown that neither the quantitative nor the qualitative researchers were hard-core positivists or constructionists (acknowledging, as we did, the value of both types of methodologies), these underlying theoretical frameworks did raise some practical difficulties.

One of the difficulties was that the qualitative study was not to interfere with the quantitative study, in order to prevent bias in the quantitative data. This meant that, originally, interviews and focus groups could only be held either before the patient started the intervention, or after the last measurement (six months after finishing the therapy). For the qualitative study this would mean that patients were interviewed long after their actual experiences with the training, which would not benefit the quality of the study (as there was a greater chance of recall bias). By talking about the different viewpoints and getting familiar with each other's concerns and objectives, the researchers gained insight into each other's perspective, resulting in a better understanding. This dialogue eventually led to a compromise that was suitable for both the qualitative and quantitative study; the interviews could be held directly after the therapy was finished (which meant that there was no risk of recall bias for the qualitative study and no direct interference during the training period, from which the quality of the quantitative study did benefit). Here, we learned as researchers about the benefits of the dialogue; it helped us to find practical solutions acceptable for all.

The collaboration in both the FACTS-2-NMD and the ALS study matches the increased interest in mixed methods designs. Nowadays it is often argued that mixed methods can solve the problems associated with a unilateral way of researching. The choice of a mixed method design is informed by the value given to multiple approaches and ways of knowing in order to understand the multifaceted and complex character of phenomena. Qualitative data will reveal processes, context and meaning. Quantitative data will measure outcomes and effects. Via a synthesis of this data, its complexity is better understood; qualitative data will provide explanations for the effects found, and how these effects should be valued from the perspectives of participants. In this thesis we saw, despite the practical difficulties mentioned, the possible value of such a mixed methods approach; it did justice to two different types of knowledge gathering and gave a broader insight into the (mal)functioning of interventions. Based on the results of this thesis we would, however, specifically plead for a dialogical collaboration in mixed methods research, as this can (in addition to the previously mentioned benefits) also contribute to a better integration, understanding and collaboration between various research approaches and traditions (19).

Our findings show that it is important to take the context of patients and their diseases explicitly into account in research. There are many ways in which the context can be taken into account, and in this thesis, we used several. First of all, the context was included as a theme in the interviews; relationships with partners and friends were discussed (Chapter 3 and 4). Secondly, the context was part of the research, as partners and health care professionals were also included (Chapter 6 and 7). Thirdly, the context was the specific research subject in Chapter 5, where the body-relatedness of people with PPS was discussed. All these means are useful for gaining insights into illness and care experiences in a context-related manner. The three pathways create a different context-awareness. The first two are more focused on the physical and social context of people, and how this might affect their experiences and care needs. The third way gives insight into a more sociological context and shows us how illness can be experienced in the specific context of our western culture. By studying how patients describe their bodies, we can gain insight into whether patients experience their body within the medical model (mind-body dualism) or within a social model (holistic body experiences) (20). It shows us what is desired as a body, and this, again, gives us insight into how society thinks about health, illness and disability on a social level and how this is reflected on an individual level.

## PRACTICAL IMPLICATIONS

Based on the findings of this thesis, we give some practical implications and recommendations for health care practice and future research.

### **Practical implications for rehabilitation practice**

The findings of this thesis plead for patient-centred rehabilitation care; taking the meaning and context of patients explicitly into account and, if necessary, referring them to, for example, psychologists, occupational therapists or physiotherapists. This is all done in agreement (and dialogue) with the patients and family (10).

Patient-centred care is defined as “respecting people for their knowledge and understanding of their own experience, their own clinical condition, their experience of the illness and how it impacts on their life” (21; 22). This definition clearly shows the importance of the experiences of patients and the meaning they give to their illness. By paying attention to the meaning and experiences, health care professionals can get an even better understanding of the care needs of the patients than asking only about the circumstances in which a patient lives (23). These illness experiences can give a lot of insight into how patients experience their body and how the illness affects their identity (Chapter 5). This is sometimes a difficult task for rehabilitation specialists, as

it can be difficult for patients to explicate these feelings and understandings (9). This would therefore mean that rehabilitation specialists might need training in recognising and articulating illness experiences. Patients sometimes do not know what they can expect or wish for (for example, patients had the idea that fatigue is part of the illness and they should just learn to live with it (Chapter 6). Only after the CBT did they realise that they could at least try to improve their situation). This recommendation implies that rehabilitation specialists should not only listen to what patients explicitly say, but also to the implicit message of illness experiences, as a patient might not mention all issues explicitly.

Taking patient experiences into account in rehabilitation practice means the further implementation of an evidence-based practice. For a long time, evidence-based medicine was seen as the highest attainable goal for health care practice. This meant in the first place that rehabilitation care should be based on the current best evidence about care. Additionally, integrating individual clinical expertise was an important element of evidence-based medicine (24), however, based on the literature and the results of this thesis, we argue that evidence-based medicine, including only these two aspects (scientific research and clinical expertise), is incomplete for rehabilitation care. Based on the results of this thesis we argue that in addition to research evidence and clinical expertise, patient experiences also should be taken into account. The combination of research evidence, clinical expertise and patient values is called 'evidence based practice' in the literature (25). Only by also taking patient experiences into account can one determine whether interventions (such as ET and CBT) are feasible for patients, or how they should be adjusted to become feasible (Chapter 6 and 7). It can give insight into the value of (elements of) certain interventions and at the same time lead to more inclusive rehabilitation care (living up to the idea of *'nothing about us, without us'*). Together with scientific research and clinical expertise, this could help to improve rehabilitation care.

### **Recommendations for future research**

The first implication for future research arises from the practical implications for health care practice, where we plead for more attentiveness towards the specific care values for people with NMDs. For people with ALS, these values have to do with attentiveness (having time for the patient, making house calls, providing pro-active care and emotional support) (Chapter 7). To our knowledge, the care values of people with FSHD and PPS have, to date, not been studied. It can, however, be assumed that the care values of these patient groups differ from the values of ALS, due to the different characteristics of the diseases (with regard to age of onset, progressiveness, affected muscles etc.). This means that basic insights into the care values of people with PPS and FSHD are still missing, when these insights could potentially be of great value to rehabilitation

specialists. We therefore recommend study of the care values and elements that might influence the values of people with PPS and FSHD.

A second implication for future research concerns a methodological issue; this study was one of the first mixed method RCTs, and showed us that combining two different methodologies can involve some (practical) difficulties. In order to resolve these problems, future research should look into ways in which both qualitative and quantitative research approaches can be fruitfully combined without compromising the quality and integrity of either one of the methods and underlying approaches. In both the FACTS-2-NMD study and the ALS study, the responsive evaluation was executed by a different department than the quantitative study. Dialogues were initiated at certain points during both studies, and found to be helpful to heighten mutual understanding. Based on this finding we would recommend intensifying this contact between different departments/scholars in a mixed methods design, and regularly scheduling time for conversation from the very beginning (in the study design). Another way to achieve greater integration and dialogue is by literally avoiding distance between the two different approaches, for example by executing both the qualitative and quantitative study within the same department.

The final recommendation for future research concerns the further implementation of all our study results. Although our results have been published in international peer reviewed journals, the practical implementations of our results are not ensured, per se, by those publications. This study was, of course, not an implementation study, and therefore this part of the research did not have priority. A small implementation study was conducted after the trial but the further implementation of the results of our study are important, as it would do justice towards the effort taken by the patients and professionals in contributing to this study. This includes the initiative for an implementation study building on the results of the FACTS-2-PPS trial. Recently, a systematic literature review on the dissemination and implementation strategies showed that a combination of different implementation strategies should be taken into account right from the very beginning of a study, when the study design is developed (26). This requires a small extension of the budget and time, but can potentially enhance the social impact of research.

## REFERENCES

1. LaDonna K. A literature review of studies using qualitative research to explore chronic neuromuscular disease. *J Neurosci Nursing* 2011;43:172-83.
2. Verbrugge L, Jette A. The disablement process. *Soc Scie Med* 1994;38:1-14.
3. Stryker S, Burke P. The past, present and future of an identity theory. *Soc Psychol Q* 2000;63:284-97.
4. World Health Organization. International classification of functioning, disability and health. 2001. Geneva.
5. Nierse C, Abma T, Horemans A, van Engelen B. Research priorities of patients with neuromuscular disease. *Disabil Rehabil* 2013;35:405-12.
6. Gergen K, Gergen M. Narrative form and the construction of psychological science. In: Sarbin T, editor. *Narrative psychology: the storied nature of human conduct*. Westport, CT: Praeger Publishers; 1986. p 22-44.
7. Benner P. Quality of life: a phenomenological perspective on explanation, prediction, and understanding in nursing science. *Adv Nurs Scie* 1985;8:1-14.
8. Carr A, Gibson B, Robinson P. Is quality of life determined by expectations or experience? *BMJ* 2001;322:1240.
9. Adams J, Drake R. Shared decision-making and evidence-based practice. *Community Mental Health Journal* 2006;42:87-105.
10. Lusk J, Fater K. A concept analysis of patient-centred care. *Nursing Forum* 2013;48:89-98.
11. Greiner A, Knebel E. *Health professions education: a bridge to quality*. 2003. Washington (DC), National Academies Press.
12. Pieterse A, Cup E, Akkermans R, Hendricks H, van Engelen B, van der Wilt G, Oostendorp R. Optimizing referral of patients with neuromuscular disorders to allied health care. *European Journal of Neurology* 2009;16:562-8.
13. Schipper K. 2011. Patient participation & knowledge [PhD dissertation]. Amsterdam: VU University Press.
14. Teunissen T. 2013. Values and criteria of people with a chronic illness or disability: strengthening the voice of their representatives in the health debate and the decision making process [dissertation]. Ede: Print Service Ede.
15. Hoppe S, Schippers A, Kool J. 2011. *Disability Studies in Nederland: verbinding in de context*.
16. Nettleton S. 2013. *The sociology of health and illness*. 3rd edition. Cambridge: Polity Press.
17. Abma T. Patients as partners in health research agenda setting: the feasibility of participatory methodology. *Evaluation & the Health Professions* 2006;29:424-39.
18. Abma T, Broerse J. Patient participation as dialogue: setting research agendas. *Health Ex* 2010;13:160-73.
19. Johnson R, Onwuegbuzie A. Mixed methods research: a research paradigm whose time has come. *Educational Research* 2004;33:14-26.
20. Frank A. 2013. *The wounded storyteller: body, illness, and ethics*. 2nd edition. Chicago: The University of Chicago Press.
21. Dahlberg K, Todres L, Galvin K. Lifeworld-led healthcare is more than patient-led care: and existential view of well-being. *Medical Journal of Health Care and Philosophy* 2009;12:265-71.
22. Department of Health. 2005. *Creating a patient-led NHS: delivering the NHS improvement plan*. London: Department of Health.
23. Charmaz K. Loss of self: a fundamental form of suffering in the chronically ill. *Soc Health Illness* 1983;5:168-95.
24. Sackett D, Rosenberg W, Gray J, Haynes R, Richardson W. Evidence based medicine: what it is and what it isn't. *BMJ* 1996;312:71-2.
25. McKibbin K. Evidence-based practice. *Bulletin of the Medical Library Association* 1998;86:396.
26. Schipper K, Bakker M, de Wit M, Ket H, Abma T. Strategies for disseminating recommendations or guidelines to patients: a systematic review. *Implementation Science* 2016 [under review].