Consequences of care: Parents of children with a chronic disease
Hatzmann, J.

Citation for published version (APA):

General rights
It is not permitted to download or to forward/distribute the text or part of it without the consent of the author(s) and/or copyright holder(s), other than for strictly personal, individual use, unless the work is under an open content license (like Creative Commons).

Disclaimer/Complaints regulations
If you believe that digital publication of certain material infringes any of your rights or (privacy) interests, please let the Library know, stating your reasons. In case of a legitimate complaint, the Library will make the material inaccessible and/or remove it from the website. Please Ask the Library: http://uba.uva.nl/en/contact, or a letter to: Library of the University of Amsterdam, Secretariat, Singel 425, 1012 WP Amsterdam, The Netherlands. You will be contacted as soon as possible.

UvA-DARE is a service provided by the library of the University of Amsterdam (http://dare.uva.nl)
1

General Introduction
1 INTRODUCTION

The number of children growing up with a chronic illness has increased enormously in the past decades. This increase is due to a combination of decreased childhood mortality, at the cost of an increase in childhood morbidity. The decrease in childhood mortality was achieved by major contributions from modern pediatric diagnostic and therapeutic modalities including the development of pediatric surgical interventions, pediatric cardiac surgery and pediatric anaesthesiology. These improvements have led to higher survival rates for many diseases that can now be considered chronic diseases, for instance for cancer, metabolic diseases, congenital heart disease and cystic fibrosis. However, the majority of these children have been treated but have not been cured, which has led to an increase of chronic disease and late effects of treatment in childhood. In addition to this, the prevalence of some diseases like obesity, asthma, and attention deficit hyperactivity disorder has increased as well in the last decades. As a consequence, chronic illness nowadays accounts for the majority of hospital admissions in children. The number of chronically ill children approximates 7 to 20 percent of all children (CBS 2008), that is about 500,000 children in the Netherlands and approximately 7 million in the United States. This number is expected to increase further due to ongoing medical advancements and genetic, social, and behavioral changes.

1.1 Children growing up with a chronic disease

Due to the improved life expectancy and the increase in number of chronically ill children more children with extra health care needs grow up into adulthood. These children experience several consequences of their health care needs. Most of them depend on medical care to some extent, varying from medication use to medical interventions (e.g., daily inhalation of corticosteroids, intravenous alimentation). Other consequences for children are frequent hospitalizations, undergoing painful medical procedures, school absenteeism and restriction of activities due to the medical regimen. More emotional and behavioural problems are observed in this population. Children are at risk for several short- and long-term academic, behavioral and negative emotional outcomes (e.g. a lower self-esteem and social adjustment). Also, chronically ill children have to meet their age-related developmental tasks in addition to managing the disease, resulting in a higher risk for a delay in reaching developmental milestones.

Parents are extremely important for the development of their children. They play a crucial role in the social and autonomy development of their children. When parents establish a supportive, caring and positive environment, this will have a positive effect on developmental outcomes for children, also in a population of children growing up with a disease. However, for parents of a child with a chronic disease, organizing an ideal environment for their children possibly will be more challenging due to different additional stressors they may face. Recent studies have shown that higher levels of parenting stress
consistently predict poorer behavioural, emotional and social adjustment of children with cancer.\textsuperscript{16} Also, parental physical, emotional, and social health influences the health and well-being of their children.\textsuperscript{17-20} Higher levels of parental stress were related to lower psychosocial well-being in their children\textsuperscript{20}, and more externalizing problems.\textsuperscript{21} In addition to the consequences of stress, parents may perceive their children more vulnerable which sometimes results in overprotection. Overprotection and lowered expectations of a child with an illness or disability can cause lowered self esteem for the child, resulting in underachievement and failure to reach their full potential.\textsuperscript{22} In short, parental functioning is one of the factors essential for the development and well-being of children, and therefore an important topic to address in pediatric research and practice.

1.2 Parents of chronically ill children

Pediatric illness affects the entire family. Although the psychosocial outcomes of children with an illness have been studied extensively, fewer studies have addressed the consequences for parents. Confrontation with the knowledge that their child has a chronic and potentially life-threatening disease is a stressful and potentially traumatic event for parents.\textsuperscript{18,23} The time of diagnosis is accompanied by several emotions including shock, disbelief, denial and anger.\textsuperscript{24} A diagnosis also ends a period of uncertainty about the kind of illness or disorder the child has, but it may result in other uncertainties about how to care for the child. After the initial shock of the diagnosis, the impact of the disease on the child and the family may gradually become clearer. When children do, or do not reach developmental milestones or transitions, e.g., starting to walk, going to (special) education, social interaction with peers, parents have to adjust their expectations.\textsuperscript{24} And parents continually have to adapt to either positive or negative consequences of their child’s disease.

Besides emotional impact, having a chronically ill child also influences family and social life as parents provide most of the daily care for their children\textsuperscript{25}. This daily care involves management of the illness, which can be complex and is increasingly transferred from the hospital to the home (e.g., home dialysis, intravenous alimentation). Parents spend on average 3-5 hrs a day on medical care management.\textsuperscript{26,27} Raina and others\textsuperscript{28} describe caring for a child with long-term disabilities as an additional role for parents, which may pose a burden on other roles. Caring for a chronically ill child not only involves management of the illness, it also includes instructing others (teacher, baby-sitter) about the care.\textsuperscript{29} Further care giving tasks comprise finding and coordinating appropriate care for their child, incorporating the care into family life, managing the consequences on siblings, and keeping the responsibilities of caring in balance with personal needs.\textsuperscript{30,31}

Changes in family demographics may also have influenced the consequences of having a chronically ill child for parents. In the past decades families have become smaller, there are more single-parent families and mothers more often are employed.\textsuperscript{32} Thus, changes in pediatrics as well as demographic changes may have altered care giving demands, and thus the impact of chronic illness on the families.
2 CONSEQUENCES OF CARE FOR PARENTS

The consequences of chronic illness for parents were first conceptualized by Hoenig & Hamilton in 1966, as ‘caregiver burden’. It was defined as “any cost to the family of which the patient is a member”, and both subjective and objective elements of caregiver burden were recognized. They described the subjective burden as ‘feelings of arousal in caregivers when fulfilling their care giving tasks’. Objective burden was described as ‘events and activities associated with negative care giving experiences’. In later studies, caregiver burden was seen as a dynamic concept, resulting from an imbalance between perceived demands and resources, affecting physical, financial, psychological, and social domains of life of the caregiver. The concept of burden has been critically evaluated as it focuses on the negative consequences, whereas caring for a family member is often not only experienced as a burden. Besides the negative consequences, care giving may also positively influence the lives of caregivers. There is a growing body of evidence describing benefits and positive consequences reported by parents of children with chronic disease. Reported benefits are increased confidence, increased awareness of what is important, and stronger family relations. Broadly applied terms to describe consequences of care that not only focus on negative aspects of the care giving role, are for instance well-being and quality of life.

In addition to the emotional consequences experienced by parents, the care giving demands may also have consequences for their social functioning and participation in society. Unpredictability of the disease, recurrent hospital admissions, and extra daily care giving time are only a few reasons possibly leading to conflicting roles, for example...
between being a parent and an employee. These role conflicts may limit parents in being an employee, member of a sports club, being a friend and may have consequences for their psychological well-being. Therefore, the distinction between subjective and objective burden may not be so explicit. Nowadays, studies mainly focus on one of both outcomes, while the relation between them justifies an integration of objective and subjective consequences within studies.

In the present thesis consequences for parents are described in terms of Health Related Quality of Life (HRQoL) (§2.1), employment and leisure activity time (§2.2), and the subjective costs of care as a monetary evaluation of subjective wellbeing (§2.3). Furthermore, the association of disease related characteristics, demographic characteristics, and social functioning with these outcomes was studied. (§3) A conceptual model was developed to explore the association of demographic, disease related with parental HRQoL, mediated by employment, income, leisure time, holiday and emotional support (§4).

2.1 Parental Health Related Quality of Life

Being a parent of a child with a chronic disease may have several emotional, physical and social consequences. Parents have to perform additional tasks compared to parents of healthy children and they may also have additional concerns about their child’s health and life. Within pediatrics, the increased survival and its consequences for children, have led to awareness of the importance of the children’s quality of life (QoL). Quality of life is also increasingly recognized as an outcome measure in pediatric treatment evaluation. The World Health Organization (WHO) defines QoL as: “individuals’ perceptions of their position in live in the context of the culture and value systems in which they live, and in relation to their goals, expectations, standards and concerns.” For parents, the effect of the child’s illness on their quality of life is not (yet) considered an important outcome. Although research on parental functioning is expanding, up to now it mainly focuses on parental depression, distress and physical and psychological health. Quality of life of parents is not often measured. Parents of children with a chronic disease report elevated stress levels and more physical and psychological problems than parents of healthy children. More information regarding how parents evaluate their physical, social and emotional functioning is needed. In addition to the general concept QoL, the multidimensional concept of HRQoL has been introduced in pediatric and in adult disease populations, to focus on the influence of health on an individual’s QoL. This concept might not seem applicable to parents, however, their health is compromised due to care giving demands. The WHO definition of health is ‘a state of complete physical, mental and social well-being, and not merely the absence of disease or infirmity’. This broad definition of health makes HRQoL a good concept to evaluate physical, emotional and social functioning not only within disease populations, but also in caregiver populations.
2.2 Employment and leisure activity time

In the Netherlands, the most common division of paid work among couples with children is the one-and-a-half earner model: the father working fulltime and the mother part-time.\(^53\) Furthermore, 10% of Dutch women withdraw from the labour market after the birth of their first child.\(^54\) As parents of chronically ill children spend on average an additional 3-5 hours a day on care giving compared to parents of healthy children\(^26,27\), the time they have left for other areas of life is limited. These areas are, among others, employment, education, household tasks, and leisure activities (e.g. sports, socializing, and other pastime). A number of studies indeed demonstrate that parents of chronically ill children are less often employed and spend less time on personal care and doing leisure activities.\(^55-57\)

These changes may lead to financial problems\(^58,59\), different career perspectives and mobility, and psychological distress.\(^44\) Leisure activities of parents of chronically ill children have not been studied extensively. Available studies show that parents do not have enough time for their leisure pursuit\(^56,60\), and half of all parents have adjusted their hobby\(^60\) and their way of going on a holiday.\(^61\) These are essential issues for parents’ social, financial and individual well-being. Both employment and leisure activities may provide distraction from the care giving tasks and care related stress. Thus, leisure activities and work could contribute to better parental functioning.\(^62\) Problems related to employment or work conflict due to the illness of the child was associated with parental stress in samples of parents of children with cancer\(^63,64\) and congenital heart disease.\(^47\)

Most studies addressing employment have been done among samples of parents of children with a chronic disease only (children with special health care needs, with disabilities, hiv, diabetes)\(^58,60,65,66\), while only a few studies made a comparison with parents of healthy children.\(^25,56,67\) Also, the majority of these studies have been conducted in the United States. Studies can only be extrapolated between countries to a certain extent, as different regulation concerning insurance and leave arrangements probably influences parental ability to stay at home or be employed. In the Netherlands, several leave arrangements exist for employees to facilitate the combination of family care and employment. Despite the relatively good position of employees in our country, little is known about employment and leisure activities among Dutch parents of children with a chronic illness.
2.3 Subjective costs of care
Parents of chronically ill children are known with higher stress levels and worse physical and psychological health outcomes than parents of healthy children. It is not easy to quantify these differences, even though this would be helpful in estimating the impact of chronic childhood illness on parents. Within econometrics, an approach has been developed to quantify differences in well-being scores in terms of household income. This method is known as the well-being valuation method and it was first applied in the health literature by Ferrer-i-Carbonell and Van Praag.\(^68\) It has proven its contribution to the valuation of aircraft noise around Schiphol airport\(^69\) and has been used to estimate the costs of informal care in a population of informal caregivers.\(^70\) In this latter study, a comparison was made between the well-being valuation method and the contingent valuation method (which directly asks how much people would be willing to pay for informal care), showing that the well-being valuation method was a useful complement to more traditional valuation methods. However, this method has not been applied in a pediatric population yet. Thus, although the emotional costs for parents have been addressed in several studies\(^67,71,72\) and are widely acknowledged, the equivalent monetary value has not been estimated. Translating the subjective impact of care in terms of euro (€) may add to the understanding of this impact as people are inclined to think in monetary values.

3 PREDICTORS OF PARENTAL HRQOL, EMPLOYMENT AND LEISURE ACTIVITY TIME

Parents of children with a chronic disease generally experience a multitude of negative consequences that influence their own lives and the lives of their children. Decreased parental well-being may hamper their ability to support, stimulate and care for their children. However, there are also parents who seem to cope well with the demands of their child’s illness. Identification of parents at risk for maladjustment is very important, for the parents, their children and health care professionals.

3.1 Demographic variables
The influence of demographic variables on health, HRQoL, employment and leisure activity time is well-known and often accounted for in research.\(^73-75\) Mothers of chronically ill children generally report lower a quality of life\(^41\), and more mental health problems than fathers.\(^76\) In addition, mothers focus on the care for their children and reduce working hours, while fathers continue working and are financial providers\(^76\), a division that is also seen among parents of healthy children in the Netherlands. Higher socio-economic status is a protective factor, and may help finding resources to manage the consequences. A higher educational level, higher household income and belonging to a non-minority positively influences parental psychosocial functioning and employment.\(^77\) Although these effects of demographic variables may not be specific for parents of children with a chronic
disease, demographics are generally of great importance and should be taken into account when studying parents as caregivers.

3.2 Disease related variables
First, the influence of the severity of the disease on parental functioning appeals to common sense. Disease severity nowadays is often measured following a generic approach including the limitations children experience in daily life. A growing body of evidence suggests that severity of the functional limitation of the children is associated with parental well-being and employment. A review by Rentinck et al demonstrated that in a number of studies the level of independent functioning of the child predicted maternal depression. Also, functional ability influences employment; parents reduce working hours when care giving demands are higher. Besides functional limitation, the nature of the disease may also influence parental functioning. Life threatening diseases with complex regimens of care will have a different effect on parents than diseases that are more easily managed. A third disease related factor known to influence parental functioning is time since diagnosis. Longer time since diagnosis is associated with better parental functioning in cancer survivors, and children with congenital heart disease, suggesting an adaptation effect in these populations. However, a progressive illness is expected to inversely influence the effect of time since diagnosis.

Most research has been performed within disease groups, describing specific disease related consequences. The association between functional limitation, time since diagnosis and parental well-being suggests the value of generic disease related predictors across diseases.

3.3 Social variables
The social environment can be protective for the consequences parents of children with a chronic disease experience. An important aspect of dealing with difficulties is the availability of emotional support. Social resources can contribute to the interpretation of the meaning of stressful events and can thus indirectly affect parental well-being by alleviating the consequences of stressful life events. Research shows the protective value of a support network for parental emotional well-being. Having a social network and especially receiving social support decreases stress levels. In addition, a support network may also contribute to the possibility for parents to spend time doing work and leisure activities. This, however, has not been subject of much research.

The influence of social variables as employment, leisure time and going on holiday on parental quality of life has not been thoroughly studied. Evidence shows that the employment situation and leisure time of parents often changes when facing the chronic illness of a child. A small number of studies have addressed the influences of these changes on parental well-being and show an association between employment and parental stress levels and mental health. The relation of changes in work and leisure activities with parental well-being should be addressed in more detail as it may provide a starting point for supportive interventions and guidelines.
3.4 Conclusion

Research findings show considerable consequences for parents of children with a chronic disease. However, data on HRQoL of parents is rare. The majority of studies either focus on subjective or objective consequences of care, while this distinction may not be so evident. Many studies up to now are limited due to small sample sizes, lack of a good definition of chronic disease, not using a comparison group and many studies are done within disease groups, making comparison between studies and diseases rather difficult. Also, most studies only explored direct predictors of parental functioning, while the complexity of the topic asks for a more complete perspective, also including indirect pathways between independent and dependent variables. Additional information including a more comprehensive perspective on parental functioning will provide important information about both objective and subjective consequences across disease groups, allowing us to develop supportive interventions with benefits for parents as well as for their chronically ill children.

4 AIM, DESIGN AND OUTLINE OF THE THESIS

This thesis reports the results of the Care-project, which investigates the HRQoL, employment, and leisure activity time of parents of chronically ill children. The Care-project, which was partly financed by the Dutch Ministry of Social Affairs and Employment, was conducted between 2005 and 2009 by the Psychosocial Department and the Department of Pediatrics of the Emma Children’s Hospital/AMC in close cooperation with the Faculty of Economics of the University of Amsterdam.

4.1 Research model

In the present study, a conceptual research model is used, describing the dynamics of parental functioning. Parental functioning is examined in terms of parental HRQoL, employment, leisure activity time, and a monetary equivalent of subjective well-being. The main outcome of the model is parental HRQoL. We have chosen for this concept because caring for a chronically ill child is related to changes in emotional, physical and social functioning, and these factors are all incorporated in the concept of HRQoL. In line with the models of Raina and King, we suppose that HRQoL of parents of chronically ill children is influenced by demographic, medical, and social factors, and that these factors are both directly and indirectly related to HRQoL. Parents of chronically ill children are known with lower employment rates and reduced leisure time activities compared to parents of healthy children. This is important information as employment is related to parental well-being. Therefore we constructed a model in which we describe the association of demographic and medical variables on parental HRQoL, mediated by employment, leisure time, income, holiday and emotional support. Within the Care-project, besides testing the model, HRQoL, employment and leisure activity time are also explored as outcome variables. For estimation of the subjective costs of care, we used a separate model. The effect of having a chronically ill child on sub-
Objective well-being was equated to the effect of household income on subjective well-being, both corrected for disease related and socio-demographic variables.

4.2 Aim of the study and research questions
The number of chronically ill children reaching adulthood has increased enormously the past decades. Furthermore, technological advancements have made medical treatment at home possible and more common. As a result, parents provide care for long time periods. The intensity and duration of this care may interfere with other areas in life and may have consequences for their health and emotional functioning.

The aim of our study was to explore:
1 Health Related Quality of Life,
2 employment and leisure time and
3 a monetary evaluation of well-being of parents caring for a chronically ill child in comparison with parents of children who are not ill, and
4 to explore associated factors with HRQoL, employment and leisure activity time.

This aim resulted in the following research questions:
1 How is the HRQoL of parents of chronically ill children compared to parents of healthy schoolchildren?
2 Which characteristics (socio-demographic, disease related, and social variables) are associated with the HRQoL of parents of chronically ill children?
3 How does chronic illness of a child relate to parental well-being in comparison
with parents of healthy children and what are the costs of differences in well-being expressed in a monetary value?

4 What is the relationship of being a parent of a child with a chronic disease with parental employment and leisure activity time and which factors (disease related and social) are associated with these outcomes in parents of children with a chronic disease?

4.3 Sample & measures
This explorative, cross-sectional, retrospective study was conducted among 580 parents of chronically ill children and 443 parents of healthy schoolchildren.

The definition of chronic disease includes the following criteria:
1 the disease occurs in children aged 0-18 years,
2 the diagnosis is based on medical scientific knowledge,
3 the disease is not (yet) curable and
4 exists for at least three months, or will probably endure longer, or at least three disease episodes have occurred the last year.

Parents of children from 10 different chronic diseases were selected: asthma, diabetes, Down syndrome, Duchenne muscular dystrophy, end stage renal disease, metabolic diseases, profound multiple handicaps, sickle cell disease, spina bifida, and survivors of brain tumors. They were either patients of the Emma Children’s Hospital or members of a patient organization (Duchenne, metabolic diseases). All children met the criteria of chronic childhood illness.

Inclusion criteria in all disease groups were:
1 the chronically ill children were aged between 1-19 years old,
2 were diagnosed at least 1 year before start of the study,
3 the children lived at home and
4 parents were able to fill out the questionnaire in either Dutch or English.

Specific criteria for each diagnosis are described in Appendix A. Parents of healthy schoolchildren formed the comparison group, they were eligible if:
1 their child was not chronically ill,
2 was aged between 1-19 years old and
3 living at home. Also, parents had to be able to fill out the questionnaire in either Dutch or English.

A self-report questionnaire was developed for the Care-project. The questionnaire includes several questions adapted from other studies, a validated questionnaire about HRQoL;
### Table 1 Description of the 10 selected chronic diseases in the Care-project

<table>
<thead>
<tr>
<th>DIAGNOSIS</th>
<th>N; RESPONSE RATE (%)</th>
<th>DESCRIPTION</th>
</tr>
</thead>
<tbody>
<tr>
<td>Asthma</td>
<td>n= 97 (48%)</td>
<td><strong>Asthma</strong> is a chronic and complex lung disease, characterized by acute symptomatic episodes of varying airflow limitation. Children with asthma have respiratory problems (coughing, wheezing and dyspnoea) during both day and night.</td>
</tr>
<tr>
<td>Brain tumor</td>
<td>n= 43 (86%)</td>
<td><strong>A brain tumor</strong> is an abnormal growth of cells within the brain or inside the skull, which can be cancerous or non-cancerous (benign). Depending on the tumor location and the damage it may have caused to surrounding brain structures, any type of neurological symptoms may occur, such as cognitive and behavioral impairment, personality changes, hemiparesis, hypoesthesia, aphasia, ataxia, visual field impairment, facial paralysis, double vision, tremor. Treatment includes most often surgical attempt to remove the tumor and chemo- and/or radiotherapy.</td>
</tr>
<tr>
<td>Diabetes Mellitus</td>
<td>n= 24 (43%)</td>
<td><strong>Diabetes mellitus</strong> is a condition where the amount of glucose in the blood is too high because the body cannot use it properly. Most children have type 1 diabetes, which develops when the insulin-producing cells in the pancreas have been destroyed. Diabetes is treated by insulin injections and a healthy diet, and regular exercise is recommended.</td>
</tr>
<tr>
<td>Down syndrome</td>
<td>n= 103 (58%)</td>
<td><strong>Down syndrome</strong>, or trisomy 21 is a chromosomal disorder caused by the presence of all or part of an extra 21st chromosome. Individuals with Down syndrome tend to have a lower than average cognitive ability, often ranging from mild to moderate developmental disabilities. A small number have severe to profound mental disability and almost half have a heart condition.</td>
</tr>
<tr>
<td>Duchenne muscular dystrophy</td>
<td>n= 61 (49%)</td>
<td><strong>Duchenne muscular dystrophy</strong> is an inherited disorder that involves rapidly-worsening muscle weakness. Duchenne results in progressive loss of strength and is caused by a mutation in the gene that encodes for dystrophin. Because dystrophin is absent, the muscle cells are easily damaged. The progressive muscle weakness leads to serious medical problems, particularly issues relating to the heart and lungs. Young men with Duchenne typically live into their late twenties.</td>
</tr>
<tr>
<td>End Stage Renal Disease</td>
<td>n= 27 (71%)</td>
<td><strong>End-stage renal disease</strong> (ESRD) is irreversible decline in kidney function, severe enough to be fatal in the absence of dialysis or transplantation (i.e. it refers to the time when renal replacement is actually needed).</td>
</tr>
</tbody>
</table>
the TAAQoL[^1], a well-being questionnaire[^2], questions adapted form other questionnaires, and questions developed by the researchers (see Appendix B for an overview of the questions used). The clarity and applicability of the total questionnaire was pre-tested with 15 parents of chronically ill children who met the inclusion criteria. Based on their suggestions, modifications were made to improve the survey’s content and clarity. The final questionnaire consisted of four parts (Table 2). Parents of chronically ill children completed all four parts, and parents of healthy children completed three out of four parts (no disease related questions).

<table>
<thead>
<tr>
<th>DIAGNOSIS</th>
<th>N; RESPONSE RATE (%)</th>
<th>DESCRIPTION</th>
</tr>
</thead>
<tbody>
<tr>
<td>Metabolic diseases</td>
<td>n=121 (52%)</td>
<td><strong>Inborn errors of metabolism</strong> comprise a large class of genetic diseases involving disorders of metabolism. In most of the disorders, problems arise due to accumulation of substances which are toxic or interfere with normal function, or to the effects of reduced ability to synthesize essential compounds. Many of the inborn errors of metabolism, including urea cycle defects, organic acidemias, and certain disorders of amino acid metabolism, present in the young infant with symptoms of an acute or chronic metabolic encephalopathy.</td>
</tr>
<tr>
<td>Profound multiple disabilities</td>
<td>n=21 (38%)</td>
<td>Children with <strong>profound multiple disabilities</strong> have profound mental retardation and possess profound physical impairments. Children have signs of very serious neuromuscular dysfunction such as severe spasticity, muscle rigidity, and skeletal deformities as well as sensory impairments, have little or no control over their movements, and appear to have minimal or no physical potential to allow for independence in performing self-care routines.</td>
</tr>
<tr>
<td>Sickle cell disease</td>
<td>n=62 (63%)</td>
<td><strong>Sickle cell disease</strong> is an inherited disease, characterized by chronic anemia and recurrent painful vaso-occlusive crises, which occur unpredictably and require immediate management. Many patients eventually develop organ damage</td>
</tr>
<tr>
<td>Spina Bifida</td>
<td>n=21 (37%)</td>
<td><strong>Spina bifida</strong> is a neuroembryological disorder with complex physical and neuropsychological morbidity. The majority of children with myelomeningocele (SBM), the severe form of SB, develop hydrocephalus and associated cerebral malformations of the posterior cortex and white matter, midbrain, cerebellum, and corpus callosum. Although children with SBM tend to score within the low average to average range of general intelligence measures, they are at risk of specific cognitive difficulties, learning disabilities and motor deficits.</td>
</tr>
</tbody>
</table>

[^1]: TAAQoL
[^2]: well-being questionnaire

---

<table>
<thead>
<tr>
<th>N; RESPONSE RATE (%)</th>
<th>DESCRIPTION</th>
</tr>
</thead>
<tbody>
<tr>
<td>n=121 (52%)</td>
<td>Inborn errors of metabolism comprise a large class of genetic diseases involving disorders of metabolism. In most of the disorders, problems arise due to accumulation of substances which are toxic or interfere with normal function, or to the effects of reduced ability to synthesize essential compounds. Many of the inborn errors of metabolism, including urea cycle defects, organic acidemias, and certain disorders of amino acid metabolism, present in the young infant with symptoms of an acute or chronic metabolic encephalopathy.</td>
</tr>
<tr>
<td>n=21 (38%)</td>
<td>Children with profound multiple disabilities have profound mental retardation and possess profound physical impairments. Children have signs of very serious neuromuscular dysfunction such as severe spasticity, muscle rigidity, and skeletal deformities as well as sensory impairments, have little or no control over their movements, and appear to have minimal or no physical potential to allow for independence in performing self-care routines.</td>
</tr>
<tr>
<td>n=62 (63%)</td>
<td>Sickle cell disease is an inherited disease, characterized by chronic anemia and recurrent painful vaso-occlusive crises, which occur unpredictably and require immediate management. Many patients eventually develop organ damage</td>
</tr>
<tr>
<td>n=21 (37%)</td>
<td>Spina bifida is a neuroembryological disorder with complex physical and neuropsychological morbidity. The majority of children with myelomeningocele (SBM), the severe form of SB, develop hydrocephalus and associated cerebral malformations of the posterior cortex and white matter, midbrain, cerebellum, and corpus callosum. Although children with SBM tend to score within the low average to average range of general intelligence measures, they are at risk of specific cognitive difficulties, learning disabilities and motor deficits.</td>
</tr>
</tbody>
</table>
4.4 Outline of the thesis

This thesis focuses on describing the above mentioned research questions, and is divided in a generic and a disease specific part. In the first part the generic results for all disease groups are presented, while the second part consists of descriptions of individual disease groups. The generic part starts with a description of parental HRQOL, including an estimation of the percentages of parents at risk for impairment of their HRQoL (Chapter 2). In the following chapter a comprehensive model predicting parental HRQoL, was tested using structural equation modelling. This model includes background, disease related variables, and social factors (Chapter 3). Subsequently, the next chapter focuses on parental well-being in comparison with parents of healthy children, and on estimating monetary costs of changes in well-being (Chapter 4). In the last chapter of the generic part, the social situation of parents of chronically ill children is addressed in terms of employment and leisure activity time (Chapter 5). The disease specific part describes two disease groups that had remarkable outcomes on one of the outcome variables. The HRQOL of mothers of children with sickle cell disease was further explored (Chapter 6), just like predictors of HRQOL of parents of children with metabolic diseases (Chapter 7). This thesis ends with a general discussion (Chapter 8) and a summary of the results.

---

**Table 2 Measurement: topics included in the Care-questionnaire**

<table>
<thead>
<tr>
<th>QUESTIONNAIRE PARTS</th>
<th>SUBJECTS</th>
</tr>
</thead>
<tbody>
<tr>
<td>Care questionnaire-General</td>
<td>The general section consisted of 68 questions regarding demographics,</td>
</tr>
<tr>
<td></td>
<td>education, employment (also for partner/spouse), child care, additional</td>
</tr>
<tr>
<td></td>
<td>burden in the family (e.g. chronic illness of parents), insurance, and</td>
</tr>
<tr>
<td></td>
<td>use of health care services, leisure activities and marital support.</td>
</tr>
<tr>
<td>Care-questionnaire-Disease related</td>
<td>This part consisted of 87 questions regarding child characteristics,</td>
</tr>
<tr>
<td></td>
<td>childcare, siblings, use of benefits, costs related to their child’s</td>
</tr>
<tr>
<td></td>
<td>disease, social changes, and employment situation.</td>
</tr>
<tr>
<td>Subjective well-being</td>
<td>On a scale from 1-10, well-being with the following aspects of life is</td>
</tr>
<tr>
<td></td>
<td>reported: employment, household chores, financial situation, housing,</td>
</tr>
<tr>
<td></td>
<td>leisure activities, available childcare, and facilities in the</td>
</tr>
<tr>
<td></td>
<td>neighbourhood, educational level, surroundings, and life as a whole.</td>
</tr>
<tr>
<td></td>
<td>The wellbeing questions have adequate validity and reliability.</td>
</tr>
<tr>
<td>Health Related Quality of Life</td>
<td>The ‘TNO-AZL Questionnaire for Adult’s Health Related Quality Of Life’</td>
</tr>
<tr>
<td></td>
<td>(TAAQOL). The questionnaire measures health status problems weighted</td>
</tr>
<tr>
<td></td>
<td>by the impact of problems on well-being on 12 multi-item scales: gross</td>
</tr>
<tr>
<td></td>
<td>and fine motor functioning, cognitive functioning, sleep, pain,</td>
</tr>
<tr>
<td></td>
<td>social functioning, daily activities, sexuality, vitality, positive</td>
</tr>
<tr>
<td></td>
<td>emotions, depressive emotions, and aggressiveness. The reliability of</td>
</tr>
<tr>
<td></td>
<td>the questionnaire was satisfactory to good.</td>
</tr>
</tbody>
</table>

---

**QUESTIONNAIRE PARTS**

- **Care questionnaire-General**
  - The general section consisted of 68 questions regarding demographics, education, employment (also for partner/spouse), child care, additional burden in the family (e.g. chronic illness of parents), insurance, and use of health care services, leisure activities and marital support.

- **Care-questionnaire-Disease related**
  - This part consisted of 87 questions regarding child characteristics, childcare, siblings, use of benefits, costs related to their child’s disease, social changes, and employment situation.

- **Subjective well-being**
  - On a scale from 1-10, well-being with the following aspects of life is reported: employment, household chores, financial situation, housing, leisure activities, available childcare, and facilities in the neighbourhood, educational level, surroundings, and life as a whole. The wellbeing questions have adequate validity and reliability.

- **Health Related Quality of Life**
  - The ‘TNO-AZL Questionnaire for Adult’s Health Related Quality Of Life’ (TAAQOL). The questionnaire measures health status problems weighted by the impact of problems on well-being on 12 multi-item scales: gross and fine motor functioning, cognitive functioning, sleep, pain, social functioning, daily activities, sexuality, vitality, positive emotions, depressive emotions, and aggressiveness. The reliability of the questionnaire was satisfactory to good.
CHAPTER 1 General introduction

REFERENCES


