Functional aspects of spina bifida in childhood

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FUNCTIONAL ASPECTS OF SPINA BIFIDA IN CHILDHOOD

FUNCTIONELE ASPECTEN VAN SPINA BIFIDA OP DE KINDERLEEFTIJD

(met een samenvatting in het Nederlands)

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“Ontmoedig nooit iemand die vooruitgang boekt,
hoe langzaam ook.”

Uit: Hulton Getty, Ga ervoor! (2003)

Voor alle kinderen met spina bifida

Denkend aan Henk en Hans

Aan mijn ouders

CONTENTS

LIST OF ABBREVIATIONS

ADL	activities of daily living
BMI	body mass index
BSID	Bayley Scales of Infant Developement
CA	caregiver assistance
CI	confidence interval
CIC	clean intermittent catheterization
CT	computerized tomography
CNS	central nervous system
CW	community walker
EW	exercise walker
FS	functional skills
HW	household walker
ICC	intraclass correlation coefficients
ICF	international classification of functioning, disability and health
ICIDH	international classification of impairments, disabilities, and handicaps
IQ	intelligence quotient
IQR	interquartile range
HC	hydrocephalus
HRQL	health related quality of life
LE	lower extremities
LMMC	lipomyelomeningocele
M-ABC	Movement Assessment Battery for Children
MMC	myelomeningocele
MMT	manual muscle testing
MRC	medical research council
MRI	magnetic resonance imaging
MV	missing value
NDTs	neural tube defects
NS	not significant

NSS	normative standard scores
NW	normal walker
OR	odss ratio
OTSB	other types of spina bifida
PEDI	Pediatric Evaluation of Disability Inventory
PSPC	Pictorial Scale of Perceived Competence and social acceptance
SBSQ	Spina Bifida Spine Questionnaire
SD	standard deviation
SPPA	Self Perception Profile for Adolescents
SPPC	Self Perception Profile for Children
SPPS	statistical package of social sciences
SS	scaled scores
TS	total scores
VUR	vesico-ureteral reflux
WCD	wheelchair-dependent
WHO	world health organization

Abbreviations in Dutch summary

CZS	centraal zenuwstelsel
KvL	kwaliteit van leven
OVSB	overige vormen van spina bifida

Introduction

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SPINA BIFIDA

Spina bifida is a complex congenital disorder that represents a broad spectrum of neural tube defects (NTDs), including spina bifida occulta and aperta. The incidence varies in different parts of the world. Nowadays, it is generally 0.4-1.0 per 1000 births in the USA, while a slightly higher incidence is found in Northern Europe [1]. Over the last 20 years, there has been an enormous decline in the number of NTDs due to primary prevention, such as universal folic acid supplementation during pregnancy. Due to widespread screening programs for pregnant women (diagnosis in utero), secondary prevention has become possible (termination of affected pregnancies) [2]. Nevertheless, these steps will probably not entirely eliminate NTDs.

In occult forms of spina bifida, representing disorders of the caudal neural tube formation, the lesions are covered with intact skin and function is often normal at birth. Therefore, the abnormality often goes undetected for years, but during life, symptoms can occur due to spinal tethered cord syndrome. Patients with symptomatic spina bifida occulta have varying degrees of lower extremity weakness, progressive locomotion problems, sensory impairment, or neurogenic bladder and bowel dysfunction [3-9].

The term spina bifida aperta refers to 'open' forms of spina bifida such as myelomeningocele (MMC) and meningocele. In MMC, there is protrusion of the spinal cord, meninges, and cerebral spinal fluid into a sac. Both brain and spinal cord are often malformed, resulting in various degrees of abnormalities, such as hydrocephalus, and malformation of the brainstem, known as Chiari II malformation [10]. Persons may also develop other problems of the central nervous system such as hydromyelia, lipomas, or tethered spinal cord syndrome [11], that occur in patients with spina bifida occulta as well. All these neurological problems affect the function of upper and lower extremities, producing varying degrees of weakness or spasticity, lack of sen-

sation, bladder and bowel incontinence, as well as neurocognitive dysfunction [12-21].

In patients with meningocele, the sac contains meninges and cerebrospinal fluid, but the spinal cord remains within the spinal canal. Therefore, clinical symptoms are less severe than in MMC. As MMC is the most common and most serious form of open neural tube defect, the majority of research has been focused on this defect.

If left untreated, MMC is a defect with an extremely high mortality. Before neurosurgical treatment was established in the 1960s, most patients died from hydrocephalus, meningitis and/or perinatal problems. Treatment of hydrocephalus became available with the introduction of the cerebrospinal fluid shunt. However, the majority of children who survived as a result of the shunt, were severely disabled [12,13]. Therefore, from the 1960s to 1980s the treatment changed from active therapy regardless of the lesion level, to selective treatment based on deficits as assessed by a neurological examination. Due to concerns about the predictive accuracy of this neurological examination aggressive therapy is carried out in many countries [1,13]. The mortality of newborn infants with NTDs has been significantly reduced by scientific advances in the medical field such as improved neonatal care, effective antibiotics, and non-invasive nervous system imaging techniques including magnetic resonance imaging (MRI), ultrasound and computerized tomography (CT-) scans.

Improved surgical techniques have further reduced morbidity. The primary neurosurgical intervention is the repair of the MMC. The major goals of this procedure are to eliminate the cerebrospinal fluid leak, to prevent infection, to preserve neural function, and to prevent secondary tethering of the spinal cord at the level of the surgical repair [22-25]. Primary closure is mostly performed within 24-72 hours after birth, allowing time for general pediatric and neurological evaluation of the newborn. Hydrocephalus is not often present at birth, but shunting is usually required within the first week of life [10,21,22]. The optimal timing for shunting is still a topic in current medical research.

Over the last years, there is a debate on the role of intrauterine surgery for children with MMC [10,21]. Bannister [10] reported arguments for and against intrauterine surgery. She stated that fetal surgery should significantly improve function in terms of bowel and bladder control, sexual function and walking abilities, compared to conventional treatment, to justify the risks that this treatment imposes on the mother and the fetus. Furthermore, minor improvements in neurological status do not necessarily significantly improve functional outcome.
Improved medical treatment in urology and orthopedics also has reduced mortality and morbidity. During earlier decades, death occurred due to renal failure as a result of vesicoureteral reflux (VUR) and hydronephrosis. With the introduction of pediatric urodynamics in the middle 1970s, it became clear that these problems were associated with abnormal activity (hyperactivity or inactivity) of pelvic floor and detrusor. In about 50% of children with MMC, detrusor-sphincter dyssynergia creates a functional obstruction of the bladder outlet that is responsible for VUR and renal damage [26]. Nowadays, with the introduction of clean intermittent catheterization to empty the urinary bladder, and treatment of detrusor overactivity with anticholinergics, obstructive uropathy and renal damage can be avoided. A major advance in orthopedic surgery, is the stabilization of scoliosis by internal fixation devices [27-29].
Today, with advanced medical treatment, more children with spina bifida survive into adulthood. It is of major importance that spina bifida research not only focuses on medical aspects, but includes the impact of the disorder on personal and social levels as well. Ongoing disability research is therefore of clinical importance.

Disability concepts

There are three major disability concepts. Firstly, such as the 'disability model' as developed by Saad Nagi [30] in 1965 and modified by Pope and Tarlov [31] in 1991. Secondly, the International Classification

of Impairments, Disabilities and Handicaps (ICIDH 1 & Beta 2 draft) developed by the World Health Organization (WHO) [32,33]. Thirdly, in the middle-nineties, the 'disablement process' based on the work of Nagi and described by Verbrugge and Jette [34], was advocated as a primary tool for physical therapy research and decision making [35]. Therefore, we chose this model to guide us in our research on the functional consequences of spina bifida, instead of the ICIDH model that was often used in European rehabilitation research. The ICIDH concept was primarily designed as a classification model for coding and manipulating data on the consequences of health conditions. Moreover, the model did not differentiate between 'functional limitations' (individual capability) and 'disability' (actual performance in response to the environment), which are important issues in childhood research [34-36]. Recently, the International Classification of Functioning, Disability and Health (ICF), designed by the WHO in 2001 is advised [37].

In the 'disablement process' as described by Verbrugge and Jette [34], the term 'disablement' refers to "impacts that chronic and acute conditions have on the functioning of specific body systems, and on people's abilities to act in necessary, usual, expected and personally desired ways in their society". The 'disablement process' describes how chronic and acute conditions can affect functioning in specific body systems, generic physical and mental actions, and activities of daily life. Additionally, it describes personal and environmental factors that can speed up or slow down the disablement [34].

The 'disablement process' consists of four different domains: 'pathology', 'impairments', 'functional limitations' and 'disability'. 'Pathology' refers to "biomedical and physiological abnormalities that are detected and medically labeled as disease, injury or congenital/developmental conditions". 'Impairments' are defined as "dysfunctions and significant structural abnormalities in specific body systems". 'Functional limitations' are "restrictions in performing fundamental physical and mental actions used in daily life". 'Disability' is defined as "experienced difficulty doing activities in any domain of

life". There are several factors that might influence the outcome of this pathway, such as intra-individual factors (coping, life style, self-esteem), extra-individual factors (medical care, rehabilitation) and risk factors (environment, predisposition). The differences between 'functional limitations' and 'disabilities' can be characterized as 'can do' versus 'does do'. 'Functional limitations' refer to individual capability, whereas 'disability' refers to actual performance in response to the environment. The combination of these two domains are included in the field of 'health related quality of life' (HRQL) in which elements of emotional well-being, competence and life satisfaction are included [35-36].

Previous disablement models, such as Nagi's [30] or the ICIDH-1 [32], presenting the main pathway of disablement as a more or less linear progression of response to illness, and disabling conditions have been viewed as static entities. Verbrugge and Jette [34-36] emphasized that "disablement is more often a dynamic process that can fluctuate in breadth and severity across the life course". The 'disablement process' as described by them, has a lot in common with the most recent International Classification of Functioning, Disability and Health (ICF) [37], and shown in Figure 1 [36]. In the ICF model, each component can be expressed in both positive and negative terminology. Therefore, it might be seen as an enablement framework as well as a disablement framework [36,37].

'Disablement process' and HRQL in spina bifida

Physical therapy is a commonly used intervention for children with spina bifida. Traditionally, therapists focused on improvement of muscle strength, muscle tone, and the prevention of contractures, to optimize childhood development and functioning [38]. However, it is not clear how these 'impairments' are related to functional independence and HRQL. In various other chronic diseases, impairment parameters do not significantly correlate with functional abilities and disability

	Anatomical Body Parts	Physiological Functioning of the Body	Task Performance	Involvement in Life Roles
Disablement Model	Pathology	Impairments	Functional limitations	Disability
	Disease, injury, congenital condition	Dysfunction and structural abnormalities in specific body systems	Restriction in basic physical and mental actions	The expression of a physical or mental limitation in a social context
ICF	Body Function and Structures		Activities and Participation	
	Physiological function of body systemanatomical parts of body		Activity: Execution and of a taskor action Participation: Involvement in a life situation	

Figure 1

Disablement model and International Classification of Functioning, Disability and Health (ICF). Adapted from Jette and Keysor, 2003 [36].

parameters [39,40]. Most outcome studies in the field of spina bifida research focus on disease aspects ('pathology') and its relation to one of the domains in the 'disablement process', such as physical functioning [12,13,16,17,20-22], cognitive abilities [14,15,20], or psychosocial adjustment [41-46].

The relation between lesion level, muscle strength and ambulation has been studied extensively [47-53]. McDonald et al. [50] studied the relationship between patterns of lower-extremity muscle strength and ambulation in 291 children with MMC. They found m. iliopsoas strength to be the best predictor of ambulation, with the m.quadriceps, m. tibialis anterior and mm. gluteï also contributing significantly. Dudgeon et al. [53] reported that patients with midlumbar lesions demonstrate most variation in ambulation level. Hunt et al. [12-14] reviewed a complete cohort of children with MMC 25 years after closure, and looked at several aspects of disability. Conditions relevant to independence were lesion level, shunt status, intelligence, ambulation level, and pressure sores. Intelligence appeared to be significantly related with shunt status. It is important to keep in mind that these findings reflect the effects of treatment strategies carried out in the 1980s. Only few studies focus on family function [41,43,45]. McCormick et al. [41] reported that the impact of a child with spina bifida on family function, related less to the clinical diagnosis than to the child's functioning at home with regard to activities of daily living. Therefore, the child's function at home should be included in evaluations.

From the above mentioned literature on long-term outcome, it became clear that spina bifida has a significant impact on the health of individuals. It is important to find out how 'pathology', 'impairments', 'functional limitations' and 'disability, and HRQL relate to each other in children with MMC, as well as in those with other types of spina bifida (OTSB). The possible interrelationships between these domains, is the main focus of this thesis.

Literature on HRQL in children with spina bifida is scarce [54-57]. Recently, Parkin et al. [54] developed a disease specific 'Health Related

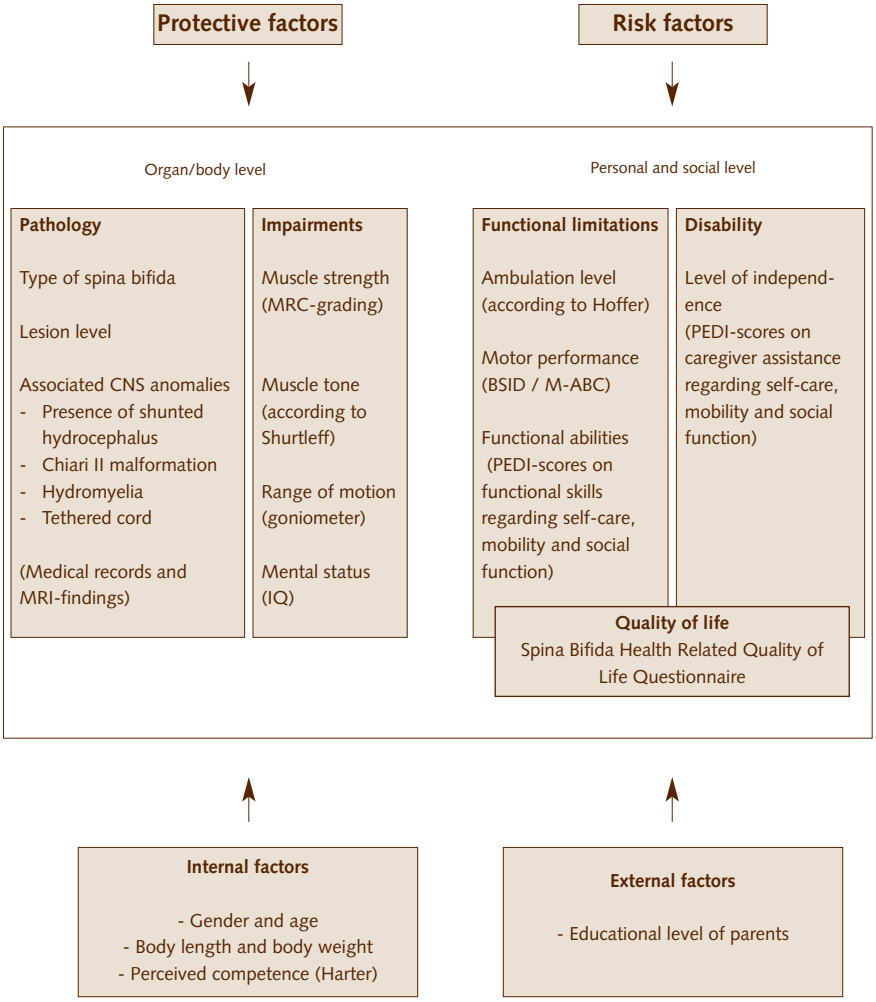


Figure 2

Variables and measurements used in this thesis to evaluate the 'disablement process' in childhood spina bifida. All measurements will be explained and discussed in the different chapters of this thesis.

Abbreviations: CNS = central nervous system, MRC = medical research council, IQ = intelligence quotient, BSID = Bayley Scales of Infant Development, M-ABC = Movement Assessment Battery for Children, PEDI = Pediatric Evaluation of Disability Inventory.

Quality of Life Questionnaire' for use in children and adolescents with spina bifida. With this questionnaire, HRQL can be evaluated from the perspective of the individuals themselves and their parents, rather than from the perspective of clinicians. It is has been shown that children with spina bifida and their families do not view their health as negatively as their physicians do [43,54].

An overview of the variables and measurements used in this thesis to evaluate the Disablement Process in childhood spina bifida, are shown in Figure 2.

AIMS OF THE THESIS

Based on the lack of knowledge on the different aspects of the 'disablement process' in children with spina bifida, as well as on the relationships between 'pathology', 'impairments', 'functional limitations', 'disability' and HRQL, the aim of this thesis is:

- to compare outcome in patients with myelomeningocele (MMC), with those with other types of spina bifida (OTSB) and to investigate associations between 'impairments', 'functional limitations', and 'disability', perceived competence and HRQL.
- to investigate determinants for functional independence and study which functional abilities were determinants for HRQL in children with MMC.
- to investigate functional outcome in two groups of children with sacral level paralysis: MMC versus lipomyelomeningocele (LMMC).

Due to the scarce knowledge of the functional consequences of neurosurgical release of spinal tethered cord and surgical correction of spinal deformities, other aims of this thesis are:

- to determine long-term outcome of neurosurgical untethering on neurosegmental motor level and ambulation level in children with tethered spinal cord syndrome.
- to determine the effects of spinal fusion on ambulation level and functional abilities in children with spina bifida.

OUTLINE OF THE THESIS

The first Chapter is a brief introduction on spina bifida and issues regarding the 'disablement process'. The conceptual framework and the aim of this thesis are formulated.
In Chapter 2, we compare 'pathology' parameters (type of spina bifida, presence of associated central nervous system abnormalities, and lesion level), 'impairment' parameters (mental status, range of motion and muscle strength), as well as 'functional limitation' and 'disability' parameters (ambulation level, functional skills and caregiver assistance, perceived competence, and HRQL) in patients with MMC with those with OTSB. Within the MMC group we compare outcome in children with and without hydrocephalus. We also investigate associations within the 'disablement process', such as associations between muscle strength ('impairment') and ambulation level ('functional limitation'), and associations of perceived competence with mental status, ambulation level, functional abilities and amount of caregiver assistance, and HRQL. Associations of HRQL with mental status, ambulation level, functional abilities and amount of caregiver assistance are also studied in this chapter.
In Chapter 3, we investigate which factors of the 'disability process' domains are the most important determinants for functional independence regarding self-care and mobility in children with MMC. With regard to HRQL, our main interest was to find out which functional abilities were the most important determinants for quality of life in order to set relevant and realistic goals for physical therapy treatment.
In Chapter 4, we investigate muscle strength, ambulation level, motor performance and functional outcome regarding self-care, mobility and social function, in two groups of children with sacral level spina bifida (MMC versus LMMC). The differences in outcome in children with MMC (who all had hydrocephalus and Chiari II malformation) and those with LMMC (without these central nervous system abnormali-

ties) are discussed. Each group is compared with reference values of the normal population, regarding motor performance and functional outcome.
In Chapter 5, the long-term effects of neurosurgical untethering on neurosegmental motor level and ambulation level are studied. We also tried to gain insight into risk factors in deterioration of ambulation level and in the occurrence of retethering.
In Chapter 6, the influence of surgical correction of spinal deformities on ambulation level and functional abilities, is studied prospectively. The course of ambulation, functional skills and the amount of caregiver assistance needed regarding self-care and mobility is evaluated from pre- to 18 months post-surgery.
In Chapter 7, we present a summary and general discussion. Conclusions on the importance of disability research are drawn, and directions for future research are given.

REFERENCES

1 Kirpalani HM, Parkin PC, Willan AR, Fehlings DL, Rosenbaum PL, King D, Van Nie AJ. Quality of life in spina bifida: importance of parental hope. Arch Dis Child 2000; 83(4):293-297.

2 Seller MJ. Risks in spina bifida. Dev Med Child Neurol 1994; 36:1021-1025.

3 Bakker-Niezen SH, Walder HAD, Merx JL. The tethered spinal cord syndrome. Z Kinderchir 1984; 39(Suppl 2):100-103.

4 Begeer JH, Wiertsema GPA, Breukers SME, Mooy JJ, Weeme CA ter. Tethered cord syndrome: Clinical signs and results of operation in 42 patients with spina bifida aperta and spina bifida occulta. Z Kinderchir 1989; 44(Suppl 1):5-7.

5 Lagae L, Verpoorten C, Casear P, Vereecken R, Fabry G, Plets C. Conservative versus neurosurgical treatment of tethered cord patients. Z Kinderchir 1990; 45(Suppl 1):16-17.

6 Pang D. Split cord malformation: Part II Clinical syndrome. Neurosurgery 1992; 31(3):481-500.

7 Sarwark JF, Weber DT, Gabrieli AP, McLone DG, Diaz L. Tethered cord syndrome in low motor level children with myelomeningocele. Pediatr Neurosurg 1996; 25(6):295-301.

8 McLone DG, LaMarca F. The tethered spinal cord: diagnosis, significance, and management. Semin Pediatr Neurol 1997; 4(3):192-208.

9 Pierre-Kahn A, Zerah M, Renier D, Cinalli G, Sainte-Rose C, Lellouch-Tubiana A, Brunelle F, Le Merrer M, Giudicelli Y, Pichon J, Kleinknecht B, Nataf F. Congenital lumbosacral lipomas. Child's Nerv Syst 1997; 13(6):298-335.

10 Bannister CM. The case for and against intrauterine surgery for myelomeningoceles. Eur J Obstet Gynecol Reprod Biol 2000; 92(1):109-113.

11 Kinsman SL, Levey E, Ruffing V, Stone J, Warren L. Beyond multidisciplinary care: a new conceptual model for spina bifida services. Eur J Pediatr Surg 2000; 10(Suppl 1):35-38.

12 Hunt GM. Open spina bifida: outcome for a complete cohort treated unselectively and followed into adulthood. Dev Med Child Neurol 1990; 32(2):108-118.

13 Hunt GM, Poulton A. Open spina bifida: a complete cohort reviewed 25 years after closure. Dev Med Child Neurol 1995; 37(1):19-29.

14 Hunt GM, Oakeshott P, Kerry S. Link between the CFS shunt and achievement in adults with spina bifida. J Neurol Neurosur Psychiatry 1999; 67(5):591-595.

15 McLone DG. Continuing concepts in the management of spina bifida. Pediatr Neurosurg 1992; 18(5-6):254-256.

16 Steinbock P, Irvine B, Cochrane DD, Irwin BJ. Long-term outcome and complications of children born with meningomyelocele. Child's Nerv Syst 1992; 8:92-96.

17 Date I, Yagyu Y, Asari S, Ohmoto T. Long-term outcome in surgically treated spina bifida cystica. Surg Neurol 1993; 40(6):471-475.

18 Dahl M, Ahlsten G, Ronne-Engström E, Lagerkvist B, Magnusson G, Norrlin S, Olsen L, Ströberg B, Thuomas K. Neurological dysfunction above cele level in children with spina bifida cystica: A prospective study to three years. Dev Med Child Neurol 1995; 37:30-40.

19 Shurtleff DB. 44 Years experience with management of myelomeningocele: presidential address, society for research into hydrocephalus and spina bifida. Eur J Pediatr Surg 2000; 10 (Suppl 1):5-8.

20 Bowman RM, McLone DG, Grant JA, Tomita T, Ito JA. Spina bifida outcome: a -25 year prospective. Pediatr Neurosurg 2001; 34(3):114-120.

21 Rintoul NE, Sutton LN, Hubbard AM, Cohen B, Melchionni J, Pasquariello PS, Adzick NS. A new look at myelomeningoceles: functional level, vertebral level, shunting, and the implications for fetal intervention. Pediatrics 2002; 109(3):409-413.

22 Wagner W, Schwarz M, Perneczky A. Primary myelomeningocele closure and consequences. Curr Opin Urol 2002; 12(6):465-468.

23 Archibeck MJ, Smith JT, Carroll KL, Davitt JS, Stevens PM. Surgical release of the tethered spinal cord: survivorship analysis and orthopedic outcome. J Pediatr Orthop 1997; 17(6):773-776.

24 Calenbergh F van, Vanvolsem S, Verpoorten C, Lagae L, Casaer P, Plets C. Results after surgery for lumbosacral lipoma: the significance of early and late worsening. Child's Nerv Syst 1999; 15(9):439-443.

25 Boemers TML, Gool JD van, Jong TPVM de. Tethered spinal cord: the effect of neurosurgery on the lower urinary tract and male sexual function. Br J Urol 1995; 76(6):747-751.

26 Gool JD van, Dik P, Jong TPVM de. Bladder-sphincter dysfunction in myelomeningocele. Eur J Pediatr 2001; 160(7):414-420.

27 Mazur J, Menelaus MB, Dickens DRV, Doig WG. Efficacy of surgical management for scoliosis in myelomeningocele: Correction of deformity and alteration of functional status. J Pediatr Orthop 1986; 6(5):568-575.

28 Banta JV. Combined anterior and posterior fusion for spinal deformity in myelomeningocele. Spine 1990; 15(9):946-951.

29 Boemers TML, Soorani-Lunsing IJ, Jong TPVM de, Pruijs JEH. Urological problems after surgical treatment of scoliosis in children with myelomeningocele. J Urology 1996; 155(3):1066-1069.

30 Nagi SZ. Some conceptual issues in disability and rehabilitation. In: Sussman MB, editor. Sociology and rehabilitation. Washington, DC: American Sociological association; 1965.

31 Pope AM, Tarlov AR. A model for disability and disability prevention. In: Disability in America: toward a national agenda for prevention. Washington, DC: Institute of Medicine National Academy Press; 1997:76-108.

32 The International Classification of Impairments, Disabilities and Handicaps -a manual relating to the consequences of disease. Geneva: World Health Organization; 1980.

33 ICIDH-2 International Classification of Functioning and Disability, Beta-2 Draft. Üstün TB, editor. (Beta 2 draft). Genua: World Health Organization; 1999.

34 Verbrugge LM, Jette AM. The disablement process. Soc Sci Med 1994; 38(1):1-14.

35 Jette AM. Physical disablement concepts for physical therapy research and practice. Phys Ther 1994; 74(5):380-386.

36 Jette AM, Keysor JJ. Disability models: Implications for arthritis exercise and physical activity interventions. Arthritis Rheum 2000; 49(1):114-120.

37 ICF International Classification of Functioning, Disability and Health. Geneva: World Health Organization; 2001.

38 Ryan KD, Ploski C, Emans JB. Myelodysplasia- the musculoskeletal problem: habilitation from infancy to adulthood. Phys Ther 1991; 71(2):935-945.

39 Net J van der, Prakken ABJ, Helders PJM, Berge M ten, Herwaarden M van, Sinnema G, Wilde EJ de, Kuis W. Correlates of disablement in polyarticular juvenile chronic arthritis; a cross sectional study. Br J Rheumtol 1996; 35(1):91-100.

40 Engelbert RHH, Graaf Y van der, Empelen R van, Beemer FA, Helders PJM. Osteogenesis imperfecta in childhood: impairment and disability. Pediatrics 1997; 99(2):1-7.

41 McCormick MC, Charney EB, Stemmler MM. Assessing the impact of a child with spina bifida on the family. Dev Med Child Neurol 1986; 28(1):53-61.

42 Hagelsteen JH, Lagergren J, Lie HR, Rasmussen FR, Börjeson MC, Lagerkvist B, Muttilainen M, Taudorf K, Kohler L. Disability in children with myelomeningocele. Acta Paediatr Scand 1989; 78(5):276-280.

43 Rasmussen FR, Lie HR, Hagelsteen JH, Lagergren J, Börjeson MC, Lagerkvist B, Kohler L. Nordic children with myelomeningocele. Parents' assessment of the handicap and physicians' classification of the disabilities. Acta Paediatr 1993; 82(3):276-280.

44 Appelton PL, Minchom PE, Ellis NC, Elliott CE, Böll V, Jones P. The self-concept of young people with spina bifida: a population based study. Dev Med Child Neurol 1994, 36(3):198-215.

45 Lie HR, Börjeson MC, Lagerkvist B, Rasmussen F, Hagelsteen JH, Lagergren J. Children with myelomeningocele: the impact of disability on family dynamics and social conditions. A Nordic study. Dev Med Child Neurol 1994; 36(11):1000-1009.

46 Minchom PE, Ellis NC, Appelton PL, Lawson V, Böll V, Jones P, Eliott CE. Impact of severity on self-concept in young people with spina bifida. Arch Dis Child 1995; 73:48-52.

47 Hoffer M, Feiwell, Perry J, Bonnet C. Functional ambulation in patients with myelomeningocele. J Bone Joint Surg Am 1973; 55(1):137-148.

48 Asher M, Olson J. Factors affecting the ambulatory status of patients with spina bifida cystica. J Bone Joint Surg Am 1983; 65(3):350-356.

49 Banta JV, Casey JM, Bedell L, Morgan J. Long-term ambulation in spina bifida. Dev Med Child Neurol 1983; 25:110.

50 McDonald CM, Jaffe KM, Mosca VS, Shurtleff DB. Ambulatory outcome of children with myelomeningocele: effect of lower-extremity muscle strength. Dev Med Child Neurol 1991; 33(6):482-490.

51 Liopis ID, Munoz MB, Agullo EM, Martinez AL, Aymerich VG, Valero JVF. Ambulation in patients with myelomeningocele: a study of 1500 patients. Paraplegia 1993; 31:28-32.

52 Swank M, Dias LS. Walking ability in spina bifida patients: a model for predicting future ambulatory status based on sitting balance and motor level. J Pediatr Orthop 1994; 14(6):715-718.

53 Dudgeon BJ, Jaffa KM, Shurtleff DB. Variations in midlumbar myelomeningocele: Implications for ambulation. Pediatr Phys Ther 1991; 4:57-62.

54 Parkin PC, Kirpalani HM, Rosenbaum PL, Fehlings DL, Van Nie A, Willan AR, King D. Development of a health-related quality of life instrument for use in children with spina bifida. Qual Life Res 1997; 6(2):123-132.

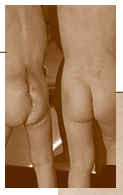
55 Pit-Ten Cate IM, Kennedy C, Stevenson J. Disability and quality of life in spina bifida and hydrocephalus. Dev Med Child Neurol 2002; 44(5):317-322.

56 Padua L, Rendeli C, Rabini A, Girardi E, Tonali P, Salvaggio E. Health-related quality of life and disability in young patients with spina bifida. Arch Phys Med Rehabil 2002; 83(10):1384-1388.

57 Sawin KJ, Brei TJ, Buran CF, Fastenau PS. Factors associated with quality of life in adolescents with spina bifida. J Holist Nurs 2003; 20(3):279-304.

Disablement process in children with spina bifida:

impairments, functional limitations, disability, perceived competence and quality of life.



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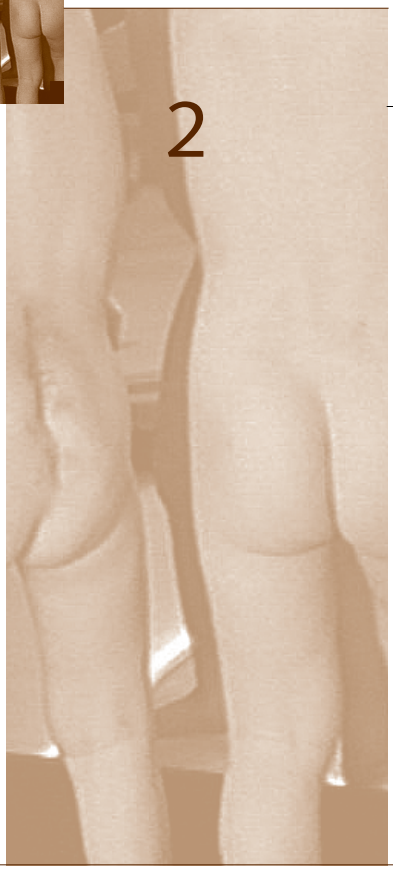

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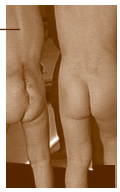
ABSTRACT

OBJECTIVES: To compare outcome in patients with myelomeningocele (MMC), and other types of spina bifida (OTSB), and to investigate associations between 'impairments', 'functional limitations', 'disability', perceived competence and quality of life.

DESIGN: Cross-sectional study of 177 children (122 MMC, 55 OTSB). Mean age 8.2 years; range 1-18 years. 'Pediatric Evaluation of Disability Inventory' (PEDI), Harter Self-Perception Profile, Health Related Quality of Life Questionnaire (HRQL), were used as main outcome measures.

RESULTS: The percentage of patients with disabilities regarding self-care, mobility and social function, was higher in MMC than in OTSB; 59%, 76%, and 43 %, compared to 31%, 27%, and 20 % respectively ($p < 0.001$). The percentage of patients with low HRQL scores was higher in MMC compared to OTSB, 63% and 15% respectively ($p < 0.001$); in the OTSB group, perceived competence was not better. HRQL showed positive associations with ambulatory status in different age groups (r_s = range 0.42-0.60, $p < 0.01$), with all domains of the PEDI (r = range 0.19-0.60, $p < 0.05$) and with mental status ($r = 0.71$, $p < 0.01$), whereas perceived competence showed no associations with most of these variables.

CONCLUSIONS: Although patients with MMC were more disabled than those with OTSB, perceived competence was not better in the latter group. Therefore, health care professionals should not mainly focus their attention on more disabled patients with MMC.



INTRODUCTION

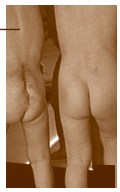
Spina bifida is a complex congenital disorder that represents a broad spectrum of neural tube defects, including spina bifida aperta and occulta. Spina bifida aperta includes myelomeningocele (MMC), the most common and more serious defect, and meningocele. In MMC, both spinal cord and brain are often malformed, resulting in serious locomotion problems, hand function problems, bladder and bowel incontinence, as well as cognitive dysfunction [1-4]. In occult forms of spina bifida, representing disorders of the caudal neural tube formation, function is often normal at birth, but during life, progressive locomotion problems or bladder and bowel dysfunction may occur, due to spinal tethered cord syndrome. These progressive problems might have impact on the quality of life in patients with occult forms of spina bifida as well as in patients with MMC [5,6].

The functional consequences of spina bifida can be displayed using the 'disablement process' as described by Verbrugge and Jette [7] consisting of four different domains: 'pathology', 'impairments', 'functional limitations' and 'disability'. There are several factors that might influence the outcome of this pathway, such as intra-individual factors (coping, life style, self-esteem), extra-individual factors (medical care, rehabilitation) and risk factors (environment, predisposition). This model has a lot in common with the most recent International Classification of Functioning, Disability and Health (ICF) designed by the World Health Organization (WHO) [8].

Most outcome studies focus on neurological outcome (parameters on organ level), while the impact of spina bifida on personal level ('functional limitation'/'activities') and social level ('disability'/'participation') is of major importance. Recently, some studies have been published on 'disability' and 'health related quality of life' (HRQL) in children with spina bifida [9,10]. However, the numbers in one of these series are small (n = 12) [9]. Moreover, in these series, patients

with MMC are not clearly differentiated from those with other types of spina bifida (OTSB) [9,10].

The aim of this study was to investigate outcome in the different domains of the 'disablement process', in a cohort of Dutch children with MMC, as well as those with other types of OTSB. Additionally, within the MMC group, we compared outcome in children with and without hydrocephalus. Moreover, we investigated associations within the 'disability process' domains, with special emphasis on 'impairments', 'functional limitations', 'disability', perceived competence and HRQL.



METHODS

The study group consisted of children, aged 1 to 18 years who have been followed-up in the outpatient spina bifida clinic of the Wilhelmina Children's Hospital. Participants included those with a diagnosis of spina bifida aperta (MMC or meningocele) and those with spina bifida occulta (lipomyelomeningocele, split cord malformation, lipomas of the conus or filum). Patients with cervical (myelo)meningocele, encephalocele, or cord traumas were excluded. Non-Dutch speaking patients were also excluded. One hundred and eighty-nine patients met the inclusion criteria, and 177 were willing to participate in the study (response rate 94%), carried out from January 1999 to September 2001. As most outcome studies often focus on patients with MMC only [1-4], we divided the different types of spina bifida into two groups, MMC (n = 122) and OTSB (n = 55). The Medical Ethics Committee of our hospital approved the protocol of this descriptive, cross-sectional study. Informed consent was obtained from the parents and from the patients themselves if they were older than 12 years of age.

Data were obtained by physical examination, and during a face-to-face structured interview with regard to functional abilities, perceived competence and health related quality of life. All measurements were performed by the same experienced paediatric physiotherapist (MS). Parents were questioned about the age of independent walking and the type of school (regular or special) their child attended.

Measurements with regard to 'pathology'

Data concerning the presence of shunted hydrocephalus, the presence of Chiari II malformation, the presence of corpus callosum aplasia or dysplasia, the position of the conus, the presence of lipomas or hydromyelia, were obtained from the medical records.

Determination of the neurosegmental motor level was based on muscle strength of the lower extremities as described by McDonald et al. [11].

Measurements with regard to 'impairments'

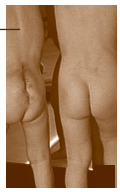
Information on mental status, such as Intelligence Quotients (IQs) (WISC-R) in children older than 4 years, was obtained from the psychological records.

Range of motion in lower extremities was measured in a standardised way with a two-legged 360-degrees goniometer, and compared to reference values for children [12]. The percentages of range of motion-loss were assessed on a 5-point scale according to Spiegel et al. [13]. Range of motion-loss of 5% or more in one or both extremities was defined as a contracture.

Muscle strength of lower extremities was graded 0-5, according to the standard Manual Muscle Testing (MMT) as described by Daniel's & Worthingham [14]. Muscle grading on a 6-point scale is not reliable, in children under the age of five years. Therefore, in these patients strength was graded as absent, weak, and full strength, as advocated by McDonald et al. [15]. Children with full strength or grade 5 were classified as having normal muscle strength.

Measurements regarding 'functional limitations', 'disability', perceived competence, and quality of life

Ambulation level was modified from Hoffer et al. [16] and documented in children older than 2.5 years of age. In Hoffer's description, patients with normal ambulatory skills are not distinguished from community walkers (walking with or without crutches or braces and may use a wheelchair for long distances). This difference was considered clinically important, and we described patients who walked without the use of aids as 'normal ambulant'.



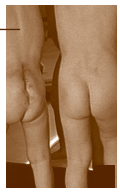
Functional abilities and the amount of caregiver assistance were scored using the Dutch version of the 'Pediatric Evaluation of Disability Inventory' (PEDI) [17,18]. The PEDI is a validated and reliable parental questionnaire which measures functional skills (FS) and caregiver assistance (CA) in three domains: self-care (including bladder and bowel management), mobility and social function. Reference values are provided for children between 0.5-7.5 years. Normal values are defined in the range of 2 SD (50 ± 20). In children > 7.5 years of age, results were calculated as a scaled score. In healthy children > 7.5 years of age, all functional skills should be mastered leading to a score of 100, which was considered to be normal.

Perceived competence was measured with the Dutch versions of the Harter Self-Perception Profile for Children (SPPC: 8-12 years) and Adolescents (SPPA: > 12 years) [19-20]. In young children (4-8 years) the Dutch version of the Pictorial Scale of Perceived Competence and Social Acceptance for young children was used (PSPC) [21]. These instruments measure the rate of competence that children and adolescents perceive their cognitive, physical and social function. Perceived competence was measured in children with IQs > 70. Items are scored on a four-point scale (1-4). A higher score on a subscale means a higher perceived competence in this area. For the purposes of the analyses of associations with disability and HRQL, we pooled the subscales into a total mean score of perceived competence.

Quality of life was measured with a Dutch-translation of the 'Spina Bifida HRQL Questionnaire' [22]. This instrument is a disease specific and age-related instrument for children and adolescents with spina bifida from 5 to 20 years of age. For children aged 5-12 years the questionnaire is designed as a proxy report, whereas adolescents aged 13-18 years completed the questionnaire by themselves. In children with an IQ below 70 the questionnaire was also used as a proxy report.

Statistical analysis

For comparison of subgroups we used Chi-square test (or Fisher's exact test when appropriate), if the variables were dichotomous. The Mann-Whitney non-parametric test was used for group comparison of mean scores. The Spearman rank-order or Pearson product moment correlation coefficient was used to assess the correlation between PEDI-scores, perceived competence and HRQL. Differences with p-values < 0.05 were considered statistically significant. Descriptive analysis was performed with the Statistical Package of the Social Sciences (SPSS 9.0).



RESULTS

Patient characteristics are listed in Table 1. We found no significant difference in age and gender between patients with MMC and those with OTSB.

Table 1 Patient characteristics.

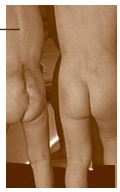
	Number n = 177
Types of spina bifida	
- myelomeningocele (MMC) (%)	122 (69)
HC+ (%)	97 (80)
HC- (%)	25 (20)
- meningocele (OTSB) (%)	9 (5)
HC+ (%)	3 (33)
HC- (%)	6 (66)
- lipomyelomeningocele (OTSB) (%)	18 (10)
- other types of occult spinal dysraphism (OTSB)	28 (16)
Male: female ratio (%)	75:102 (42:58)
Age in years	
- Mean (SD)	8.2 (5.2)
- Range	1.0 -18.8
Age distribution	
- 1-3.9 years (%)	49 (28)
- 4-7.9 years (%)	45 (25)
- 8-12.9 years (%)	42 (24)
- 13-17.9 years (%)	41 (23)

Abbreviations: MMC = myelomeningocele, HC+ = with shunted hydrocephalus, HC- = without hydrocephalus, OTSB = other types of spina bifida, SD = standard deviation.

Table 2 Patient numbers and percentages with regards to the 'pathology' domain of the 'disablement process', in patients with myelomenigocele (with and without hydrocephalus) compared to those with other types of spina bifida.

	MMC	OTSB		MMC HC+	MMC HC-	
	n = 122 (%)	n = 55 (%)	p-value	n = 97 (%)	n = 25 (%)	p-value
HC+	97 (80)	3 (5)	< 0.001	_	_	_
- shunt revisions	63 (65)	0		63 (65)	_	
Epilepsy	16 (13)	0	0.007	15 (16)	1 (4)	0.19 ns
Chiari II malformation	94 (77)	1 (2)	< 0.001	79 81)	15 (60)	0.003
- decompression	5 (5)	1 (2)		5 (6)	0	
- tracheostomy	2 (2)	1 (2)		2 (3)	0	
Corpus callosum						
- mv (n=39)	35	4		28	7	
- aplasia	9 (10)	2 (4)	< 0.001	9 (13)	0	< 0.001
- dysplasia	54 (62)	4 (8)	< 0.001	47 (68)	7 (39)	< 0.001
Conus below L2)	72 (85)	45 (82)	0.65 ns	53 (82)	19 (95)	0.14 ns
- mv (n=37)	37	0		32	5	
Lipomas	20 (24)	30 (55)	< 0.001	14 (22)	6 (30)	0.44 ns
- mv (n=37)	37	0		32	5	
Retethering	10 (8)	12 (22)	0.01	8 (8)	2 (8)	1.0 ns
Hydromyelia	23 (26)	10 (19)	0.30 ns	21 (31)	2 (10)	0.052 ns
- mv (n=35)	34	1		30	4	
Lesion level			< 0.001			0.005
- TH12	25 (21)	0		23 (24)	2 (8)	
- L1-L3	26 (21)	0		21 (22)	5 (20)	
- L4	23 (19)	6 (11)		21 (22)	2 (8)	
- L5	22 (18)	3 (5)		17 (17)	5 (20)	
- below S1	15 (12)	12 (22)		11 (11)	4 (16)	
- no loss LE	11 (9)	34 (62)		4 (4)	7 (28)	

Abbreviations: MMC = myelomeningocele, OTBS = other types of spina bifida, HC+ = with shunted hydrocephalus, HC- = without hydrocephalus, ns = not significant, mv = missing values, LE = lower extremities.



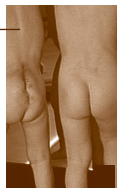
Results with regard to the 'pathology' domain are shown in Table 2. In patients with MMC and shunted hydrocephalus, 65% (63/97) needed revision of their shunt, with a mean number and standard deviation (SD) of 1.6 (2.5), ranging from 1 to 17. The percentage of patients with Chiari II malformation or corpus callosum aplasia or dysplasia was significantly higher in those with MMC and hydrocephalus than in those without hydrocephalus. Lipomas and retethering were significantly more frequently seen in patients with OTSB than in those with MMC. The OTSB group included 18 patients with lipomyelomeningocele, and 12 out of the remaining 37 patients with OTSB also had lipomas. Symptoms of retethering were mostly seen in patients with lipomyelomeningocele, 50% (9/18) compared to 8% (3/37) of the remaining patients in the OTSB group. The percentage of patients with high lesion levels (above L4) was significantly higher in patients with MMC and hydrocephalus than in those without hydrocephalus or other OTSB.

Results regarding 'impairments' are listed in Table 3. The percentage of patients with lower IQs was higher in those with MMC and shunted hydrocephalus, than in those without hydrocephalus. The mean IQ (SD) of patients with MMC, with and without hydrocephalus, was 80.7 (18.1) and 96.2 (23.4) respectively. The mean IQ (SD)of patients with OTSB was 98.8 (12.4). We found that 46% (56/122) of the children in the MMC group visited special schools. Although not statistically significant, the percentage was higher in those with shunted hydrocephalus than in those without, 50% (48/97) compared to 32% (32/35). The percentage of patients with contractures or abnormal muscle strength of lower extremities (MRC < 5) was significantly higher in patients with MMC and hydrocephalus than in those without hydrocephalus or OTSB. In the OTSB group, contractures were only seen in the feet, whereas patients with MMC showed hip and knee contractures as well, 21% (25/122) and 27% (33/122) respectively. Muscles innervated from the lower lumbo-sacral segments (hip abductor-, hip extensor- and calf muscles) showed weakness in 24 to 33% of the OTSB group, compared to 79 to 91% in the MMC group.

Table 3 Patient numbers and percentages regarding 'impairments', in patients with myelomenigocele (with and without hydrocephalus), compared to those with other types of spina bifida.

	MMC	OTSB		MMC HC+	MMC HC-	
	n = 122 (%)	n = 55 (%)	p-value	n = 97 (%)	n = 25 (%)	p-value
Mental status						
- mv (n=49)	40	9		20	8	
- IQ < 80	34 (41)	5 (11)	0.01	32 (42)	2 (12)	0.02
Contractures LE	63 (52)	16 (29)	0.005	55 (57)	8 (32)	0.03
Abnormal muscle strength (<5)						
- mv (n=1)	1	0		1	0	
- hip flexors	57 (47)	3 (6)	< 0.001	50 (52)	7 (28)	0.03
- hip abductors	96 (79)	13 (24)	< 0.001	82 (85)	14 (56)	0.001
- hip extensors	105 (87)	13 (24)	< 0.001	50 (52)	7 (28)	0.03
- knee extensors	53 (44)	2 (4)	< 0.001	47 (49)	6 (24)	0.03
- knee flexors	76 (63)	7 (13)	< 0.001	65 (68)	11 (44)	0.03
- ankle dorso-flexors	84 (69)	12 (22)	< 0.001	72 (86)	12 (48)	0.009
- ankle plantar-flexors	110 (91)	18 (33)	< 0.001	94 (98)	16 (64)	< 0.001

Abbreviations: MMC = myelomeningocele, OTBS = other types of spina bifida, HC+ = with shunted hydrocephalus, HC- = without hydrocephalus, mv = missing values, IQ = intelligence quotient, LE = lower extremities.



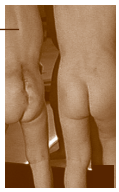
Results regarding 'functional limitations', 'disability', perceived competence and HRQL are listed in Table 4. In children who were old enough to ambulate (> 2.5 yrs), 38% percent of patients with MMC were functional walkers (normal or community) compared to 97% of those with OTSB. Within the MMC group functional ambulation was seen in 30% of the patients with hydrocephalus, compared to 73% in those without hydrocephalus. The mean age (SD) for walking independently was 32 (19.1) months in patients with lumbosacral MMC and 20 (9.6) months in those with OTSB. The percentage of patients with deviant PEDI-scores on functional skills and caregiver assistance was significantly higher in the MMC group than in the OTSB group, for all PEDI domains. Of the total group of patients, 36% (64/177) was completely independent (normal scores on caregiver assistance). The remaining 64% (113/177) needed some kind of caregiver assistance, especially regarding self-care, mainly concerning bowel and bladder management, even in non-MMC patients. Seventy-one percent (126/177) of the patients were on intermittent catheterization and 43% (76/177) needed colon enemas, for faecal continence.

We found no significant differences in perceived competence between these groups, nor within the MMC group (Table 4). Mean scores on the different subscales of perceived competence are shown in Figure 1a-c. There were no significant differences between the different types of spina bifida, in any of the subscales in the different age groups. The percentage of patients with lower scores (< P50) on HRQL was significantly higher in patients with MMC than in those with OTSB. Mean scores (SD) on HRQL were significantly lower in the MMC group compared to the OTSB, 165 (24.1) in MMC compared to 197.5 (19.8) in OTSB for children ≤ 12 years ($p < 0.001$), and 187.6 (18.9) in MMC compared to 200.7 (20.8) in OTSB for children > 12 years ($p = 0.01$) respectively.

Table 4 Patient numbers and percentages regarding 'functional limitations', 'disability', perceived competence and quality of life in patients with myelomenigocele (with and without hydrocephalus) compared to those with other types of spina bifida.

	MMC	OTSB		MMC HC+	MMC HC-	
	n = 122 (%)	n = 55 (%)	p-value	n = 97 (%)	n = 25 (%)	p-value
Ambulation level			< 0.001			0.007
- mv: too young (n=23)	19	4		16	3	
- normal	21 (20)	37 (73)		12 (15)	9 (41)	
- community	19 (18)	12 (24)		12 (15)	7 (32)	
- household	13 (13)	2 (4)		13 (16)	0	
- exercise	7 (7)	0		7 (9)	0	
- wheelchair	43 (42)	0		37 (45)	6 (27)	
Deviant PEDI-scores FS						
- self-care	72 (59)	17 (31)	< 0.001	60 (62)	12 (48)	0.20 ns
- mobility	93 (76)	15 (27)	< 0.001	79 (81)	14 (56)	0.004
- social function	53 (43)	11 (20)	0.002	45 (46)	8 (32)	0.19 ns
Deviant PEDI-scores CA						
- self-care	85 (70)	28 (51)	0.01	72 (74)	13 (52)	0.03
- mobility	67 (55)	7 (13)	< 0.001	58 (60)	9 (36)	0.02
- social function	32 (26)	4 (7)	0.003	28 (29)	4 (16)	0.19 ns
Perceived competence						
- mv: (n=72)	57	15		47	10	
(incl. too young n=58)						
- total score (< 2.5)	10 (15)	6 (15)	0.96 ns	7 (14)	3 (20)	0.69 ns
HRQL (below P50)	49 (63)	6 (15)	< 0.001	40 (66)	9 (53)	0.34 ns
- mv: too young (n=58)	44	14		36	8	

Abbreviations: MMC = myelomeningocele, OTBS = other types of spina bifida, HC+ = with shunted hydrocephalus, HC- = without hydrocephalus, mv = missing values, ns = not significant, PEDI = Pediatric Evaluation of Disability Inventory, FS = functional skills, CA = caregiver assistance, HRQL = health related quality of life.



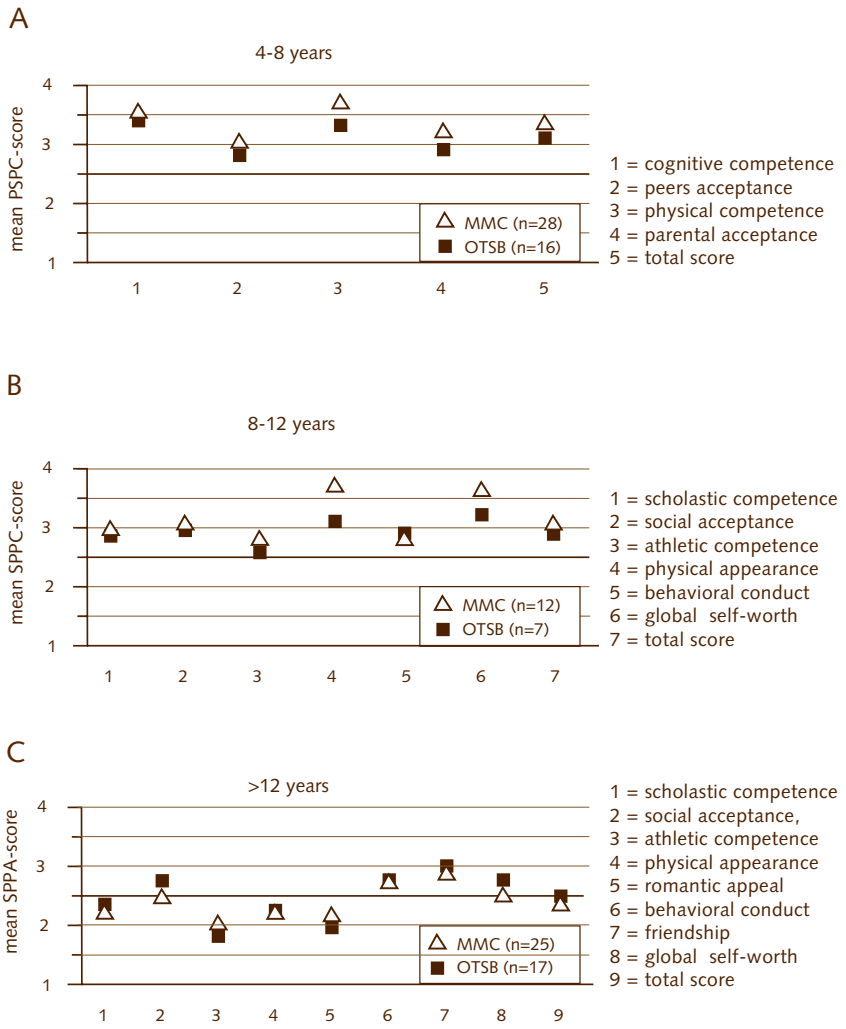


Figure 1a-c

Mean subscale-scores of perceived competence (PSPC-, SPPC-, SPPA- score) for children with myelomeningocele (MMC) compared to other types of spina bifida (OTSB) in three different age groups.

Abbreviations: PSPC = Pictorial Scale of Perceived Competence, SPPC= Self-Perception Profile for Children, SPPA = Self-Perception Profile for Adolescents

Associations within different domains of the disablement process

We found a significant correlation between lesion level and ambulatory status (r_s = 0.64, $p < 0.001$). In Figure 2 the percentage of patients in the MMC group and their ambulatory status at different lesion levels is presented.

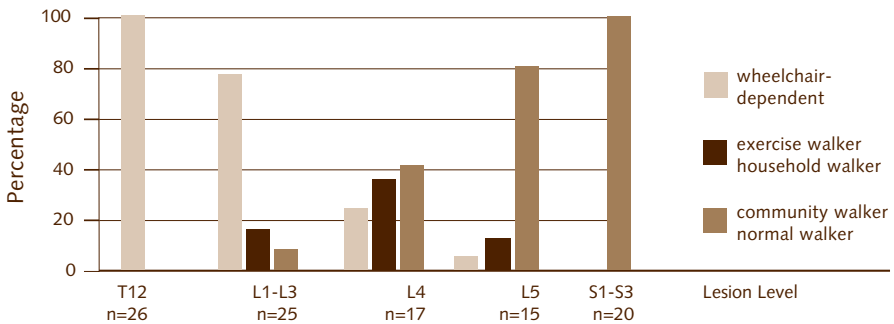


Figure 2

Percentage of patients with myelomeningocele (n = 103) and their ambulatory status at different motor levels

We also found a significant correlation between muscle strength and ambulatory status (r_s = 0.50-0.72, $p < 0.001$). All community walkers had muscle strength grade 5 of hip flexor, grade 4 to 5 of hip abductor and extensor muscles and ≥ grade 3 of ankle dorso-flexors.
Results regarding associations between perceived competence and mental status (IQ), type of school, ambulation level, PEDI-scores, and HRQL respectively, are shown in Table 5. A significant relation

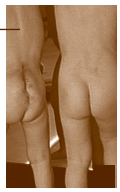


Table 5 Age specific correlations of perceived competence (total scores) with mental status (IQ), type of school, ambulation level, scores in the different domains of the PEDI, and HRQL-scores.

	Perceived competence TS		
	Numbers	Spearman's rho	p-value
IQ			
- 4-8 years	16	0.12	0.65 ns
- 8-12 years	22	0.03	0.94 ns
- > 12 years	44	-0.20	0.32 ns
Type of school			
- 4-8 years	44	0.02	0.91 ns
- 8-12 years	19	0.17	0.50 ns
- > 12 years	42	-0.22	0.15 ns
Ambulation level			
- 4-8 years	44	0.30	0.052 ns
- 8-12 years	19	0.15	0.53 ns
- > 12 years	42	- 0.02	0.92 ns
PEDI-scores (6 domains)			
- 4-8 years	28	Range -0.17 - 0.30	0.12 - 0.94 ns
- 8-12 years	18	Range -0.18 - 0.39	0.11 - 0.60 ns
- >12 years	42	Range -0.28 - 0.15	0.08 - 0.97 ns
HRQL			
- 4-8 years	42	0.27	0.08 ns
- 8-12 years	18	0.25	0.32 ns
- > 12 years	36	0.37	0.03

Abbreviations: TS = total score, IQ = intelligence quotient, ns = not significant, PEDI = Pediatric Evaluation of Disability Inventory, HRQL = health related quality of life.

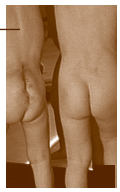
between perceived competence and HRQL was only found in children > 12 years of age. Ambulation level and HRQL in children 4 to 8 years showed a borderline significant association with perceived competence. With the different PEDI domains, associations did not reach the level of significance, although trends were seen in the age group 4-8 years for mobility (r_s = 0.30), in the age group 8-12 years for social function (r_s = 0.39), and in children > 12 years for social function (r_s = -0.28).

The results regarding associations between HRQL, and mental status (IQ), ambulation, and PEDI-scores respectively are shown in Table 6. HRQL appeared to be significantly associated with ambulatory status, functional abilities and mental status as well.

Table 6 Age specific correlations of 'health related quality of life' scores with mental status (IQ), ambulation level, and scores in the different domains of the PEDI.

		HRQL scores		
	Numbers	Pearson correlation	Spearman's rho	p-value
IQ				
- ≤ 12 years	38	0.71	-	< 0.001
- > 12 years	44	0.31	-	0.15 ns
Ambulation level				
- ≤ 12 years	76	-	0.60	< 0.001
- > 12 years	36	-	0.42	0.01
PEDI-scores (6 domains)				
- ≤ 12 years	95	Range 0.19 - 0.20	-	0.01 - 0.01
- > 12 years	36	Range 0.44 - 0.60	-	< 0.001 – 0.008

Abbreviations: HRQL = health related quality of life, IQ = intelligence quotient, ns = not significant. PEDI = Pediatric Evaluation of Disability Inventory.



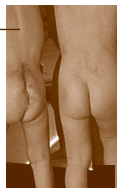
DISCUSSION

This study describes the problems that children with spina bifida encounter, with special emphasis on 'impairments', 'functional limitations', 'disability', perceived competence and HRQL. Our study suggests that children and adolescents with myelomeningocele, especially those with shunted hydrocephalus, appeared to be significantly more impaired, to have more 'functional limitations' and lower scores on health related quality of life, compared to patients with other types of spina bifida. However their perceived competence did not differ from those who had less 'impairments' and more functional abilities. HRQL appeared to be significantly associated with ambulation level, functional abilities and mental status, whereas perceived competence showed no associations with most of these variables.

Regarding the 'pathology', the percentage of hydrocephalus and Chiari II-malformation found in the MMC group, 80% and 77% respectively, is in agreement with current literature [3,4,23]. Moreover, it is an interesting finding that Chiari II malformation was also found in 60% (15/25) of patients with MMC without hydrocephalus. This might suggest that the Chiari II malformation is more a result of abnormal development of the central nervous system itself, rather than a result of the hydrodynamic theory, as described by Gardner [24]. He suggested that overproduction of cerebrospinal fluid causes the Chiari II malformation by pushing the cerebellar tonsils into the upper cervical spinal canal [24,25]. In problems related to the spinal cord, our findings are in agreement with current literature [5,6]. We found no significant differences in the presence of a long cord (conus below L2), or the presence of hydromyelia, in patients with MMC compared to those with OTSB. The presence of lipomas and retethering was significantly higher in the OTSB group.

With regard to mental status, we found that the IQs of children with MMC without hydrocephalus were comparable to those with OTSB, 96.2 and 98.8 respectively, whereas children in the MMC group with

shunted hydrocephalus had lower IQs (80.7). These findings are in agreement with Zurmöhle et al. [26] who also found IQs of children with MMC without hydrocephalus, to be within the normal ranges.
Over the last decade more attention has been paid to the self-concept of children with spina bifida [26-29]. Appelton et al. described that children with spina bifida have lower feelings of competence compared to able-bodied children [28]. As we were interested in possible differences in children with MMC and OTSB, we compared those groups and found no significant differences in perceived competence, in any of the subscales. These findings could be influenced by the fact that 46% of the patients with MMC attended special schools, and hence compare themselves with other physically disabled children. Moreover IQs were lower in the MMC group with shunted hydrocephalus. Minchom et al. [27] suggested that lower IQs would have a protective effect on global self worth. They studied the impact of severity of 'disability' on self-concept in 79 young patients with spina bifida, and found that "greater feelings of global self worth and of self esteem in physical appearance were associated with greater severity of disability". This association was partially attributed to the lower IQ scores in the more disabled children. Our data on perceived competence are partially in agreement with these findings. In children older than 12 years, an inverse association between perceived competence and mental status or school type was found (the higher the IQ or the higher the school level, the lower the score on perceived competence). Although the association did not reach the level of statistical significance, this tendency is of clinical relevance. In contrary to Minchom et al. [27] we found no significant associations between perceived competence and the amount of 'disability' (PEDI-scores in different domains). From the literature it is suggested that it is incorrect to assume that the psychological impact is less in mildly disabled young persons [9,27]. There is growing evidence that it might be displaced directing resources in relationship to severity of disability. Many mildly disabled children attend mainstream schools, where they might be at risk for psychosocial problems. This is supported by the studies of

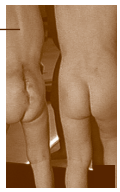


Appelton et al. [28,29] showing that physically disabled children in mainstream schools were in a difficult position, as they compared themselves with healthy peers, but at the same time they felt less competent and less accepted than matched able-bodied controls.

HRQL appeared to be related with perceived competence. In older children we found that the higher the score on perceived competence, the higher HRQL scores. In younger children the associations did not reach a significant level. This might be due to the small sample size in children aged between 8-12 years. HRQL was also found to be associated with ambulation level, and functional abilities (PEDI-scores). Patients with a greater amount of 'disability' showed lower scores on HRQL. This is in agreement with Padua et al. [9] who studied the relation between HRQL and 'disability' in 12 young patients with spina bifida (14-18 yrs). They found lower scores on the physical aspects of HRQL in the more disabled persons but unexpectedly for 'the mental aspects' of quality of life; less 'disability' was associated with higher psychological distress. With regard to the mental aspect, our data are not completely in agreement with Padua et al. [9], as we found that HRQL showed a positive correlation with mental status (the higher IQ, the higher HRQL) in younger children (< 12 yrs). Our findings might be biased by the fact that in younger children and in those with an IQ below 70, the parents completed the questionnaires. Although parents were instructed to respond from their child's viewpoint, rather than their own, the data might reflect the parent's expectations of their child's HRQL. In children older than 12 years, higher scores on IQ were not significantly associated with higher HRQL scores. This might be due to the fact that older children with higher IQs, might be more aware of their limitations than younger children with lower IQs. Moreover, children older than 12 years completed the HRQL questionnaire themselves. Therefore, the scores might reflect more of the patient's own perspective.

Our study has some shortcomings. We found HRQL to be significantly associated with ambulation level and the amount of 'disability' (PEDI-scores). However, it is important to find out if these compo-

nents are influenced by other factors. It is also necessary to find out which factors within the 'disablement process' might be influenced by physiotherapy in order to improve functional independence and quality of life. Therefore, a more detailed study on independent determinants of specific aspects of the PEDI and HRQL is already in process.
Our study is a descriptive and cross-sectional study. With the advanced medical care, more and more spina bifida patients grow into adulthood. Associations between 'disability', perceived competence and HRQL, found in children could change in time and might even be totally different in adulthood. Therefore long-term prospective follow-up studies will be needed to study the dynamics of the inter-relationship within the different 'disablement process' domains. This also addresses the issue that nowadays functional abilities and HRQL are measured in different ways around the world. Consensus on an international standard set of instruments, used for the evaluation of the 'disablement process' would be of great value.



CONCLUSIONS

Although patients with myelomeningocele were found to be more disabled and had lower scores on HRQL than those with other types of spina bifida, perceived competence was not better in the latter group. Therefore, health care professionals should not mainly focus their attention on myelomeningocele patients with severe disabilities, especially regarding psychosocial support. This kind of support might be needed in children with other types of spina bifida and minor disabilities as well.

ACKNOWLEDGEMENTS

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REFERENCES

1 Hunt GM, Poulton A. Open spina bifida: a complete cohort reviewed 25 years after closure. Dev Med Child Neurol 1995; 37(2):19-29.

2 Hunt GM, Oakeshott P, Kerry S. Link between the CFS shunt and achievement in adults with spina bifida. J Neurol Neurosurg Psychiatry 1999; 67(5):591-595.

3 McLone DG. Continuing concepts in the management of spina bifida. Pediatr Neurosurg 1992; 18(5-6):254-256.

4 Bowman RM, McLone DG, Grant JA, Tomita T, Ito JA. Spina bifida outcome: a -25 year prospective. Pediatr Neurosurg 2001; 34(3):114-120.

5 Pierre-Kahn A, Zerah M, Renier D, Cinalli G, Sainte-Rose C, Lellouch-Tubiana A, Brunelle F, Le Merrer M, Giudicelli Y, Pichon J, Kleinknecht B, Nataf F. Congenital lumbosacral lipomas. Child's Nerv Syst 1997; 13(6):298-335.

6 Cornette L, Verpoorten C, Lagae L, Calenbergh F van, Plets C, Vereecken R, Casaer. Tethered cord syndrome in occult spinal dysraphism; timing and outcome of surgical release. Neurology 1998; 50(6):1761-1765.

7 Verbrugge LM, Jette AM. The disablement process. Soc Sci Med 1994; 38(1):1-14.

8 ICF International Classification of Functioning, Disability and Health. Geneva: World Health Organization; 2001.

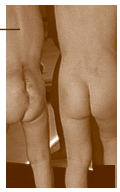
9 Padua L, Rendeli C, Rabini A, Girardi E, Tonali P, Salvaggio E. Health-related quality of life and disability in young patients with spina bifida. Arch Phys Med Rehabil 2002; 83(10):1384-1388.

10 Pit-Ten Cate IM, Kennedy C, Stevenson J. Disability and quality of life in spina bifda and hydrocephalus. Dev Med Child Neurol 2002; 44(5):317-322.

11 McDonald CM, Jaffe KM, Shurtleff DB, Melelaus MB. Modification to the traditional description of the neurosegmental innervation in myelomeningocele. Dev Med Child Neurol 1991; 33(6):473-481.

12 Bernbeck G. Kinderorthopädie. Stuttgart: Georg Thieme Verlag; 1988.

13 Spiegel TM, Spiegel JS, Paulus HE. The joint alignment and motion scale, a simple measure of joint deformity in patients with rheumatoid arthritis. J Rheumatol 1987; 14(5):887-892.



14 Hislop HJ, Montgomery JI. Daniel's and Worthingham's muscle testing; techniques of manual examination. 6th ed. Toronto (Can): WB Saunders Company; 1995.

15 McDonald CM, Jaffe KM, Shurtleff DB. Assessment of muscle strength in children with myelomeningocele; accuracy and stability of measurement over time. Archives of Phys Med Rehabil 1986; 67:855-861.

16 Hoffer M, Feiwell, Perry J, Bonnet C. Functional ambulation in patients with myelomeningocele. J Bone Joint Surg Am 1973; 55(1):137-148.

17 Hayley SM, Coster WJ, Ludlow LH, Haltiwanger JT, Andrellos PJ. 'Pediatric Evaluation of Disability Inventory' (PEDI). Development, standardization and administration manual. Boston, MA: New England Center Inc. and PEDI Research Group; 1992.

18 Custers JWH. Pediatric Evaluation of Disability Inventory, the Dutch adaptation [Dissertation]. Utrecht (the Netherlands): University Utrecht; 2001.

19 Van Dongen-Melman JEW, Koot HM, Verhulst FC. Cross-cultural validation of Harter's self-perception profile for children in a Dutch sample. Educ Psychol Measurement 1993; 53:739-53.

20 Straathof MAE, Treffers PHDA. Translation of the Dutch self-perception profile for adolescents. Oegstgeest (the Netherlands): Academic Center of Psychiatry for Children and Youth Curium;1988.

21 Veenhof C, Vermeer A. De platenschaal voor zelf-waargenomen competentie en sociale acceptatie bij kinderen met cerebrale parese: een onderzoek naar betrouwbaarheid en validiteit. Bewegen en hulpverlening 1997; 14:117-126.

22 Parkin PC, Kirpalani HM, Rosenbaum PL, Fehlings DL, Van Nie A, Willan AR, King D. Development of a health-related quality of life instrument for use in children with spina bifida. Qual Life Res 1997; 6(2):123-132.

23 Steinbock P, Irvine B, Cochrane DD, Irwin BJ. Long-term outcome and complications of children born with myelomeningocele. Child's Nerv Syst 1992; 8:92-96.

24 Gardner WJ. The dysraphic states of syringomyelia to anencephaly. Amsterdam (the Netherlands): Experta Medica, 1973; 1-201.

25 Bannister CM. The case for and against intrauterine surgery for myelomeningoceles. Eur J Obstet Gynecol Reprod Biol 2000; 92:109-113.

26 Zurmöhle UM, Homann T, Schroeter C, Rothgerber HR, Hommel G, Ermert JA. Psychosocial adjustment of children with spina bifida. J Child Neurol 1998; 13(2):64-70.

27 Minchom PE, Ellis NC, Appelton PL, Lawson V, Böll V, Jones P Eliott CE. Impact of severity on self-concept in young people with spina bifida. Arch Dis Child 1995; 73:48-52.

28 Appelton PL, Minchom PE, Ellis NC, Elliott CE, Böll V, Jones P. The self-concept of young people with spina bifida: a population based study. Dev Med Child Neurol 1994, 36(3):198-215.

29 Appelton PL, Ellis NC, Minchom PE, Lawson V, Böll V, Jones P. Depressive symptoms and self-concept in young people with spina bifida. J Pediatr Psychol 1997; 22(5): 707-722.

Determinants of functional independence and quality of life in children with spina bifida:

a physical therapy perspective.

3

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Submitted for publication

ABSTRACT

OBJECTIVES: To investigate determinants of functional independence and study which functional abilities were determinants for 'health related quality of life' (HRQL) in children with myelomeningocele.
DESIGN: Cross-sectional study by means of clinical assessment, 'disability' measurement, and questionnaires.
SETTING: Outpatient spina bifida clinic at a university hospital.
PARTICIPANTS: 122 children with myelomeningocele. Mean age 7.9; range 1-18 years.
MAIN OUTCOME MEASURES: Functional independence as measured by the 'Pediatric Evaluation of Disability Inventory' (PEDI), and quality life as measured by the 'Spina Bifida HRQL Questionaire'. Uni- and multivariate logistic regression models were used to investigate factors that were determinants for these outcomes. Results were expressed as odds ratios (OR) and 95% confidence intervals (CI).
RESULTS: Lesion level below L3 (OR: 0.4, CI: 0.1-1.0), mental status of IQ ≥ 80 (OR: 4.2, CI: 1.2-14.9), having no contractures in lower extremities (OR: 3.4, CI: 1.3-8.8), and having normal strength of knee extensor muscles (OR: 4.1, CI: 1.4-11.5), were most strongly associated with independence in self-care. Mental status (OR: 16.1, CI: 2.8-93.9), having no contractures in lower extremities (OR 1.5, CI: 1.4-5.3), and normal strength in knee extensors (OR: 11.0, CI: 1.3-97.0), were the most important determinants for independence in mobility. Concerning functional abilities, being independent with regard to mobility was the most important determinant for HRQL (OR: 5.3, CI: 1.6-17.4).
CONCLUSIONS: In children with myelomeningocele, good muscle strength, mental ability and being independent in mobility, appeared to be much more important for daily life function and quality of life than other medical indicators of the disorder. This information is of clinical significance in planning a comprehensive and realistic rehabilitation program.

INTRODUCTION

Spina bifida is a complex congenital disorder that represents a broad spectrum of neural tube defects, including spina bifida aperta and spina bifida occulta. The incidence varies in different parts of the world, but is generally 0.4-1.0 per 1000 live births in the USA [1], and a higher incidence is found in Northern Europe. In myelomeningocele (MMC), the most common and more serious form of spina bifida aperta, both brain and spinal cord are often malformed, resulting in hydrocephalus, Chiari II malformation, muscle weakness and lack of sensation in lower extremities, bladder and bowel incontinence, as well as cognitive dysfunction [2-5].

The functional consequences of spina bifida can be displayed using the disablement framework as described by Verbrugge and Jette [6], consisting of four different domains: 'pathology', 'impairments', 'functional limitations' and 'disability'. Several factors might influence functional outcome, such as intra-individual factors (coping, life style), extra-individual factors (medical care, rehabilitation) and risk factors. This model has a lot in common with the most recent International Classification of Functioning, Disability and Health (ICF) designed by the World Health Organization (WHO) [7].

Most studies focus on the relation between 'pathology' and outcome. Hydrocephalus, lesion level and presence of neural tissue in the sac, are considered important factors that influence the functional prognosis of patients with spina bifida [2-5,8,9]. Hunt et al. and Bowman et al. [2,3,5] reviewed complete cohorts of patients with myelomeningocele from birth into adulthood. These important studies reflect the results of treatment that was carried out in the sixties and seventies. As treatment strategies change with time, ongoing research is therefore extremely important.

Physical therapy is a common intervention for children with spina bifida. Traditionally, therapy sessions focussed on improvement of muscle strength, muscle tone, and prevention of contractures, to opti-

mize childhood development and functioning [10]. However, it is not clear how these impairments are related to functional independence and 'health related quality of life'. In various other chronic diseases, 'impairment' parameters do not significantly correlate with functional abilities and 'disability' parameters [11,12].

In this study, we investigate which factors of the 'disability process' domains are the most important determinants for functional independence regarding self-care and mobility in children with myelomeningocele. Regarding HRQL, our main interest was to find out which functional abilities were most important determinants, in order to find sufficient and realistic goals for physical therapy treatment.

METHODS

Patients

The study group consisted of children born between 1981-2000 (aged 1 to 18 years), who have been followed-up in the outpatient spina bifida clinic of the Wilhelmina Children's Hospital. Participants included those with a diagnosis of spina bifida aperta (myelomeningocele). Patients with meningocele and those with cervical (myelo)meningocele, encephalocele, or cord traumas were excluded. Non-Dutch speaking patients were also excluded. One hundred and thirty patients met the inclusion criteria, and 122 were willing to participate in the study (response rate 94%) carried out from January 1999 to September 2001. The Medical Ethics Committee of our hospital approved the protocol of this descriptive, cross-sectional study. Informed consent was obtained from the parents and from the patients themselves if they were older than 12 years of age.

Procedures

From the domain of 'pathology', the investigated determinants for functional independence on self-care and mobility were presence of shunted hydrocephalus, number of shunt revisions, and lesion level. From the 'impairment' domain, intelligence, contractures in lower extremities, muscle-tone, and muscle strength from upper and lower extremities were included. In order to find out which functions from the domain of functional abilities might influence the quality of life in children with myelomeningocele, we included ambulation level and hand function as well as independence in self-care, mobility and social function (caregiver assistance scores of the 'Pediatric Evaluation of Disability Inventory' (PEDI) [1,13,14].

Data were obtained by physical examination, and during a face-to-face interview regarding functional abilities (PEDI-scores) and HRQL. All measurements were performed by the same experienced pediatric physical therapist (MS). Parents were questioned about their educational level.

Measurements with regard to 'pathology'

Information on the presence of shunted hydrocephalus and the number of shunt revisions was obtained from the medical record. Determination of the neurosegmental motor level was based on muscle strength of the lower extremities as described by McDonald et al. [15]. Many different systems are used to classify neurological level in spina bifida patients, while agreement in levels from L3 and downwards is lacking [16]. For the purposes of the analyses, we therefore divided the neurological level into two groups: below L3, and from L3 up to thoracic.

Measurements with regard to 'impairments'

Information on mental status, such as Intelligence Quotients (IQs) (WISC-R) in children older than 4 years, was obtained from the psychological records. IQs ≥ 80 were considered as normal [2,3,5]. Body weight and body height were measured, crown-to-heel for ambulators and upper-arm span for non-ambulators.
Range of motion in lower extremities was measured in a standardized way with a two-legged 360-degrees goniometer, and compared to reference values for children [17]. The percentages of range of motion-loss were assessed on a 5-point scale according to Spiegel et al. [18]. Range of motion-loss of 5% or more in one or both extremities was defined as a contracture.

Muscle tone of upper extremities, trunk, and lower extremities was scored on a 5-point scale ranging from severe hypotonia (1), mild hypotonia (2), normal tone (3), mild hypertonia (4), to severe hypertonia (5) as described by Shurtleff [19]. Both mild or severe hypotonia and hypertonia were classified as an abnormal score.
Muscle strength of upper and lower extremities was graded 0-5, according to the standard Manual Muscle Testing (MMT) as described by Daniel's & Worthingham [20]. In children under the age of 5 years, muscle grading on a 6-point scale is not reliable. Therefore, in these patients strength was graded as absent, weak, or full strength as advocated by McDonald et al. [21]. In upper extremities, elbow flexors and hand muscles were tested. In lower extremities, flexors, extensors and abductors of the hip, as well as extensors of the knee and dorsal flexors of the ankle joint were tested, as these muscle groups seem to correlate strongly with ambulation level [15]. Children with full strength or grade 5 were classified as having normal muscle strength.

Measurements regarding functional abilities and quality of life

Ambulatory status was modified from Hoffer et al. [22]. We grouped patients into non-ambulators (completely wheelchair-dependent or walking in therapeutic situations) and functional-ambulators (household or community walkers).
In children older than 4 years, hand function was measured with the Movement Assessment Battery for Children (Movement-ABC) [23]. This instrument has been developed to evaluate gross and fine motor function in children aged 4-12^{+} years. Percentile scores of the child's motor abilities can be compared with a normal age-matched sample of children [23].
To evaluate the level of functional independence we used the caregiver assistance scores of the Dutch adapted version of the PEDI [24,25]. The PEDI evaluates function in terms of *capabilities* (functional skills) and *performance* of what the child actually does in response to the

environment (the amount of caregiver assistance required to accomplish daily tasks) in three domains: self-care, mobility and social function [24]. Reference values are provided for children between 0.5-7.5 years. Normal values are defined in the range of 2 SD (50 ± 20). In children > 7.5 years of age, results were calculated as a scaled score. In healthy children > 7.5 years of age, all functional skills should be mastered leading to a score of 100, which was considered to be normal [25]. We defined patients with normal caregiver-assistance scores as being functional independent. The intra-interviewer and inter-interviewer reliability of the adapted Dutch version of the PEDI are excellent. Intra-class Correlation (ICC) Coefficients were all but one above 0.90. The content validity and construct validity are acceptable [25].
Quality of life was measured with a Dutch-translation of the 'Spina Bifida HRQL Questionnaire' [26]. This instrument is a disease specific and age-related instrument for children (5-12 years) and adolescents (13-20 years) with spina bifida. For children, the questionnaire is used as a proxy report, whereas adolescents complete the questionnaire by themselves [26]. In a reference group of Canadian children with spina bifida, the mean score (SD) was 168 (24); for adolescents the mean score (SD) was 182 (30). Reproducibility was good (ICC = 0.78 for children; 0.96 for adolescents). Internal consistency was good (Cronbach's α = 0.93 for children, and 0.94 for adolescents). Construct validity correlations were 0.63 (children) and 0.37 (adolescents) [26]. The 'Spina Bifida HRQL questionnaire' is not yet cross-culturally adapted; therefore the median of our study population was used as cut-off, to determine patients with higher or lower level of quality of life.

Statistical analysis

Descriptive analysis (means, standard deviations; medians, P25-P75) was used to characterize functional independence (PEDI caregiver

assistance scores) and 'health related quality of life' (Spina Bifida HRQL scores).

To identify the most important independent factors associated with the defined outcome parameters (functional independence and HRQL) we first conducted univariate logistic regression analysis. We assessed whether the associations were independent of known confounders. Factors concerning functional independence were adjusted for age and body mass index (BMI). Factors concerning HRQL were adjusted for age, mental status and educational level of parents. With regard to strength of upper and lower extremities we first looked for the muscle group that was most strongly associated with independence in self-care and mobility. This muscle group was used for further analysis as an indicator muscle for lower and upper extremity strength respectively.

Next, in order to find out which was the most important determinant, we used multivariate logistic regression models with all statistically significant factors from the univariate analysis as independent variables, for each outcome parameter. Analysis was performed with the Statistical Package for Social Sciences (SPSS 9.0).

RESULTS

Patient characteristics are listed in Table 1.

Table 1 General data and clinical characteristics of 122 children with myelomeningocele.

		Number	
Age in years (SD)		7.9	(5.2)
Age distribution:	- 1-3.9 years (%)	39	(32)
	- 4-7.9 years (%)	25	(20)
	- 8-12.9 years (%)	33	(27)
	- 13-18.0 years (%)	25	(20)
males (%)		54	(44)
Body Mass Index in kg/m^2 (SD)		18.4	(4.6)
Educational level of parents:			
- bachelor or university level (%)		47	(38.6)
- lower than bachelor level (%)		75	(61.4)
Shunted hydrocephalus (%)		97	(80)
Shunt revisions:	- 0 (%)	40	(41)
	- 1 (%)	24	(25)
	- 2-4 (%)	25	(26)
	- more than 4 (%)	8	(8)
Lesion level			
- thoracic (%)	25	(20)	
- upper lumbar (L1-L3) (%)		26	(21)
- lower lumbar (L4 –L5) (%)		45	(37)
- sacral (%)		26	(21)

Table 1 continued.

	Number	
Mental status in children > 4 years [a]: mean IQ (SD)	83.3	(19.8)
Ambulatory status in children > 2.5 years[b]		
- non-ambulant (%)	53	(52)
- functional ambulant (%)	50	(48)
PEDI		
- deviant self care CA-score (%)	85	(69.6)
- deviant mobility CA-score (%)	67	(54.9)
- deviant social function CA-score (%)	32	(26.2)
≤ 7.5 years: - self care mean CA-score (SD)	30.7	(18.0)
- mobility mean CA-score (SD)	27.1	(15.7)
- social function mean CA-score (SD)	43.9	(12.6)
> 7.5 years: - self care mean CA-score (SD)	66.4	(20.5)
- mobility mean CA-score (SD)	88.9	(19.2)
- social function mean CA-score (SD)	86.1	(22.02)
Spina Bifida HRQL questionnaire in children > 5 years [c]		
- ≤ 12 years median score (25-75th centile)	166	(151-186)
- > 12 years median score (25-75th centile)	185	(176-198)

Abbreviations: SD = standard deviation, IQ = intelligence quotient, PEDI = Pediatric Evaluation of disability Inventory, CA = caregiver assistance, HRQL = health related quality of life.
a = number of valid values (n = 83); b: n =103, c: n = 73.

With regard to the muscle group most strongly associated with independence in self-care, we found hand muscle to contribute statistically significantly (OR: 4.4, CI: 1.5-36.2), whereas elbow flexor muscles did not (OR: 4.6, CI: 0.6-37.8). Independence in mobility was significantly associated with all lower extremity muscles groups: hip flexor muscles (OR: 4.7, CI: 1.7-13.1), hip abductor muscles (OR: 6.3, CI: 1.9-29.2), hip extensor muscles (OR: 4.9, CI: 1.4-17.0), and knee extensor muscles (OR: 4.1, CI: 1.4-11.5), and foot dorsal flexor muscles (OR: 5.5, CI: 12.2-14.2). Multivariate logistic regression modeling indicated that the odds ratio of knee extensor muscle strength remained significant (OR: 4.2, CI: 1.4-15.1), whereas the others did not. For further analyses, hand muscles and knee extensor muscles were used as indicator muscles for upper and lower-extremity muscle strength respectively.

Results regarding functional independence in self-care and mobility are shown in Table 2. The factors from the domains of 'pathology' and 'impairments', that are univariately associated with functional independence in self-care and mobility, after being adjusted for confounders (age, BMI), are shown in the middle column (univariate analysis). The results of analyses, aimed to find out which component from the different domains was independently associated with each of the two outcome parameters for functional independence are presented in the right column (multivariate analysis). Independence in self-care was statistically significantly associated with lesion level, having no contractures, mental status and muscle strength of lower extremities. From the multivariate logistic regression model, none of the odds ratios remained significant. Independence in mobility was significantly associated with hydrocephalus, lesion level, mental status, having no contractures, muscle tone above the cele level, and muscle strength of lower extremities. From the multivariate logistic regression model, the odds ratios of mental status, having no contractures, and muscle strength of lower extremities remained significant.

Table 2 Determinants for functional independence (= normal PEDI scores on caregiver assistance) regarding self-care and mobility.

	Univariate analysis	Multivariate analysis
	odds ratio (95% CI)	odds ratio (95% CI)
SELF-CARE		
Pathology		
- non shunted hydrocephalus	3.59 (0.23 – 10.49)	-
- less than two shunt revisions	1.14 (0.80 – 4.77)	-
- lesion level below L3	**0.37 (0.14 – 0.98)**	1.31 (0.23 – 7.31)
Impairments		
- mental status (IQ ≥ 80)	**4.23 (1.20 – 14.94)**	2.73 (0.75 – 9.57)
- no contractures lower extremities	**3.39 (1.31 – 8.75)**	2.43 (0.85 – 6.99)
- normal muscle tone above cele level	2.77 (0.94 – 8.15)	-
- normal strength of knee extensors muscles	**4.06 (1.44 – 11.46)**	3.62 (0.63 – 20.83)
- normal strength of hand muscles	4.39 (0.53 – 36.23)	-
MOBILITY		
Pathology		
- non shunted hydrocephalus	**2.76 (1.01 – 7.56)**	0.99 (0.20 – 5.00)
- less than two shunt revisions	1.36 (0.61 – 3.03)	-
- lesion level below L3	**0.12 (0.04 – 0.31)**	0.34 (0.05 – 2.61)
Impairments		
- mental status (IQ ≥ 80)	**16.89 (4.24 – 67.28)**	**16.09 (2.76 – 93.93)**
- no contractures lower extremities	**3.66 (1.48 – 7.66)**	**1.45 (1.40 – 5.32)**
- normal muscle tone above cele level	**8.27 (2.73 – 25.06)**	0.32 (0.05 – 1.98)
- normal strength of knee extensors muscles	**13.37 (4.63 – 38.67)**	**10.99 (1.25 – 96.97)**
- normal strength of hand muscles	35.06 (0.00 – 59.72)	-

All values that are statistically significant are indicated in bold.

Abbreviations: CI = confidence interval, IQ = intelligence quotient, PEDI = Pediatric Evaluation of Disability Inventory

Results regarding quality of life are shown in Table 3. Factors that remained significant after being adjusted for age, mental status, and educational level of the parents are shown in the column of univariate analysis. HRQL was significantly associated with being functional ambulant (household or community walker), and being independent with regard to mobility. From the multivariate logistic regression model, the odds ratio of being independent in mobility remained significant.

Table 3 Functional abilities associated with 'health related quality of life' (HRQL).

	Univariate analysis	Multivariate analysis
	odds ratio (95% CI)	odds ratio (95% CI)
Functional abilities		
- functional ambulant	**4.52 (1.01 – 20.15)**	2.17 (0.76 – 6.22)
- normal hand function	7.29 (0.29 – 179.77)	-
- independent in self care (normal CA-scores)	0.25 (0.33 – 1.90)	-
- independent in mobility (normal CA-scores)	**3.61 (1.33 – 40.13)**	**5.26 (1.59 – 17.41)**
- independent in social function (normal CA-scores)	3.34 (0.47 – 23.79)	-

All values that are statistically significant are indicated in bold.

Abbreviations: CI = confidence interval, CA = caregiver assistance.

DISCUSSION

From our study it is suggested that lesion level, mental status, contractures, and muscle strength of lower extremities particularly determine independence in self-care. All these factors are mutually dependent. Mental status, having no contractures, and muscle strength of lower extremities were the most important determinants for functional independence in mobility. With regard to HRQL, being independent in mobility seemed to contribute more to HRQL, than other functional abilities such as being independent in self-care or being wheelchair-dependent.

Knowledge of determinants of independence is of utmost importance for treatment in order to "focus on realistic goals rather than wasting efforts on attempting the impossible" [2]. The relation between 'pathology' (hydrocephalus, Chiari II malformation, lesion level) and outcome in patients with myelomeningocele has been studied extensively [2-5,8], but few has been published on the impact of 'impairments' and 'functional limitations'. Dahl et. al. [27] studied self-care skills in 35 young children with myelomeningocele. They found many of these children to be slow in the development of independence in self-care; 60% needed moderate or maximal caregiver assistance. Poor self-care skills were related to both the child's function above and below the level of the cele. Low intelligence, hypotonia above the level of the cele, being non-ambulant and poor executive function seemed to be significant risk factors for poor self-care skills. These results are partially in agreement with our data. We found poor self-care skills in 69% of our patients. We also found mental status to be an important determinant for independence in self-care, but muscle tone above the level was not. Nevertheless, motor function below the cele level appeared to be also important for complete independence in self-care.

With regard to mobility we found that variables in the 'pathology' domain such as the presence of shunted hydrocephalus, the number of shunt revisions and lesion level seemed to be of less importance.

Several researchers have published information on the relation between lesion level, muscle strength and ambulatory status [22,28,29]. The level of neurological lesion and associated strength of lower extremity muscles are the most important factors influencing ambulatory status. However, there is a large discrepancy in ambulatory outcomes across different lesion groups. McDonald et al. [29] studied the relationship between patterns of strength and ambulation in 291 children with myelomeningocele. They reported that m. iliopsoas strength was found to be the best predictor of ambulation with the m. quadriceps, m. tibialis anterior, and mm. glutei also contributing significantly. In our study the same muscle groups, particularly knee extensors, appeared to be significantly associated with independence in mobility, including transfers, indoor and outdoor ambulation, and climbing stairs. This information is of clinical relevance for rehabilitation interventions. We agree with McDonald et al. [29] when they recommend the use of specific patterns of lower-extremity muscle strength, rather than lesion level, to predict walking abilities. We found lesion level to be of less importance, than muscle strength of lower extremities with regard to independence in mobility.

Our finding of contractures being an important determinant for mobility is in agreement with findings of other authors [28,30]. It is questionable to what extent contractures can be prevented or treated by physical therapy. Often, surgical procedures are needed to restore the congenital muscle imbalance. Further research is needed to investigate whether physical therapy programs designed to increase power of unaffected muscles, lead to functional improvement [31]. In future research it is important to identify relevant strength thresholds for specific functional activities in order to create realistic rehabilitation programs.

Regarding HRQL, our main interest was to find out which functional abilities were most important determinants for HRQL, in order to find sufficient and realistic goals for physical therapy treatment. From current literature there is growing evidence that physical functioning of patients with spina bifida, is related to HRQL [1,32,33]. Pit-Ten Cate

et al. [32] showed that children with spina bifida had lower scores on quality of life with regard to self-care, incontinence and mobility, compared to children with hydrocephalus alone. Lesion level, type of spina bifida, and presence of hydrocephalus itself were not significantly related to quality of life. McCormick et al. [13] studied the impact of caring for a child with spina bifida on family function. The impact on the family was related less to the clinical diagnosis, than to the child's functioning at home. We found that being independent in mobility seemed to contribute more to HRQL, than other functional abilities such as being independent in self-care or being wheelchair-dependent.

Our study has some limitations. Regarding HRQL, we focussed on 'disability' aspects only, but from psychosocial literature it is suggested that personal and environmental factors might be of great importance [13,14,32]. Kirpalani et al. [1] studied the influence of parental hope on HRQL in children and adolescents with spina bifida. Although physical functioning explained a significant proportion of variance in HRQL, parental hope explained an additional 19 to 24% of the variance. The study of Pit-Ten Cate et al. [32] also confirmed that other factors than disability are associated with quality of life. They found that severity of the condition and family resources independently predicted quality of life. Therefore, it is extremely difficult for health professionals to predict future health related quality of life and furthermore it is always from the outsider's perspective. We agree with Kirpalani et al. [1] that patients and families are the best judges of their own quality of life. For future research, a close collaboration between behavioral and medical researchers is needed, as it will assist in the search for better ways to prevent disability [31].

When interpreting our findings it should be considered that in children with spina bifida, physical and intellectual disabilities often become more manifest when these children grow older. Realities in adulthood might be totally different from childhood [2]. Therefore, long-term follow-up remains important.

Our observations, that being independent in mobility appeared to contribute more to HRQL than being wheelchair-dependent, should be kept in mind when planning a realistic rehabilitation program regarding ambulation. Strategies to persist walking abilities with aids is a common goal in physical therapy, but the use of these aids should never disadvantage the patient in terms of independence.

CONCLUSIONS

Our findings showed that parameters on the level of 'impairments' were significantly associated with 'disability'. We found that good muscle strength, having no contractures, and mental ability appeared to be much more important for daily life function of spina bifida patients than other medical indicators of disorder. Being independent in mobility appeared to contribute more to HRQL, than being independent in self-care or being wheelchair-dependent.

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REFERENCES

1 Kirpalani HM, Parkin PC, Willan AR, Fehlings DL, Rosenbaum PL, King D, Van Nie AJ. Quality of life in spina bifida: importance of parental hope. Arch Dis Child 2000; 83(4):293-297.

2 Hunt GM, Poulton A. Open spina bifida: a complete cohort reviewed 25 years after closure. Dev Med Child Neurol 1995; 37(1):19-29.

3 Hunt GM, Oakeshott P, Kerry S. Link between the CFS shunt and achievement in adults with spina bifida. J Neurol Neurosur Psychiatry 1999; 67(5):591-595.

4 McLone DG. Continuing concepts in the management of spina bifida. Pediatr Neurosurg 1992; 18(5-6):254-256.

5 Bowman RM, McLone DG, Grant JA, Tomita T, Ito JA. Spina bifida outcome: a - 25 year prospective. Pediatr Neurosurg 2001; 34(3):114-120.

6 Verbrugge LM, Jette AM. The disablement process. Soc Sci Med 1994; 38(1):1-14.

7 ICF International Classification of Functioning, Disability and Health. Geneva: World Health Organization; 2001.

8 Date I, Yagyu Y, Asari S, Ohmoto T. Long-term outcome in surgically treated spina bifida cystica. Surg Neurol 1993; 40(6):471-475.

9 Steinbock P, Irvine B, Cochrane DD, Irwin BJ. Long-term outcome and complications of children born with meningomyelocele. Child's Nerv Syst 1992; 8:92-96.

10 Ryan KD, Ploski C, Emans JB. Myelodysplasia- the musculoskeletal problem: habilitation from infancy to adulthood. Phys Ther 1991; 71(12):935-945.

11 Net J van der, Prakken ABJ, Helders PJM, Berge M ten, Herwaarden M van, Sinnema G, Wilde EJ de, Kuis W. Correlates of disablement in polyarticular juvenile chronic arthritis; a cross sectional study. Br J Rheumatol 1996; 35(1):91-100.

12 Engelbert RHH, Graaf Y van der, Empelen R van, Beemer FA, Helders PJM. Osteogenesis imperfecta in childhood: impairment and disability. Pediatrics 1997; 99(2):1-7.

13 McCormick MC, Charney EB, Stemmler MM. Assessing the impact of a child with spina bifida on the family. Dev Med Child Neurol 1986; 28(1):53-61.

14 Lie HR, Börjeson MC, Lagerkvist B, Rasmussen F, Hagelsteen JH, Lagergren J. Children with myelomeningocele: the impact of disability on family dynamics and social conditions. A Nordic study. Dev Med Child Neurol 1994; 36(11):1000-1009.

15 McDonald CM, Jaffe KM, Shurtleff DB, Melelaus MB. Modification to the traditional description of the neurosegmental innervation in myelomeningocele. Dev Med Child Neurol 1991; 33(6):473-481.

16 Bartonek A, Saraste H, Knutson LM. Comparison of different systems to classify the neurlogical level of the lesion in patients with myelomeningocele. Dev Med Child Neurol 1999; 41(12):796-805.

17 Bernbeck G. Kinderorthopädie. Stuttgart: Georg Thieme Verlag; 1988.

18 Spiegel TM, Spiegel JS, Paulus HE. The joint alignment and motion scale, a simple measure of joint deformity in patients with rheumatoid arthritis. J Rheumatol 1987; 14(5):887-892.

19 Shurtleff DB. Computer data bases for pediatric disability. Clinical and research applications. Phys Med Rehab Clin North Am 1991;2(4):655-87.

20 Hislop HJ, Montgomery JI. Daniel's and Worthingham's muscle testing; techniques of manual examination. 6th edition Toronto (Can): WB Saunders Company; 1995.

21 McDonald CM, Jaffe KM, Shurtleff DB. Assessment of muscle strength in children with myelomeningocele; accuracy and stability of measurement over time. Arch Phys Med Rehab 1986; 67(12):855-861.

22 Hoffer M, Feiwell, Perry J, Bonnet C. Functional ambulation in patients with myelomeningocele. J Bone Joint Surg Am 1973; 55(1):137-148.

23 Henderson SE, Sugden DA. Movement Assessment Battery for Children. United Kingdom, Psychological Corporation; 1992.

24 Hayley SM, Coster WJ, Ludlow LH, Haltiwanger JT, Andrellos PJ. 'Pediatric Evaluation of Disability Inventory' (PEDI). Development, standardization and administration manual. Boston MA, New England Central Center Inc. and PEDI Research Group; 1992.

25 Custers JWH. Pediatric Evaluation of Disability Inventory, the Dutch adaptation [Dissertation]. Utrecht (the Netherlands): University Utrecht; 2001.

26 Parkin PC, Kirpalani HM, Rosenbaum PL, Fehlings DL, Van Nie A, Willan AR, King D. Development of a health-related quality of life instrument for use in children with spina bifida. Qual Life Res 1997; 6(2):123-132.

27 Dahl M, Ahlsten G, Butler A, Norrlin S, Strinnholm M, Winberg A. Self-care skills in young children with myelomeningocele. Eur J Pediatr Surg 2000; 10 (Suppl 1):52-53.

28 Asher M, Olson J. Factors affecting the ambulatory status of patients with spina bifida cystica. J Bone Joint Surg 1983; 65(3):350-356.

29 McDonald CM, Jaffe KM, Mosca VS, Shurtleff DB. Ambulatory outcome of children with myelomeningocele: effect of lower-extremity muscle strength. Dev Med Child Neurol 1991; 33(6):482-490.

30 Liopis ID, Munoz MB, Agullo EM, Martinez AL, Aymerich VG, Valero JVF. Ambulation in patients with myelomeningocele: a study of 1500 patients. Paraplegia 1993; 31:28-32.

31 Jette AM, Assmann SF, Rooks D, Harris BA, Crawford S. Interrelationships among disablement concepts. J Gerontol A Biol Sci Med Sci 1998; 53(5):395-404.

32 Pit-Ten Cate IM, Kennedy C, Stevenson J. Disability and quality of life in spina bifda and hydrocephalus. Dev Med Child Neurol 2002; 44(5):317-322.

33 Padua L, Rendeli C, Rabini A, Girardi E, Tonali P, Salvaggio E. Health-related quality of life and disability in young patients with spina bifida. Arch Phys Med Rehabil 2002; 83(10):1384-1388.

Spina bifida at the sacral level:

more than minor gait disturbances.

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ABSTRACT

OBJECTIVE: To investigate functional outcome in two groups of children with sacral level paralysis: myelomeningocele (MMC) versus lipomyelomeningocele (LMMC). Additionally both groups were compared to each other and when possible with reference values.
DESIGN: Cross-sectional study by means of (1) clinical assessment, and (2) disability measurement.
SETTING: Spina bifida outpatient clinic at a university hospital in the Netherlands.
SUBJECTS: Sample of 30 children with MMC, and 14 with LMMC. Mean age (SD) 6.0 (4.9) and 8.4 (4.9) years respectively.
MAIN MEASURES: Muscle strength, ambulation level, motor performance (BSID and Movement- ABC), and the 'Pediatric Evaluation of Disability Inventory' (PEDI).
RESULTS: The majority of patients in both groups were normal ambulant, 14/21 (67%) in MMC and 9/14 (64%) in LMMC. Ambulation was strongly associated with muscle strength of hip abductors (odds ratio [OR]: 13.5, 95% confidence interval [CI] 2.5-73.7), and ankle dorsal-flexor muscles (OR: 110, CI: 8.9-135.9). No significant differences where found in lesion and ambulation level. Muscle strength and motor performance were significantly lower in the MMC group than in the LMMC group ($p < 0.05$). PEDI-scores were comparable in both groups. Most problems were noted in mobility skills and caregiver assistance in self-care, especially regarding bladder and bowel management.
CONCLUSIONS: Gross motor and functional problems were seen in both groups. The MMC group showed more muscle weakness and motor problems. However, in both groups caregiver assistance was needed for a prolonged period, especially regarding bladder and bowel management. These findings need special attention, particularly in children who attend regular schools.

INTRODUCTION

Approximately 20% of all myelomeningoceles occur at the sacral level. The majority of patients with sacral level lesions are community walkers, and supposed to have fewer complications compared to high level lesions [1]. Therefore health care professionals tend to focus their research attention on the more disabled patients with spina bifida.

Over the last decade, few studies have reported on problems in patients with sacral level paralysis [1,2]. It has been suggested that infants with myelomeningocele at the sacral level have an early gross motor developmental delay, associated with abnormalities in postural control. It is not clear to what extent this delay can be attributed to the spinal cord lesion, or to abnormalities of the brainstem or cerebellum secondary to hydrocephalus and Chiari II malformation, often being present in patients with myelomeningocele [2].

It has also been reported that patients with sacral level lesion often have a decline in ambulatory skills in the long-term [1,3]. These problems are seen in patients with lipomyelomeningocele as well as patients with myelomeningocele. In contrast to patients with myelomeningocele, there is only involvement of the spinal cord in those with lipomyelomeningocele. The cord is often elongated and tethered due to intradural lipomas in the lumbosacral region [4,5].

It has been suggested that children with sacral level paralysis might be more impaired psychosocially. Not only do they have a difficulty fitting in with their healthy peers, but they do not fit in with more severely disabled children either [6].

Research data are scarce about functional outcome in children with sacral level spina bifida. This information is important for planning comprehensive care for these children. Therefore, the aim of this study was to investigate muscle strength, ambulation level, motor performance, and functional outcome regarding self-care, mobility and social function, in two groups of children with sacral level spina bifida. We compared the outcome of children with myelomeningocele

(MMC), who all had hydrocephalus and Chiari II malformation, to those with lipomyelomeningocele (LMMC), without these central nervous system abnormalities. In addition, each group was compared with reference values of the normal population regarding motor performance and functional outcome.

METHODS

Patients

The study group consisted of children diagnosed with MMC or LMMC, who were followed-up in the outpatient spina bifida clinic of the Wilhelmina Children's Hospital. Participants included those with paralysis from the S1 level or below, aged between 0-18 years. Non-Dutch speaking patients were excluded. Forty-four patients met the inclusion criteria, and participated in the study, carried out between January 1999 to January 2001. The Research and Medical Ethics Committee of our hospital approved the protocol of this descriptive, cross-sectional study. Informed consent was obtained from all the parents.

Methods

Data were obtained by physical examination, and during a face to face structured interview with regard to functional abilities. All measurements were performed by the same experienced paediatric physiotherapist (MS). Data concerning the presence of shunted hydrocephalus, the presence of Chiari II malformation, mental status and lesion level, were obtained from the medical records. Muscle strength, ambulation level, motor performance, and functional abilities were measured, and compared to reference values of the normal population.

Muscle strength of lower extremities was graded 0-5, according to the standard manual muscle testing (MMT) as described by Daniel's & Worthingham [7]. Inter-tester reliability is poor with coefficients ranging from 0.11-0.58. Intra-tester reliability ranges from 0.65 to 0.93 for different muscles groups, and from 0.80 to 0.99 for different muscle strength grades. Grades in the gravity eliminated position (< 3) have

the highest reliability values [8]. MMT appears to be less specific for scores ≥ 4 [9]. Muscle grading on a 6-point scale is less reliable in children under the age of five years. Therefore, in these patients strength was graded as absent, weak, and full strength, as advocated by McDonald et al. [10]. The following muscles were tested: flexors, -extensors and abductors of the hip, knee extensors and dorsal- and plantar-flexors of the ankle joint.

Ambulation level was defined according to Hoffer et al. [11] and documented in children older than 2.5 years of age. The scoring was adapted for patients with completely normal ambulatory skills. In Hoffer's description, patients with normal ambulatory skills (walking without crutches or braces) are not distinguished from community walkers (walking with or without crutches or braces and may use a wheelchair for long distances). This difference was considered clinically important, and we described patients who walked without the use of aids as 'normal ambulant'.

Motor development in infants was measured with the Dutch version of the Bayley Scales of Infant Development (BSID) [12]. The motor scale of the BSID allowed measurement of the acquisition of specific gross motor, fine motor and adaptive skills. Normal values are provided for infants from 2 to 30 months of age, and defined in the range of 2 SD (100 ± 32). Gross and fine motor performance in children from four years of age was measured with the Movement Assessment Battery for Children (Movement-ABC) [13].This instrument has been developed to evaluate gross and fine motor function in children aged 4-12^{+} years. A score below the 5th centile indicates that the child has significant movement difficulties. The test-retest reliability is 0.75 and the inter-rater reliability ranges from 0.70 to 0.89. The concurrent validity is 0.53 ($p < 0.0001$) [12].

Functional abilities were measured with the Dutch adapted version of the 'Pediatric Evaluation of Disability Inventory' [14,15]. The PEDI is based on a structured interview with parents. It evaluates function in terms of both capabilities (*functional skills*) and performance of what the child actually does in response to the environment (the amount of

caregiver assistance required to accomplish daily tasks) in three domains: self-care (including bladder and bowel management), mobility and social function. Reference values are provided for children between 0.5-7.5 years. Normal values are defined in the range of 2 SD (50 ± 20). In children > 7.5 years of age results were calculated on a scaled score. In healthy children > 7.5 years of age, all functional skills should be mastered leading to a score of 100, which was considered to be normal. The intra-interviewer and inter-interviewer reliability of the adapted Dutch version of the PEDI are excellent. Intra-class Correlation Coefficients (ICC) were all but one, above 0.90. The content validity and construct validity are acceptable [15].

Statistical analysis

Descriptive analysis (means, standard deviations) was performed for comparison of the scores with the normal population. The Mann-Whitney test for independent samples ($\alpha < 0.05$) and the Chi-square test were used to analyse the differences between patients with MMC and those with LMMC.
Using a logistic regression model we assessed the association between ambulatory status as dependent variable and muscle strength of lower extremities as independent variables. Analysis was performed with the Statistical Package of the Social Sciences (SPSS 9.0).

RESULTS

General characteristics are presented in Table 1. We found no significant difference in age, gender, mental status, lesion level or ambulation level between the MMC and the LMMC group. Both, hydrocephalus and Chiari II malformation were present in all patients with MMC, and in none of the patients with LMMC. The majority of patients in both groups were completely normal ambulant, 14/21 (67%) in the MMC group and 9/14 (64%) in the LMMC group.

The results regarding muscle strength of the lower extremities are shown in Table 2. The mean strength of hip extensor muscles and calf muscles in patients with MMC were found to be below grade four. The strength of these muscles was significantly lower in the MMC group than in the LMMC group. No significant differences were measured between the two groups in the other lower extremity muscle groups.

Normal ambulatory skills were only significantly associated with normal muscle strength hip abductors and ankle dorsal-flexors (odds ratio [OR]: 13.5, 95% confidence interval [CI] 2.5-73.7 and OR: 110, CI: 8.9-135.9 respectively). Multivariate logistic modelling indicated that these muscle groups were dependent of each other.

Results with regard to motor performance are presented in Table 3. The motor scale of the BSID was performed on 16 children. Eight children with MMC showed a significant delay in motor development. The mean score on the BSID was significantly lower in patients with MMC with hydrocephalus and Chiari II malformation than in those with LMMC without these abnormalities ($p = 0.007$). In six patients (three in each group), aged between 2.5-4 years, scores on motor performance were missing due to lack of normative values. In the remaining 22 children, the Movement-ABC was performed. Deviant scores on this test were seen in 9/14 (64%) of the MMC group compared to 2/8 (25%) of the LMMC group. The mean score on the Movement-ABC was significantly lower in the MMC group than in the

Table 1 Patient characteristics in two groups of children with sacral level paralysis (MMC versus LMMC).

	MMC n = 30	LMMC n = 14
Mean age in years (SD)	6.0 (4.9)	8.4 (4.9)
- range in years	1-17	1-17
Gender		
- male : female (n)	14:16	8:6
Shunted hydrocephalus (n)	22	0
Chiari II malformation (n)	22	0
Mental status[a]		
- mean IQ (SD)	93.8 (13.0)	99.8 (7.7)
Sacral lesion		
- symmetrically (n)	24	4
- asymmetrically (n)	5	7
- no loss (n)	1	3
Ambulation level		
- normal ambulant (n)	14	9
- community ambulant (n)	4	4
- household ambulant (n)	2	1
- non ambulant (n)	1	0
- too young to be ambulant (< 2.5 years) (n)	9	0

a = valid values (children ≥ 4 years of age): MMC n = 14, LMMC n = 8.

Abbreviations: MMC = myelomeningocele, LMMC = lipomyelomeningocele, SD = standard deviation, IQ = intelligence quotient.

Table 2 Lower-extremity muscle strength in children with MMC versus LMMC.

Muscle groups	MMC HC+ n=30	LMMC HC- n=14	P- value
Mean muscle strength (SD)			
Hip flexor muscles	4.9 (0.5)	4.9 (0.3)	0.98
Hip abductor muscles	4.5 (0.9)	4.6 (0.7)	0.59
Hip extensor muscles	3.5 (1.5)	4.4 (1.1)	0.01*
Knee extensor muscles	4.9 (0.5)	5.0 (0.0)	0.50
Ankle dorso-flexor muscles	4.4 (1.2)	4.1 (1.6)	0.49
Calf muscles	2.9 (1.5)	4.0 (1.6)	0.01*

* significant differences between MMC and LMMC: $p < 0.05$

Abbreviations : MMC = myelomeningocele, LMMC = lipomyelomeningocele, HC+ = with shunted hydrocephalus, HC- = without hydrocephalus, SD = standard deviation.

LMMC group ($p = 0.004$). For both motor performance tests, the scores of the LMMC group were below the mean but within the normal ranges, whereas the scores of the MMC group were deviant on the BSID, and borderline on the Movement-ABC. Problems with fine motor function were only seen in the MMC group with shunted hydrocephalus and Chiari II malformation: in 5/27 (19%). Children who were old enough to ambulate (n = 35), showed problems with items concerning static and dynamic balance, such as standing on one leg, jumping, and hopping. This was seen in both groups: 21/23

Table 3 Motor performance (BSID and Movement-ABC) in children with MMC versus LMMC.

	MMC HC+ (n=27)	LMMC HC- (n=11)
BSID		
- number of cases	13	3
- number of children with deviant scores (- 2 SD)	8	-
- mean score (SD)	61.7 (22.1)	84.3 (11.2)*
Movement-ABC		
- number of cases	14	8
- number of children with deviant scores (< P5)	9	2
- M-ABC mean total centile score (SD)	5.8 (7.2)	41.7 (37.6)*

Scores below the normal ranges are indicated in bold.
*significant differences between MMC and LMMC: $p < 0.05$

Abbreviations: BSID = Bayley Scale of Infant Development, Movement-ABC = Movement-Assessment Battery for Children, MMC = myelomeningocele, LMMC = lipomyelomeningocele, HC+ = with shunted hydrocephalus, , HC- = without hydrocephalus, SD = standard deviation.

(90%) in the MMC group compared to 7/12 (58%) in the LMMC group.

Functional ability results are shown in Table 4. Deviant scores on functional skills (FS) were most frequently seen in the mobility domain for both groups: 15/30 (50%) in the MMC group, and 6/14 (43%) in the LMMC group. Regarding caregiver assistance (CA), in both groups most problems were seen in the self-care domain: 14/30 (47%) in the MMC group, and 5/14 (36%) in the LMMC group.

Table 4 Functional abilities (PEDI-scores) in children with MMC versus LMMC.

	MMC HC+		LMMC HC-	
PEDI				
- number of cases	30		14	
- number (%) of patients with deviant scores on:				
self-care FS	11	(37)	3	(21)
mobility FS	15	(50)	6	(43)
social function FS	7	(23)	2	(13)
self-care CA	14	(47)	5	(36)
mobility CA	9	(30)	2	(13)
social function CA	4	(13)	-	
Mean NS (SD) ≤ 7.5 years				
- number of cases	20		7	
- self-care FS	40.2	(14.5)	41.3	(9.1)
- mobility FS	**28.7**	**(15.5)**	41.8	(11.6)
- social function FS	42.9	(10.7)	46.2	(9.8)
- self-care CA	64.0	(16.2)	69.9	(15.3)
- mobility CA	36.0	(13.9)	44.9	(15.4)
- social function CA	46.2	(8.9)	52.8	(16.1)
Mean SS (SD) > 7.5 years				
- number of cases	10		7	
- self-care FS	**84.6**	**(12.3)**	**93.1**	**(9.8)**
- mobility FS	**89.1**	**(13.5)**	**89.7**	**(12.2)**
- social function FS	**93.4**	**(8.5)**	**98.9**	**(1.8)**
- self-care CA	**76.2**	**(14.8)**	**88.0**	**(15.5)**
- mobility CA	**91.6**	**(11.6)**	**97.5**	**(6.6)**
- social function CA	**93.3**	**(10.1)**	100.0	(0.0)

Abbreviations: MMC = myelomeningocele, LMMC = lipomyelomeningocele, HC+ = with shunted hydrocephalus, HC- = without hydrocephalus, PEDI = Pediatric Evaluation of Disability Inventory, SD = standard deviation, FS = functional skills, CA = caregiver assistance, NSS = normative standard scores, SS = scaled scores.

Scores below the normal ranges are indicated in bold.

In the mean PEDI-scores (Table 4) no significant differences were found between the MMC group with hydrocephalus and Chiari II malformation, and the LMMC group without these abnormalities. In children ≤ 7.5 years of age, most of the scores were below the mean standard scores found in non-disabled children, but still within the normal range, with the exception of mobility score in the MMC group. Almost all scores were deviant in children > 7.5 years of age. The lowest scores were found in self-care CA, mainly concerning items on bladder and bowel management in both groups.

In this study-cohort, 33 patients were on Clean Intermittent Catheterisation (CIC) (27 in the MMC group, and 6 in the LMMC group). Nine of them were completely independent, without the need of supervision. Their mean age (SD) was 12.7 (3.6) years. Fifteen patients used colon enemas for faecal continence (9 in the MMC group, and 6 in the LMMC group). Five of them were completely independent, with a mean age (SD) of 14 (3.7) years.

DISCUSSION

Our main interest was to investigate and compare functional outcome in two groups of children with sacral level paralysis: myelomeningocele versus lipo-myelomeningocele. Children with (lipo)myelomeningocele at the sacral level appeared to have significant gross motor difficulties, mainly concerning balance, and needed caregiver assistance for a prolonged period, especially regarding bladder and bowel management. Muscle strength and motor performance were significantly lower in patients with myelomeningocele and hydrocephalus with Chiari II malformation, than in patients with lipomyelomeningocele without these brain abnormalities, but their ambulation level and PEDI-scores were not significantly different. Moreover, fine motor problems were only seen in the MMC group.

It has been suggested that 90 to 100% of patients with a sacral level lesion are community ambulant [1,11,16,17]. Our findings are in agreement with these data, as we found 31/35 (89%) of the patients to be community or normal walkers. Moreover, we found that patients with normal strength in hip abductors and ankle dorsal-flexors had a higher chance of ambulating normally without aids or braces, than those with weakness of these muscles. These findings are in agreement with McDonald et al. [18], who also reported on the relationship between muscle strength and ambulation. They found grade four to five strength of gluteal and tibialis anterior muscles to be associated with community ambulation. In our descriptive study, we used MMT for the evaluation of muscle strength groups in children with sacral level paralysis. Surprisingly, we found grade four or more for most muscle groups. In retrospect, the use of hand-held myometry might have given more detailed information on the amount of muscle weakness when compared to reference values of the normal population [9].

Wolf and McLaughlin [2] studied early motor development in seven infants with sacral level paralysis. They found a significant motor delay in these children, increasing with age. Performance is at -1 SD at six months and at -2.5 SD at 18 months of age. It remained unclear to what extent the delay should be attributed to the spinal lesion or to the abnormalities of the central nervous system, such as hydrocephalus and Chiari II malformation, which were present in all their patients. In our study, we included patients with hydrocephalus and Chiari II malformation (MMC), as well as those without these central nervous system abnormalities (LMMC) (Table 1). Although the lesion levels were comparable in both patient groups, motor performance was significantly lower in patients with myelomeningocele, compared to those with lipomyelomeningocele. This might suggest that the presence of hydrocephalus or Chiari II malformation might be more responsible for the delay than the spinal lesion itself. Although we included more infants than Wolf and McLaughlin [2], 16 compared to 7, the number of patients in our study is still small for both patient groups. Therefore, larger studies will be needed to test this hypothesis.

Moreover, in children older than four years of age, we found motor performance in the myelomeningocele group to be borderline, whereas motor performance in the lipomyelomenigocele group appeared to be within the normal range. Nevertheless, balance problems, as measured with items from the BSID and Movement-ABC, such as hopping into squares, jumping, and standing on one leg, were seen in more than half of them (7/12) compared to 21/23 (90%) in the myelomeningocele group. This might interfere with regular sporting activities, which in our experience are often limited. Whether there is a causal link between muscle strength, static and dynamic balance problems and its relation to limitations in regular sporting activities needs further investigation.

Tsai et al. [19] studied functional skills in children with spina bifida using the PEDI. Scores were below the mean standard scores found in non-disabled children. Deviant scores were only seen in the mobility

domain. They found lower scores in the mobility and social function domain in children with myelomeningocele compared to those with lipomyelomeningocele. Our data are partially in agreement with their findings. Functional skills were found to be most deviant in the mobility domain for both groups (15/30, 50% and 6/14, 43% respectively). Even though lower scores were found in patients with myelomeningocele compared to those with lipomyelomeningocele, the differences were not statistically significant. This might be due to the fact that Tsai et al. [19] included patients with higher lesion levels (thoracic and lumbar lesions). In their study, the neurological level was significantly higher in myelomeningocele patients than in those with lipomyelomeningocele. In our study, the mean IQ (SD) score of myelomeningocele patients, was significantly higher compared to those in the study of Tsai et al. [19], 93.8 (13.0), and 78 (23.2) respectively. This might also explain why we did not find any significant differences in PEDI-scores between patients with myelomeningocele and those with lipomyelomeningocele. Tsai et al. [19] did not report on the amount of caregiver assistance needed for daily activities. We found most deviant scores in the domain of self-care, mainly concerning bladder and bowel management. Only nine patients in this cohort were independent regarding their CIC or colon enemas. Most patients needed caregiver assistance for a prolonged period.

Our study has intrinsic limitations, especially regarding motor performance. The assessment of motor performance has been conducted with two different instruments due to age difference, leading to a small sample size in the different subgroups. Therefore, these results should be interpreted with caution, and limits the ability to generalise them to all children with (lipo)myelomeningocele at the sacral level. A larger number of patients with less age difference, and a control group are needed to address this issue. Despite these limitations, our finding might be additional to other sparse studies that have been reported on patients with sacral level paralysis [1,2].

In conclusion, the children with sacral level paralysis in this study had more than minor gait disturbances. Gross motor and functional prob-

lems, were seen in both groups. The myelomeningocele group showed more muscle weakness and motor problems than the lipomyelomeningocele group, but their functional abilities did not differ significantly.

CLINICAL MESSAGE

- Most children with sacral level paralysis appeared to have gross motor problems, more severe in myelomeningocele than in lipomyelomeningocele.
- Both groups needed caregiver assistance for a prolonged period, especially regarding bladder and bowel management.
- Periodic monitoring is warranted, so that appropriate intervention can be prescribed when needed.

REFERENCES

1 Brinker MR, Rosenfield SR, Feiwell E, Granger SP, Mitchell DC, Rice JC. Myelomeningocele at the sacral level. Long-term outcome in adults. J Bone and Joint Surg 1994; 76(9):1293-1300.

2 Wolf LS, McLaughlin JF. Early motor development in infants with meningomyelocele. Pediatr Phys Ther 1992; 4:12-16.

3 Ong LC, Lim YN, Sofiah A. Malaysian children with spina bifida: relationship between functional outcome and level of lesion. Singapore Med J 2002; 43(1):012-017.

4 Pierre-Kahn A, Zerah M, Renier D, Cinalli G, Sainte-Rose C, Lellouch-Tubiana A, Brunelle F, Le Merrer M, Giudicelli Y, Pichon J, Kleinknecht B, Nataf F. Congenital lumbosacral lipomas. Child's Nerv Syst 1997; 13(6):298-335.

5 Calenbergh F van, Vanvolsem S, Verpoorten C, Lagae L, Casear P, Plets C. Results after surgery for lumbosacral lipomas: the significance of early and late worsening. Child's Nerv Syst 1999; 15(9):439-443.

6 Holmbeck GN, Faier-Routbaum J. Spinal lesion level, shunt status, family relationships, and psychosocial adjustment in children and adolescents with spina bifida myelomeningocele. J Pediatr Psychol 1995; 20(6):817-832.

7 Hislop HJ, Montgomery JI. Daniel's and Worthingham's muscle testing; techniques of manual examination. 6th edition Toronto (Can): WB Saunders Company; 1995.

8 Florence JM, Pandya S, King WM, Robinson JD, Baty J, Milller JP, Schierbecker J, Signore LC. Intrarater reliability of manual muscle test (medical research council scale) grades in Duchenne's muscular dystrophy. Phys Ther 1992; 72(2):115-126.

9 Schwarz S, Cohen ME, Herbison GJ, Shah A. Relationship between two measures of upper extremity strength: manual muscle test compared to hand-held myometry. Arch Phys Med Rehabil 1992; 73:1063-1068.

10 McDonald CM, Jaffe KM, Shurtleff DB. Assessment of muscle strength in children with myelomeningocele; accuracy and stability of measurement over time. Arch Phys Med Rehab 1986; 67(12):855-861.

11 Hoffer M, Feiwell, Perry J, Bonnet C. Functional ambulation in patients with myelomeningocele. J Bone Joint Surg Am1973; 55(1):137-148.

12 Meulen BF van der, Smrkovski M. 'Bayley Ontwikkelingsschaal', Dutch translated and adapted version of the Bayley Scales of Infant Development. Lisse (the Netherlands): Swets & Zeitlinger; 1983.

13 Henderson SE, Sugden DA. Movement Assessment Battery for Children. United Kingdom, Psychological Corporation; 1992.

14 Hayley SM, Coster WJ, Ludlow LH, Haltiwanger JT, Andrellos PJ. 'Pediatric Evaluation of Disability Inventory' (PEDI). Development, standardization and administration manual. Boston MA, New England Central Center Inc. and PEDI Research Group; 1992.

15 Custers JWH: Pediatric Evaluation of Disability Inventory, the Dutch adaptation. [Dissertation], Utrecht (the Netherlands): University Utrecht; 2001.

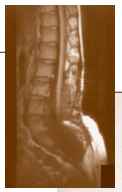
16 Asher N, Olson J. Factors affecting the ambulatory status of patients with spina bifida cystica. J Bone and Joint Surg 1983; 65(3):350-356.

17 Findley TW, Argre JC, Habeck RV, Schmalz R, Birkebak RR, McNally MC. Ambulation in the adolescent with myelomeningocele. I: Early childhood predictors. Arch Phys Med and Rehab 1987; 68(8):512-522.

18 McDonald CM, Jaffe KM, Mosca VS, Shurtleff DB. Ambulatory outcome of children with myelomeningocele: effect of lower-extremity muscle strength. Dev Med Child Neurol 1991; 33(6):482-490.

19 Tsai PY, Yang TF, Chan RC, Huang PH, Wong TT. Functional investigation in children with spina bifida - measured by the Pediatric Evaluation of Disability Inventory (PEDI). Child's Nerv Syst 2002; 18:48-53.

Long-term outcome of neurosurgical untethering on neurosegmental motor level and ambulation level.



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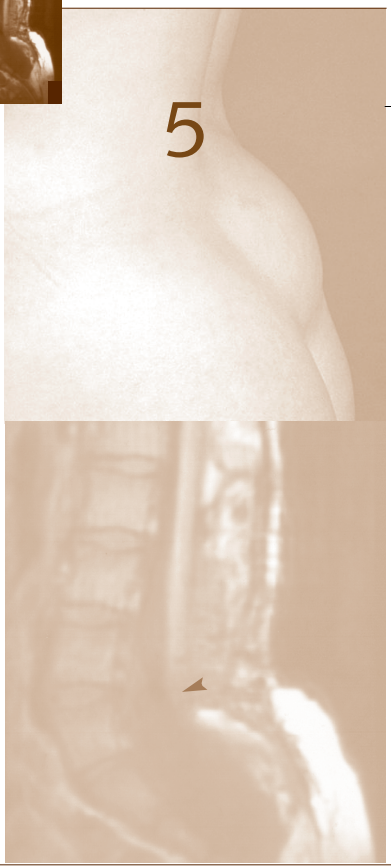


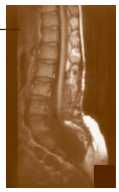
ABSTRACT

AIMS: To determine long-term outcome of neurosurgical untethering on neurosegmental motor level and ambulation level in children with tethered spinal cord syndrome.

METHODS: Forty-four children were operated (17 males, 27 females; mean age at operation 6 years and two months, SD 5 years). Sixteen patients had myelomeningocele, nine had lipomyelomeningocele, and 19 had other types of spinal dysraphism. Motor level and ambulation level were assessed before surgery and three times after (mean duration of follow-up 7 years and 1 month, SD 1 year 8 months).

RESULTS: Deterioration of motor level was seen in five of 44 patients, 36 of 44 remained stable, while improvement was seen in three of 44. Deterioration of ambulation level was seen in five of 44 patients, and remained stable in 26 of 44. Thirteen children of 44 were too young to ambulate at time of operation (< 2 years and 6 months). Late deterioration of motor- or ambulation level was only seen in (lipo)myelomeningocele patients. Deterioration of ambulatory status was strongly associated with obesity and retethering. Revision of the initial tethered cord release was performed in nine of 44 patients, mainly in patients with lipomyelomeningocele.

CONCLUSIONS: The results show that late deterioration and retethering occurred in a minority of patients, mainly in lipomyelomeningocele patients.



INTRODUCTION

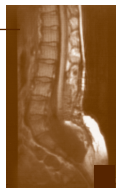
Tethered spinal cord syndrome is a descriptive term used to indicate progressive neurological deficits in individuals with limited movement of the spinal cord, usually at the caudal end of the spinal canal. In these individuals, the cord is abnormally fastened to immovable structures, such as lipoma, vertebra, dura, or skin. This limitation in movement stretches the spinal cord and compromises blood supply. Ischemic damage to the neural tissue is thought to be the main cause of neurological deterioration [12].

With the introduction of MRI it became less difficult to recognize different forms of congenital and acquired spinal cord lesions as causes of neurological deterioration. So-called primary tethered spinal cord syndrome is seen in those with sacral agenesis, tight filum, and in those with occult spinal dysraphism such as lipomyelomeningocele, intradural lipoma, diastematomyelia, and dermal sinus. Secondary tethered spinal cord syndrome includes those with spinal cord tethering due to retethering of the initial closure of a myelomeningocele or surgical corrections of other primary spinal dysraphic lesions [12].

Symptoms of tethered spinal cord syndrome can occur at any time in the development of the child, therefore careful monitoring of urological, neurological, and musculoskeletal status is necessary to determine the onset of symptoms [4,6,12,14]. Many studies have suggested that neurosurgical untethering can stop neurological deterioration [2,6,10] but mostly report on overall outcome. Few studies focus on evaluation of individual symptoms or group of symptoms, rather than overall outcome [4,14].

One of the major complaints of patients or parents/carers is change in muscle activity and progressive gait disturbances [11]. Therefore, in our prospective study, we focused on these functional aspects in our prospective study on the long-term outcome of 44 individuals who underwent neurosurgical correction of their tethered spinal cord. The emphasis of this study was on neurosegmental motor level and ambu-

lation level. In addition, we tried to gain insight into risk factors such as deterioration of ambulation level and in the occurrence of retethering.



METHOD

Participants

From January 1990 to December 1994, 73 patients underwent neurosurgical treatment for tethered cord syndrome at the University Medical Center Utrecht, the Netherlands. All patients were referred directly to the department of neurosurgery, by neurologists, or pediatricians, because of problems suggestive of tethered cord. Of these patients, 44 (17 males and 27 females) were younger than 18 years of age and are the subject of this study. Diagnosis, symptoms, and signs of these patients are detailed in Table 1. Informed consent was obtained from their parents and also from the children if they were older than 12 years of age. The Medical Ethics Committee of our hospital approved the protocol of this prospective study.

Measurements

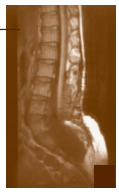
Sex, type of spinal dysraphism, presence of shunted hydrocephalus, MRI findings, age at operation, and age at long-term follow-up were documented. Body weight, and body length were also measured, crown-to-heel for ambulators and upper-arm span for non-ambulators. Reference values for body weight compared to length were used to determine obesity that was present when the patients' weight was above the 98th centile.

Determination of the neurosegmental motor level was based on Manual Muscle Testing (MMT) according to Daniel's and Worthingham [7] of the lower extremities (muscles that were capable of an antigravity voluntary contraction were scored grade 3 or more). For identification of the motor level, we used the modification of traditional description of neurosegmental innervation of lower limb muscles as described by McDonald et al. [11].

Ambulation level was scored according to the criteria as described by Hoffer et al. [8]. We adapted the scoring for patients with normal ambulatory skills. Ambulation level was described as non-ambulant (wheelchair-dependent), exercise ambulant (walking only in therapeutic situations), household ambulant (using braces or crutches for indoors and using wheelchair outdoors), community ambulant (ambulating outdoors with or without braces but using a wheelchair for longer distances). In Hoffer's description, patients with complete normal ambulatory skills are not distinguished from community walkers. We considered this an important issue to distinguish, and described them as 'normal ambulant' (complete function without aids).
Neurosegmental motor level and ambulation level were assessed and evaluated prospectively one day before surgery, and after surgery (6 weeks, 6 months, and long-term follow-up ranging from 4 to 9 years). All measurements were performed by two well-trained and experienced pediatric physical therapists.
Surgical techniques for release of the tethered cord were: (1) removal of excessive lipomatous tissue; (2) resection of the midline bony- or cartilaginous spur in cases of split cord malformation; (3) transsection of the filum and (4) a duroplasty. All untethering procedures were performed using standard microsurgical techniques. Intrinsic lipomas were debulked using the microscope-mounted CO2-laser. All initial surgical releases were performed by the same, experienced pediatric neurosurgeon.
Diagnosis of retethering was defined as having more than one tethered cord release. In our study, repeated surgery was performed on clinical grounds if new current symptoms related to the spinal cord dysfunction developed in the patients.

Statistical analysis

We hypothesized that age at long-term follow-up, obesity, and the occurrence of retethering could be significant risk factors in the dete-



rioration of ambulation level. We used univariate logistic regression models to test these hypotheses. The same techniques were used to analyze the occurrence of retethering as a dependent variable, and the different forms of spinal dysraphism and age at operation as independent variables. Analysis was performed using the SPSS (version 9.0).

RESULTS

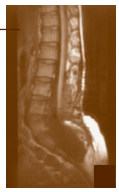
Mean age at operation was 6 years and 2 months (SD 5 years). Four patients were younger than 5 years of age at the time of operation, 38 were aged between 5 to 10 years, and two patients were older than 10 years at time of surgery. Mean age at long term follow-up was 13 years and 7 months (SD 5 years and 6 months). The duration of long-term follow-up ranged from 4 years and 1 month to 9 years and 5 months (mean 7 and 1 month, SD 1 year and 8 months).

The different types of spinal dysraphism are shown in Table 1, as well as the initial symptoms. Hydrocephalus was present in all patients (Table 1), with myelomeningocele (n = 16): eleven of these had shunts inserted. Within the myelomeningocele group, high level lesions (above L4) were seen in five of 16 patiens, low lumbar lesions in six of 16, and lesions at the sacral level in two of 16 patients. Three patients

Table 1 Diagnosis and initial complaints at time of referral in 44 patients with tethered cord syndrome.

Diagnosis	Pain	Progressive neurological loss	Progressive orthopedic deformities	Progressive urological problems	Combination	Profylactic TCR
Meningomyelocele (n=16)	2	3	2	3	6	-
Lipomyelomeningocele (n=9)	-	-	2	3	4	-
Other types of spinal dysraphism						
- Filum terminale lipoma (n=8)	1	-	2	1	3	1
- Tight filum terminale (n=4)	3	-	-	-	1	-
- Split cord malformation (n=2)	-	-	1	-	1	-
- Sacrum dysgenesis (n=4)	1	-	1	1	1	-
- Meningocystocele (n=1)	-	-	-	-	-	1

Abbreviations: TCR = tethered cord release



had no loss of lower-extremity muscle strength. Forty-two patients underwent neurosurgical untethering because they presented with signs of a symptomatic tethered cord, such as pain or deterioration of the existing urinary or neurological status. Two patients had asymptomatic tethered cord: one was operated for mid-cervical meningocele, which proved to be a meningomyelocystocele, the other patient had a lumbosacral dimple in the midline and lipomatous filum terminale. In these patients prophylactic untethering was carried out in order to prevent expected clinical deterioration. At long-term follow-up, all these patients were still asymptomatic.

The immediate postoperative complications we observed, were leakage of cerebrospinal fluid through the wound (six patients), and superficial wound infection (one patient).

Data concerning MRI findings in the different types of spinal dysraphism are shown in Table 2. Nine of 44 patients had a normal position of the conus, above the second lumbar vertebra (L2) [15]. Their cord was tethered due to lipoma, fatty tissue in the fila, or bony struc

Table 2 MRI findings of 44 children who underwent neurosurgical untethering.

Diagnosis	MRI findings		
	Conus at normal level	Presence of lipomas	Syrinx
Meningomyelocele (n=16)	-	4	1
Lipomyelomeningocele (n=9)	-	9	1
Other types of spinal dysraphism			
- Filum terminale lipoma (n=8)	2	8	1
- Tight filum terminale (n=4)	4	-	1
- Split cord malformation (n=2)	1	-	-
- Sacrum dysgenesis (n=4)	1	2	1
- Meningocystocele (n=1)	1	-	1

tures. In 23 of 44 of the patients, lipomas were present. Syringomyelia was seen in five patients and it was small in all cases. Four patients had both lipoma and syringomyelia.

Data concerning neurosegmental motor level before and ≥ 4 years after surgical intervention are presented in Table 3. In none of the patients did neurosegmental motor level change 6 weeks or 6 months after surgery compared to the preoperative situation. Late deterioration of neurosegmental motor level (≥ 4 years after surgical intervention) was seen in five patients; two of them had lipomyelomeningocele and three had myelomeningocele with intradural lipomas. Improvement of neurosegmental motor level was seen in three children, all of them had myelomeningocele. In 36 of 44 of the children, neurosegmental motor level remained stable during the entire follow-up period.

Data concerning pre- and late postoperative ambulation level (≥ 4 years after surgical intervention) are listed in Table 4. In all patients, 6 weeks to 6 months after surgery, ambulation level remained stable compared with the preoperative situation. Late deterioration of the ambulatory status was seen in five patients; three of them had lipomyelomeningocele and two myelomeningocele with intradural lipomas. In 26 of the 44 patients ambulation remained stable during the entire follow-up period. Thirteen children were too young to ambulate at time of operation (< 2 years and six months). In the long-term, four of the patients became community ambulant, they all had lipomyelomeningocele. In two of these children, ambulation level was normal half a year after operation (at the age of 2 years and 6 months), but it deteriorated in the long-term and they became community ambulators. All other children younger than 2 years and 6 months of age (n = 9) at time of operation, developed normal walking abilities that remained stable during the entire follow-up.

Obesity and retethering were strongly associated with deterioration of ambulatory status, whereas age at long-term follow-up had no statistically significant influence (Table 5).

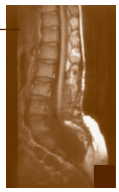


Table 3 Motor level before and ≥ 4 years after neurosurgical untethering in patients with myelomeningocele, ipomyelomeningocele and other types of spinal dysraphism.

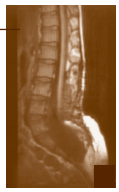
	Number	Motor level Pre-surgery	Motor level Post-surgery
Myelomeningocele			
Deterioration (n=3)	1	L1 – L3	T12
	1	L5 – S1	L1 – L3
	1	L5 – S1	L4 – L5
Stabilization (n=10)	2	≥ T12	=
	2	L1 – L3	=
	2	L5 – S1	=
	1	<S2	=
	3	no loss	=
Improvement (n=3)	2	L5 – S1	< S2
	1	< S1	no loss
Lipomyelomeningocele			
Deterioration (n=2)	2	L5 – S1	L4 – L5
Stabilization (n=7)	1	L4 – L5	=
	1	L5 – S1	=
	1	< S1	=
	4	no loss	=
Other types of spinal dysraphism			
Stabilization (n=19)			
Filum terminale lipoma	1	L5 – S1	=
	1	< S1	=
	6	no loss	=
Tight filum terminale	4	no loss	=
Split cord malformation	2	no loss	=
Sacrum dysgenesis	1	L4 – L5	=
	1	S1 – S2	=
	2	no loss	=
Meningocystocele	1	no loss	=

Abbreviations: = : pre surgery level is not different from post surgery level

Table 4 Ambulation level before and ≥ 4 years after neurosurgical untethering in patients with myelomeningocele, lipomyelomeningocele and other types of spinal dysraphism.

	Number	Ambulation level Pre-surgery	Ambulation level Post-surgery
Myelomeningocele (n=16)			
Deterioration (n=2)			
	1	household walker	therapeutic walker
	1	community walker	household walker
Stabilization (n=14)	4	wheelchair-dependent	=
	1	household walker	=
	9	normal walker	=
Lipomyelomeningocele (n=9)			
Deterioration (n=3)	3	normal walker	community walker
Stabilization (n=1)	1	normal walker	=
	1	*too young*	normal walker
	4	*too young*	community walker
Other types of spinal dysraphism (n=19)			
Stabilization (n=11)			
Filum terminale lipoma	2	community walker	=
	5	normal walker	=
	1	*too young*	normal walker
Tight filum terminale	4	normal walker	=
Split cord malformation	2	*too young*	normal walker
Sacrum dysgenesis	4	*too young*	normal walker
Meningocystocele	1	*too young*	normal walker

Abbreviations: = : pre surgery level is not different from post surgery level



During long-term follow-up (ranging from 4 to 9 years), 44 children underwent a total of 57 untethering operations. Thirty-five patients had a single untethering procedure. Revision of the initial tethered cord release was seen in nine of 44 of the patients. Five patients had lipomyelomeningocele, three had myelomeningocele, and one had filum terminale lipoma. In the patients with lipomyelomeningocele, two patients required three releases, and one patient underwent four releases. All these patients presented with recurrent acute back pain, combined with progressive foot deformities due to progressive neurological loss. The other six patients had two releases. The second release was carried out 1 year and six months to 6 years after the initial untethering. The presence of lipomas (17 of 44), and age at operation were not significantly associated with the occurrence of retethering (see Table 5).

Table 5 Risk factors in deterioration of ambulatory status and in the occurrence of retethering.

	Odds Ratio	95% Confidence Interval
Deterioration of ambulatory status		
- age at long-term follow-up	0.96	0.8 - 1.1
- obesity	**16.7**	**2.1 - 133.0**
- occurrence of retethering	**20.6**	**2.9 - 143.6**
Occurrence of retethering		
- age at operation	0.91	0.77-1.1
- presence of lipomas	4.2	0.75-22.9

All significant values are indicated in bold

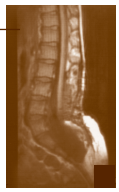
DISCUSSION

This prospective long-term follow-up study shows that after neurosurgical untethering, late deterioration (≥ 4 years after surgery) of neurosegmental motor level and ambulatory status occurred mainly in patients with lipomyelomeningocele or myelomeningocele. Obesity and retethering were strongly associated with the deterioration of ambulation level. In this study retethering was mostly seen in patients with lipomyelomeningocele (five of nine), although the presence of lipomas itself was not a statistically significant factor.

We did not study the changes in bladder function, because this series included a smaller group of patients, whose lower urinary tract and sexual function were previously reviewed and reported by Boemers et al. [3]. They concluded that changes in bladder-sphincter function after untethering are usually transient and the result of partial denervation of the lower urinary tract. This postoperative denervation was reversible and lasted for < 6 months. It only occurred in patients with (lipo)myelomeningocele. Therefore, this group seemed to distinct from patients with other forms of spinal dysraphism.

Traditionally, the tethered cord syndrome has been associated with an 'abnormally low-lying conus', but tethering of the spinal cord can occur at any level, and can be caused by different structures [13]. Our study included nine of 44 patients with the conus in the normal position. Their cord was tethered due to lipoma, fatty tissue in the fila, or bony structures. Seven of them developed symptomatic tethered cord. This is in agreement with Warder and Oakes [16], who found tethering of the spinal cord in 13 out of 73 patients with their conus in a normal position on MRI. All of them had lipoma or fatty tissue in fila, and two patients had split-cord malformation. Ten of 13 patients developed neurological symptoms.

Comparison of postoperative outcome with other studies is difficult, because, as in our study, most studies include different forms of spinal dysraphism. Sarwark et al. [15] retrospectively investigated the



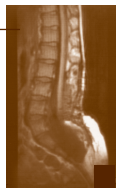
results of surgical untethering in 30 children with myelomeningocele. In 14 of these patients there was a decreased muscle function or change in motor level before surgery. None of these patients deteriorated in long-term follow-up after surgery. Eleven patients showed improvement and three of 14 remained stable. This is not in agreement with our data. We included several forms of spinal dysraphism but saw deterioration of motor level in three of 16 patients with myelomeningocele. In 10 of them motor level remained stable while it improved in three patients with myelomeningocele. The number of patients who improved as reported by Sarwark et al. [15] might be biased by the fact that their study included very young children (1 year of age) and their age-appropriate development of motor function might be recorded as improvement. Calenbergh et al. [4] reported long-term outcome after surgery in 32 patients with lipomas. Preoperative progression of symptoms that were mostly seen were motor deficits (15 of 32), urodynamic changes (14 of 32), and orthopedic deformities (13 of 32). In their long-term follow-up, motor problems improved or stabilized in 27 of 32 patients and it worsened in five of 32. In our study with a mean duration of follow-up of 7 years and 1 month, neurosegmental motor level and ambulation level deteriorated in five of 23 of patients with lipomas, and in five of the total group of 44, whereas the others remained stable or improved.

Late neurological deterioration is often reported, especially in patients with lumbosacral lipomas [1,4,5,9,12,14]. This phenomenon is frequently attributed to the occurrence of retethering, but several authors argue that it might also be part of the natural history of lumbosacral lipomas or myelodysplasia. Pierre-Kahn et al. [14] studied 291 patients with congenital lumbosacral lipomas. They distinguished lipomas of the filum from lipomas of the conus because the long-term outcome was much less favorable for the latter group than for lipomas of the filum. They observed that postoperative long-term deterioration was a risk only in patients with lipomas of the conus and in patients with myelomeningocele, and was never seen in inividuals with lipomas of the filum. As in the large series of Pierre-Kahn et al. [14], we also

found that late deterioration only occurred in patients with lipomyelomeningocele and myelomeningocele.

The incidence of retethering varies from 10 to 20 % in current literature, and is more frequently reported in patients with lipomyelomeningocele or transitional lipomas, than in patients with dorsal, caudal or filum lipomas [1,5]. In the first group satisfactory untethering surgery can be done without risk, but in those with lipomyelomeningocele and transitional lipomas, the treatment has certain risks and does not prevent all patients from further deterioration [1,14]. In our study, nine of the 44 patients showed signs of retethering, which is comparable to the incidence in current literature. The diagnosis of retethering is a very difficult one. The goal for surgery is not only to completely release the tethered cord, but also to obtain a watertight dural closure as to prevent retethering through scarring. Nevertheless, postoperative MRI often shows that the spinal cord has retethered again. This radiological retethering was never an indication for repeated surgery, in our study. However, if new symptoms related to the spinal cord dysfunction developed in the patients, repeated surgery was performed on clinical and neurophysiological grounds and based on urodynamic investigation. Retethering mainly occurred in those with complex forms of lipomas. This might explain why the presence of lipomas itself (23 of 44), is not significantly associated with the occurrence of retethering, in this study.

In the past, there were few barriers to carrying out neurosurgical untethering. In our series, prophylactic operations were performed in two patients with occult forms of spinal dysraphism, who had normal neurological function. In these patients no deterioration occurred regarding motor- and ambulation level, but it remains unclear whether or not, this can be attributed to the operation. In current literature, and from our own experience it has become clear that complete untethering of the cord can be done savely and straightforwardly in many cases, but on the other hand, sometimes it can be very difficult and not entirely free of risks. Therefore many authors are against prophylactic surgery in patients with complex forms of lipomas, suggest-



ing that these individuals should only be operated when they develop symptoms [1,2,4,14]. Unfortunately, a significant number of these patients develop new symptoms of neurological deterioration, despite surgery. Therefore, Pierre-Kahn et al. [14] raised the question as to whether or not the results of surgery in patients with complex forms of lipomas are any better than the natural history of the disease. This needs attention in future studies.

CONCLUSIONS

At long-term follow-up after neurosurgical untethering, neurosegmental motor level and ambulatory status remained stable in the majority of patients. Deterioration occurred only in patients with lipomyelomeningocele or myelomeningocele. Obesity and retethering were strongly associated with deterioration of ambulatory status. Retethering was seen in nine of 44 patients - the majority of them had lipomyelomeningocele.
We agree with Cornette et al. [6] that careful multidisciplinary follow-up is necessary to monitor the development of neurological changes that can be used to determine the timing of surgical untethering.

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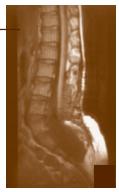


REFERENCES

1 Arai H, Sato K, Okuda O, Miyajima M, Hishii M, Nakanishi H, Ishii H. Surgical experience of 120 patients with lumbosacral lipomas. Acta Neurochir 2001; 143(9):857-864.

2 Archibeck MJ, Smith JT, Carroll KL, Davitt JS, Stevens PM. Surgical release of the tethered spinal cord: survivorship analysis and orthopedic outcome. J Pediatr Orthop 1997; 17(6):773-776.

3 Boemers TML, Gool JD van, Jong TPVM de. Tethered spinal cord: the effect of neurosurgery on the lower urinary tract and male sexual function. Br J Urol 1995; 76(6):747-751.

4 Calenbergh F van, Vanvolsem S, Verpoorten C, Lagae L, Casaer P, Plets C. Results after surgery for lumbosacral lipoma: the significance of early and late worsening. Child's Nerv Syst 1999; 15(9):439-443.

5 Colak A, Pollack IF, Albright AL. Recurrent tethering: a common long-term problem after lipomyelomeningocele repair. Pediatr Neurosurg 1998; 29(4):184-190.

6 Cornette L, Verpoorten C, Lagae L, Calenbergh F van, Plets C, Vereecken R, Casaer P. Tethered spinal cord syndrome in occult spinal dysraphism; timing and outcome of surgical release. Neurology 1998; 50(6):1761-1765.

7 Hislop HJ, Montgomery JI. Daniel's and Worthingham's muscle testing; techniques of manual examination. 6th ed. Toronto (Can): WB Saunders Company; 1995.

8 Hoffer MM, Feiwell E, Perry R, Perry J, Bonnett C. Functional ambulation in patients with myelomeningocele. J Bone Joint Surg Am 1973; 55(1):137-148.

9 Lagae L, Verpoorten C, Casear P, Vereecken R, Fabry G, Plets C. Conservative versus neurosurgical treatment of tethered cord patients. Z Kinderchir 1990; 45(Suppl 1):16-17.

10 Mazur JM, Menelaus MB. Neurological status of spina bifida patients and the orthopedic surgeon. Clin Orthop 1991; 264:54-64.

11 McDonald CM, Jaffe KM, Shurtleff DB, Menelaus MB. Modifications to the traditional description of neurosegmental innervation in myelomeningocele. Dev Med Child Neurol 1991; 33(6):473-481.

12 McLone DG, LaMarca F. The tethered spinal cord: diagnosis, significance, and management. Pediatr Neurol 1997; 4(3):192-208.

13 Pang D. Split cord malformation: Part II Clinical syndrome. Neurosurgery 1992; 31(3):481-500.

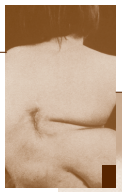
14 Pierre-Kahn A, Zerah M, Renier D, Cinalli G, Sainte-Rose C, Lellouch-Tubiana A, Brunelle F, Le Merrer M, Giudicelli Y, Pichon J, Kleinknecht B, Nataf F. Congenital lumbosacral lipomas. Child's Nerv Syst 1997; 13(6):298-335.

15 Sarwark JF, Weber DT, Gabrieli AP, McLone DG, Dias L. Tethered cord syndrome in low motor level children with myelomeningocele. Pediatr Neurosurg 1996; 25(6):295-301.

16 Warder DE, Oakes WJ. Tethered cord syndrome and the conus in a normal position. Neurosurgery 1993; 33(3):374-378.

Spinal fusion in children with spina bifida:

influence on ambulation level and functional abilities.



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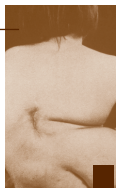
ABSTRACT

AIMS: To determine the effects of spinal fusion on ambulation level and functional abilities in children with spina bifida.

METHODS: Ten children (3 males and 7 females) with myelomeningocele were prospectively followed. Their mean age at operation was 9.3 years (standard deviation (SD): 2.4). Spinal curvature was measured according to Cobb. Pelvic obliquity and trunk decompensation were measured as well. The ambulation level was scored according to Hoffer and functional abilities, as well as the amount of caregiver assistance were documented using the 'Pediatric Evaluation of Disability Inventory'. All patients were assessed before surgery, and three times after surgery, with a total follow-up duration of 18 months after surgery.

RESULTS: After spinal fusion, magnitude of primary curvature decreased significantly ($p = 0.002$). Pelvic obliquity and trunk decompensation did not change. The ambulation level showed a significant regression ($p = 0.03$). Functional abilities and amount of caregiver assistance concerning self-care and mobility showed a non-significant trend to deteriorate within the first six months after surgery, but recovered afterwards. From pre- to 18 months after surgery, functional skills on self-care showed borderline improvement ($p = 0.07$), whereas mobility did not ($p = 0.2$). Mean scores on caregiver assistance improved significantly on self-care ($p = 0.03$), and borderline on mobility ($p = 0.06$).

CONCLUSIONS: Within the first six months after spinal fusion, more caregiver assistance is needed in self-care and mobility. It takes about 12 months to recover to pre-surgery level, while small improvement is seen afterwards. After spinal fusion, ambulation often becomes difficult. These findings are important for health care professionals, in order to inform and prepare the patients and their parents properly for a planned spinal fusion.



INTRODUCTION

Scoliosis is a serious and common problem in patients with myelomeningocele. The incidence varies from 50-80% in the literature [2,7,11]. Almost all children with thoracic or high lumbar lesion appeared to develop a scoliosis, with a decrease in incidence of 5 to 10% in those with sacral level lesion [7]. The most progression is often seen in early teenage years, but it can increase significantly in younger children as well, especially in curvatures of more than 40° [11]. Another serious spinal deformity that tends to show severe progression at an early age is lumbar kyphosis [2]. Progressive scoliosis often result in instability of the trunk, especially in curvatures > 40°, and when associated with pelvic obliquity > 15°. This might endanger sitting balance, ambulation and activities of daily living (ADL) [1,7]. Therefore, the main goal of surgical intervention is prevention of an unbalanced spine, and to maintain or improve sitting balance. Additionally, but not of less importance, is the aim to maintain or improve mobility, and ADL.

Few have been published on the functional consequences of spinal fusion [1,7,8,10,11] when compared to technical outcomes of surgery. Studies on functional consequences report a deterioration of ambulation level after surgery. However, no negative effects were found on ADL. After spinal fusion, patients are often immobilized for quite a long time (20-24 weeks). This must have consequences for functional abilities. It has been suggested that early intensive physical therapy to promote ambulation and transfers, could prevent patients from deterioration after spinal fusion, but literature on the course of functional abilities after spinal fusion is scarce [1].

The aim of this prospective study was to evaluate the course of ambulation, functional skills and the amount of caregiver-assistance needed regarding self-care and mobility, from pre- to 18 months post-surgery, in children with myelomeningocele where early mobilization was stimulated.

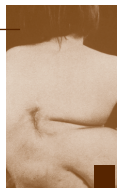
PATIENTS AND METHODS

From January 1998 to December 1999, ten consecutive patients with myelomeningocele, younger than 18 years of age, underwent surgical treatment for spinal deformities at the Wilhelmina Children's Hospital, University Medical Center Utrecht, the Netherlands. All these patients have been followed-up in the outpatient spina bifida clinic of the hospital, and were treated in the department of orthopedics because of development of progressive kyphosis, scoliosis, or both. All patients (3 males and 7 females) were all willing to participate in the present prospective study approved by the Medical Ethics Committee of our hospital. Informed consent was obtained from all the parents. One patient had kyphosis in the lumbar area, eight children had scoliosis (one thoracic, six thoracolumbar, and one lumbar scoliosis), and one boy was referred for treatment of recurrent deformation. He developed a pseudarthrosis at the thoracolumbar junction after a prior posterior fusion.

The clinical assessments were performed one day before surgery, and 6, 12 and 18 months after surgery, by the same pediatric physical therapist (MS). Radiographs of the spine were performed at all four measurements.

Table 1 Scoring of ambulation.

Score 1	community walker	ambulating outdoors with or without braces but using wheelchair for longer distances
Score 2	household walker	using braces or crutches for indoors and using a wheelchair outdoors
Score 3	exercise walker	walking only in therapeutic situations
Score 4	non-walker	wheelchair-dependent



Measurements

Spinal curvatura, pelvic obliquity, and trunk decompensation were measured using roentgenograms. The scoliosis or kyphosis angle was measured according to Cobb with the spinal rotation meter [13]. The degrees of pelvic obliquity were measured as the angle between the iliac crest and the horizontal line from radiographs taken with the patient in sitting position [14]. Trunk decompensation was measured as centimeters of deviation from the vertical line drawn from the 7th cervical vertebra to horizontal crests from radiographs. The same orthopedic surgeon (JEHP) performed all the radiographic measurements.

Gender, presence of shunted hydrocephalus, neurosegmental motor level, Intelligence Quotient (IQ), and age at operation were documented. Sitting height was measured. Ambulation level was scored according to the criteria of Hoffer et al. [6], as shown in Table 1.

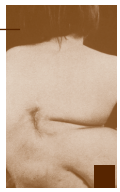
Functional abilities, and the amount of caregiver assistance concerning self-care and mobility were scored using the Dutch version of the 'Pediatric Evaluation of Disability Inventory' PEDI[3,5]. The PEDI is a validated and reliable parental questionnaire that measures functional skills (FS), and caregiver assistance (CA) in three domains: self-care, mobility and social function. For the purposes of this study we used the scores on the self-care (FS and CA) and mobility (FS and CA) domains as outcome measures. Results were calculated as a scaled score ranging from 0 to 100. In healthy children (≥ 7.5 years of age) all functional skills should be mastered leading to a score of 100. Regarding caregiver assistance, the higher the score the higher the level of independence.

In seven patients, surgical release of tethered cord prior to spinal fusion was carried out by a pediatric neurosurgeon. Surgical techniques used for correction of kyphosis, scoliosis, or both, are listed in Table 2. Three patients had undergone a two-stage anterior-posterior procedure. The same pediatric orthopedic surgeon (JEHP) performed all the operations.

Table 2 Patient characteristics.

Patient no.	Gender	Age	Motor level	Ambulation level	Deformity	Convexity and localization of primary curvature	Cobb angle		Pelvic obliquity		Type and localization of spinal fusion
							Pre-surg.	Post-surg. 18mo	Pre-surg.	Post-surg. 18 mo	
1	M	9.3	T12	WCD	Scoliosis	R: T4-L4	39	35	0	0	Ant: T12-L5 + Posterior: T3-S1
2	F	9.1	L3-L4	WCD	Scoliosis	L: T12-L5	50	28	8	11	Ant: T9-L4
3	F	12.0	L2	WCD	Scoliosis	R: T4-T12	60	32	0	5	Posterior: T3-S1
4	F	8.4	↑ T11	WCD	Kyphosis	T12-S1	90	109	0	6	Ant: T12-L5 + Strut-graft
5	F	11.1	L3	EW	Scoliosis	R: T7-L2	87	32	0	5	Ant: T11-L5 Posterior: T3-S1
6	M	9.3	T12	EW	Scoliosis	L: T11-L4	47	20	15	0	Ant: T12-L4 + Posterior: T3-S1
7	F	10.6	S1	HW	Scoliosis	R: T12-L3	40	27	0	7	Ant: T12-S1
8	F	3.4	L5	EW	Scoliosis	L: T11-L5	43	27	15	10	Ant: T12-L5
9	F	10.0	↑ T11	WCD	Scoliosis	R: T6-L4	75	40	36	0	Posterior: T11-S1
10	M	10.3	↑ T11	WCD	Pseud-arthrosis	R: T12-L1	58	40	41	25	Ant: T12-L1 + Strut-graft

Abbreviations: M = male, F = female, WCD = wheelchair-dependent, EW = exercise walker, HW = household walker, R = right, L = left, Ant = anterior, mo = months.



All children were nursed on a regular bed for an average of seven days after the final operation, and then immobilized in a bivalved total-contact orthosis (hip-spica with both legs) for a period of 12 weeks. This orthosis allowed upright posture in a wheelchair, and permitted regular inspection for pressure sores. Afterwards, patients received a customized body-jacket for an additional 12 weeks. In this period, transfer training was started during physical therapy sessions, and patients were encouraged to walk if possible. Six months after surgery, mobilization was completely unrestricted.

Statistical analysis

Due to small sample sizes and data distribution, non-parametric Friedman tests were used for analyzing the four repeated measurements on all variables (Cobb angle of primary curvature, pelvic obliquity, trunk decompensation, sitting height, ambulation level, and PEDI-scores). When differences between the measurements appeared to be significant ($p < 0.05$), the Wilcoxon signed-rank test was used to detect which specific follow-up measurements significantly differed from each other. Data are presented as mean and standard deviation, or median and interquartile range (25th - 75th centile) (IQR) [p1]when appropriate. Descriptive analysis was performed with the Statistical Package of the Social Sciences (SPSS 9.0).

RESULTS

General characteristics are presented in Table 2. The mean age (SD) at operation was 9.3 (3.4) years. All patients had shunted hydrocephalus and Chiari II malformation. Their mean IQ was 72.7 (range: 60-95). One patient had assisted mechanical ventilation (tracheostomy) because of brainstem dysfunction (case 9). Two girls had a lumbosacral lesion (case 7, 8). Both had restricted walking abilities prior to surgery due to hypertonia in the lower extremities.

Median values of Cobb angle, pelvic obliquity, trunk decompensation, and sitting height, before and after surgery are shown in Table 3. Magnitude of primary curvature (Cobb angle) decreased significantly after operation. Significant changes were seen between measurement 1 and 2 ($p = 0.03$), 1-3 ($p = 0.02$), and 1-4 ($p = 0.02$) respectively. Within 18 months after surgery some loss of correction occurred. Sitting height improved after surgery. Significant changes were seen between measurement 1-2 ($p = 0.04$), 1-3 ($p = 0.04$), 1-4 months ($p = 0.04$), and between 2-4 ($p = 0.03$).

Ambulation deteriorated significantly ($p = 0.03$). After surgery, two out of three exercise walkers lost walking ability permanently (case 5, 6). One girl (case 8) was operated at a young age (3.4 years) because of rapid development of a severe scoliosis (43°) with pelvic obliquity (15°), resulting in an unbalanced sitting. After surgery, she lost the ability to walk in therapeutic situations temporarily, but regained it within one year after surgery. Another girl (case 7), a household walker prior to surgery, became permanently wheelchair-dependent. She developed a (right) hip luxation within six month after spinal surgery due to severe hypertonia in her leg, despite tethered cord release prior to spinal fusion.

The mean PEDI-scaled scores (SD) on functional skills self-care showed a non-significant tendency to deteriorate from measurement one to six months after surgery (measurement two). Most problems were seen in items concerning dressing upper and lower body, and

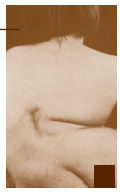


Table 3 Results before and after surgery with regard to Cobb angle of primary curvature, amount of correction, pelvic tilt, trunk decompensation, sitting height, and hip flexion contractures.

	Before surgery	6 months after surgery	12 months after surgery	18 months after surgery	p-value
Measurement	1	2	3	4	
Cobb angle (degrees)					**0.002**
- median (P50)	54.0	26.0	32.0	32.0	
- IQR (P25-P75)	42.3 - 78.0	22.5 - 38.0	26.8 - 39.8	27.0 - 40.0	
- minimum - maximum	39.0 - 90.0	16.0 - 120.0	15.0 - 110.0	20.0 - 109.0	
Pelvic obliquity(degrees)					0.82
- median (P50)	4.0	3.5	6.0	5.5	
- IQR (P25-P75)	0.0 - 20.3	0.0 - 15.0	0.0 - 20.0	0.0 - 10.3	
- minimum - maximum	0.0 - 41.0	0.0 - 32.0	0.0 - 35.0	0.0 - 25.0	
Trunk decompensation (cm)					0.58
- median (P50)	0.8	0.0	0.8	1.0	
- IQR (P25-P75)	0.0 - 2.0	0.0 - 1.3	0.0 - 1.1	0.0 - 2.0	
- minimum - maximum	0.0 - 3.0	0.0 - 3.0	0.0 - 3.0	0.0 - 3.0	
Sitting height (cm)					**0.003**
- median (P50)	63.0	66.6	69.5	70.6	
- IQR (P25-P75)	55.0 - 67.8	58.8 - 70.3	58.2 - 73.9	65.9 - 74.4	
- minimum - maximum	50.0 - 68.5	51.5 - 77.2	51.0 - 76.5	54.0 - 77.0	

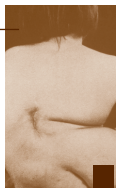
Values are presented in median and IQR as they were not normally distributed. All values that are statistically significant are indicated in bold.

Abbreviations: IQR = interquartile range, cm = centimeters.

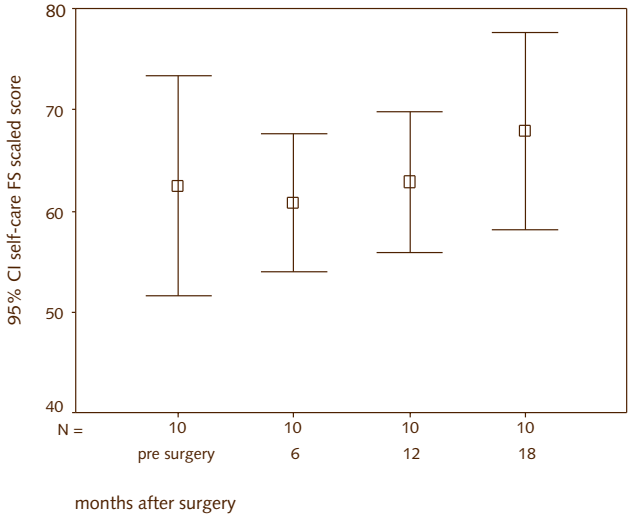
bowel and bladder management, such as inability to catheterize themselves. After six months, scores showed a significant improvement ($p < 0.01$: measurement 2-4, and 3-4), and restored to pre-surgery level at 12 months. The changes are shown in Figure 1a. Eighteen months after surgery, functional skills on self-care showed borderline improvement ($p = 0.07$) compared to pre-surgery level. The same trends were seen for mobility functional skills (Figure 1b). Deterioration six months after surgery mainly concerned transfers-items. From first measurement to 18 months after surgery, functional skills on mobility did not improve significantly ($p = 0.2$).

Changes for caregiver assistance regarding self-care and mobility are shown in Figure 2a and Figure 2b respectively. The higher the score on caregiver assistance, the higher the level of independence, meaning that less caregiver assistance was needed 18 months after surgery compared to pre-surgery. Mean scores on caregiver assistance improved significantly on self-care ($p = 0.03$), and borderline on mobility ($p = 0.06$). PEDI-scores improved from baseline to 18 months follow-up, in all but one patient. In the girl that developed a hip luxation (case 7), scores on all PEDI-domains deteriorated.

Complications were seen in 8 out of 10 patients. In one girl, case 4 with severe lumbar kyphosis, no correction was obtained. Due to resorption of the strut-graft, her kyphosis showed progression from 90° before to 109° after surgery. In three other patients (case 2, 5 and 6) the instrumentation came loose, leading to some loss of correction in one patient (case 2). One of the patients (case 10) had osteomyelitis. His instrumentation had to be removed after six months, but he did not loose correction. Three out of 10 patients (case 1, 7, and 8) increased their curvature above or below the fusion mass. In two patients (case 3 and 9), no complications were seen.



A



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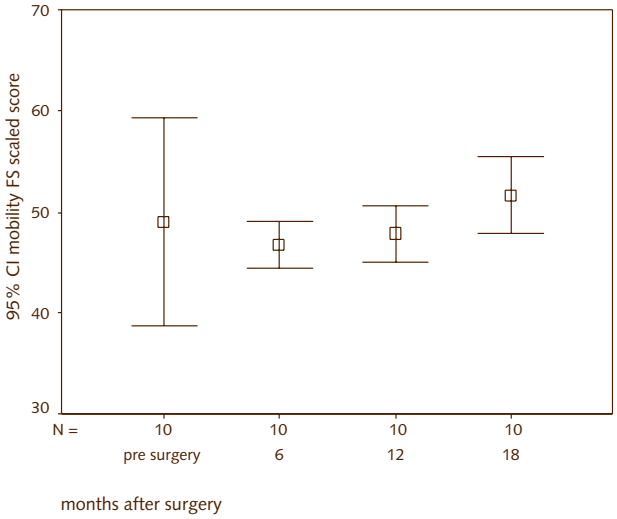
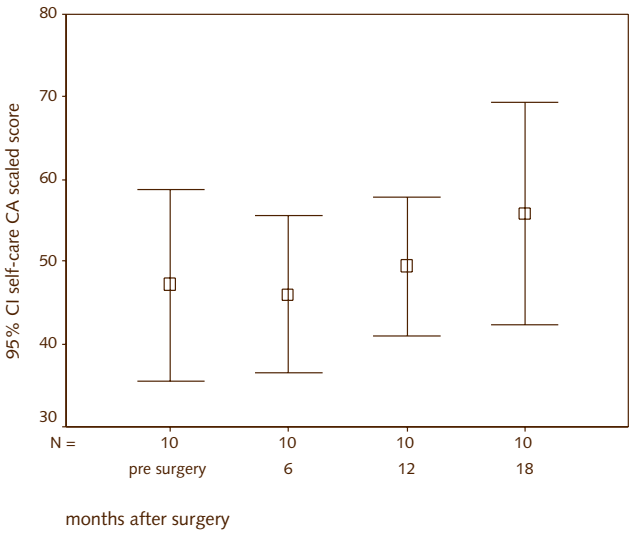


Abbreviations: CI = confidence interval, FS = functional skills.

Figure 1A and B

Functional skills scaled scores with regard to self-care (A) and mobility (B) before and after surgery.

A



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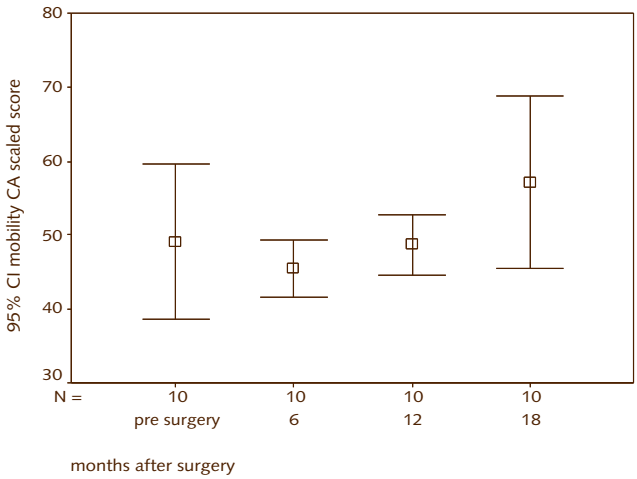
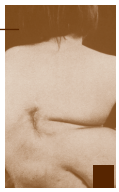


Abbreviations: CI = confidence interval, FS = functional skills.

Figure 2A and B

Caregiver assistance scaled scores with regard to self-care (A) and mobility (B) before and after surgery.



DISCUSSION

This study describes the reconvalence of children with myelomeningocele after spinal fusion, with special emphasis on ambulation, functional abilities and amount of caregiver assistance needed. Our study suggests that functional abilities on self-care and mobility showed a tendency to decrease within the first six months after surgery, but gradually improved to pre-surgery level within 12 months, and improved further at 18 months after surgery. The same trend was seen in the amount of caregiver assistance. Eighteen months after surgery less caregiver assistance was needed on self-care compared to pre-surgery level. Caregiver assistance on mobility did not change significantly. After spinal fusion, ambulation became difficult in three out of four patients who were able to ambulate prior to surgery.

Our main interest was not to evaluate the efficacy of different surgical procedures on scoliosis, but to follow-up the course of functional abilities after spinal fusion followed by immobilization. We evaluated the group as a whole and did not distinct for different types of fusion, as all our patients were immobilized in a standardized way, for the same period.

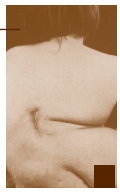
As in other series, we found a significant correction of primary curvature after surgery, in all but one patient. The severity of the curvature at final follow-up (median: 32°) in our study, is comparable with that in current literature [1,2,9,12,14]. As in these series, some loss of correction is seen at final follow-up, mostly due to hardware problems. As expected, we found a significant increase in sitting height compared to pre-surgery, as this is caused by correction of the scoliosis. Moreover, we also found an increase in sitting height within the follow-up period (measurement 2-4). This means that spinal growth is still possible after fusion, probably due to the Luque technique we used in posterior fusion.

Although spinal alignment improves significantly after surgery, morbidity is high in most series. In our study, problems in terms of loss of

correction, progression above or below fusion mass or osteomyelitis occurred in eight out of 10 patients.

Mazur et al. [8] studied functional status and ambulation after spinal fusion in 49 children with spina bifida. They found deterioration in ambulation in 67% of patients who had undergone combined anterior and posterior fusion, in 57% following anterior fusion, and in 27% following posterior fusion alone. Muller et al. [10] reported that 57% (8/14) of their patients lost some of their ambulation capacity after spinal fusion. We also found deterioration of ambulatory skills after surgery. Permanent deterioration of ambulation was seen in three out of four patients who were able to walk prior to surgery. One girl with sacral level paralysis (case 7) lost ambulation due to progressive hypertonia, and two patients (case 5 and 6) had thoracic and high lumbar lesion level. It remains unclear whether or not walking abilities would have been lost anyway in these patients. It is known that most children with high level myelomeningocele often loose the ability to walk during their teenage years, due to high energy consumption when walking with aids, and as a result of growth or obesity [4].

Few studies report on the influence of spinal fusion on functional status. These series report that spinal fusion does not adversely effect motor skills or Activities of Daily Living (ADL) [1,8,10]. Our data are partially in agreement with these findings, as we also found no adverse effects in ADL on the long-term. However, a temporary decrease of functional abilities within the first six months after surgery was found, but it gradually improved to pre-surgery level at 12 months. Moreover, 18 months after surgery, we found a significant improvement in scores in self-care, as well as in the amount of caregiver assistance needed compared to pre-surgery level. Regarding overall mobility, non-significant changes were found. Askin et al. [1] also studied the influence of spinal fusion on functional status in 20 patients with four different neurological disorders. They found a tendency for overall abilities in all patient groups to deteriorate for the first six months. They returned to their pre-surgery level at 12 months. Their series included five children with myelomeningocele. Of these



children 6-months assessments were not available as these patients were still in a postoperative brace, but 12 months after surgery all patients returned to their pre-surgery level in ADL. However, walking abilities deteriorated in all of them. Our data are in agreement with these findings.

When interpreting our findings, it should be kept in mind that the sample size of our study is small. Therefore, long-term prospective follow-up studies have to be continued with larger numbers. On the other hand, our results confirm other small series and scarce literature on this subject. Hence, with ongoing advances in technology to improve spinal fusion in children with myelomeningocele, future studies should not only focus on the effectiveness of surgery in terms of spinal curvature, pelvic obliquity, and complications. It is inevitable to evaluate the effect of such major surgery on children with spina bifida and their families.

This addresses the issue that the functional consequences of spinal fusion are measured in different ways, making comparison of results difficult. Moreover, outcome measures used in these series [8,10] are often clinician oriented and might not reflect the abilities that are important to patients themselves. To overcome this problem, we used the PEDI to evaluate functional abilities and the amount of caregiver assistance needed pre- and post-surgery. The PEDI is a very long questionnaire based on a structured interview with caregivers and by that, time consuming. This makes it less suitable for routine follow-up. Recently, Wai et al. [15] developed the 'Spina Bifida Spine Questionnaire' (SBSQ). The SBSQ appeared to be a valid and reliable self-administered questionnaire, to assess the effectiveness of surgical and other therapeutic interventions for children with spina bifida and scoliosis. In retrospect, this instrument could have been more appropriate for our study. However, it was not available when we started our study in 1998. The SBSQ will be a useful instrument for future follow-up studies on the effectiveness of spinal surgery in patients with spina bifida.

CONCLUSIONS

Within the first six months after spinal fusion, more caregiver assistance is needed compared to pre-surgery, especially with regard to dressing upper and lower body, bowel and bladder management, and transfers. It will take about 18 months before patients and their parents experience the beneficial effects that spinal fusion can have on functional abilities. After surgery, walking abilities often become difficult. These findings are important for health care professionals in order to inform and prepare the patients and their parents properly for a planned spinal fusion.

ACKNOWLEDGEMENTS

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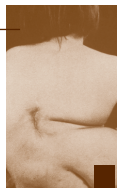


REFERENCES

1 Askin GN, Hallett R, Hare N, Webb JK. The outcome of scoliosis surgery in the severely physically handicapped children. Spine 1997; 22(1):44-50.

2 Banta JV. Combined anterior and posterior fusion for spinal deformity in myelomeningocele. Spine 1990; 15(23):943-952.

3 Custers JWH, Wassenberg-Severijnen JE, Net J van der, Vermeer A, Hart HT, Helders PJM. Dutch adaptation and content validity of the 'Pediatric Evaluation of Disability Inventory'. Disabil Rehabil 2002; 24(5):250-258.

4 Findley TW, Agre JC, Habek RV, Schmalz R, Birkebak RR, McNally MC. Ambulation in the adolescent with myelomeningocele I; early childhood predictors Arch Phys Med Rehabil 1987; 68(8):518-522.

5 Hayley SM, Coster WJ, Ludlow LH, Haltiwanger JT, Andrellos PJ. 'Pediatric Evaluation of Disability Inventory' (PEDI). Development, standardization and administration manual. Boston, MA: New England Central Center Inc. and PEDI Research group; 1992.

6 Hoffer M, Feiwell, Perry J, Bonnet C. Functional ambulation in patients with myelomeningocele. J Bone Joint Surg Br 1973; 55:137-148.

7 Kahanovitz N, Duncan JW. The role of scoliosis and pelvic obliquity on functional disability in myelomeningocele. Spine 1981; 6(5):494-497.

8 Mazur J, Menelaus MB, Dickens DRV, Doig WG. Efficacy of surgical management for scoliosis in myelomeningocele: correction of deformity and alteration of functional status. J Pediatr Orthop 1986; 6(5):568-575.

9 McMaster J. Anterior and posterior instrumentation and fusion of thoracolumbar scoliosis due to myelomeningocele. J Bone Joint Surg Br 1987; 69B(1):20-25.

10 Müller EB, Nordwall A, Von Wendt L. Influence of surgical treatment of scoliosis in children with spina bifida on ambulation and motor skills. Acta Pediatr 1992; 81(2):173 -176.

11 Müller EB, Nordwall A, Odén A. Progression of scoliosis in children with myelomeningocele. Spine 1994; 19(2):147-150.

12 Parsch D, Geiger F, Brocai DRC, Lang RD, Carstens C. Surgical management of paralytic scoliosis in myelomeningocele. J Pediatr Orthop (Part B) 2001; 10(1):10-17.

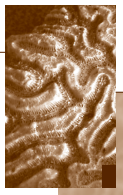
13 Pruijs JEH, Hageman MA, Keessen W, Meer R van der, Wieringen JC van. Spinal rotation meter: development and comparison of a new device. Acta Orthop Belg 1995; 61(2):107-122.

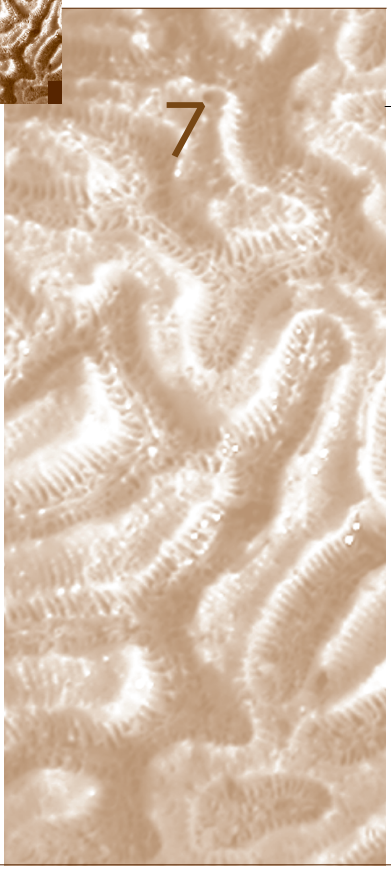
14 Sponseller PD, Young AT, Sarwark JF, Lim R. Anterior only fusion for scoliosis in patients with myelomeningocele. Clin Orthop 1999; 364:117-124.

15 Wai EK, Owen J, Fehlings D, Wright JG. Assessing physical disability in children with spina bifida and scoliosis. J Pediatr Orthop 2000; 20(6):765-770.

Summary, general discussion and conclusions

MAGC Schoenmakers





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SUMMARY

Due to advanced medical treatment, more children with spina bifida survive into adulthood. Most outcome studies in the field of spina bifida research focus on disease aspects ('pathology') and its relation to one of the domains in the 'disablement process' such as physical functioning, cognitive abilities, or psychosocial adjustment [1-5]. From these studies it became clear that spina bifida has significant impact on the health of individuals, but little is known on 'health related quality of life' (HRQL).
The main goal of this thesis is to get more insight into the different domains of the 'disablement process' in children with spina bifida. We were especially interested in how 'pathology', 'impairments', 'functional limitations', 'disability' and HRQL relate to each other in order to set relevant and realistic goals for physical therapy treatment. Physical therapy traditionally focuses on improvement of muscle strength and muscle tone, and the prevention of contractures, to optimize childhood development and functioning. However, it is not clear how these impairments relate to functional independence and HRQL. As there is often an intense demand for commitment on children and their families regarding physical therapy, knowledge of determinants of independence is of utmost importance in order to focus "on realistic goals rather than wasting efforts on attempting the impossible" [2].

In Chapter 1, a brief introduction is presented with respect to childhood spina bifida and the advances in medical treatment over the last decades. The symptoms of occult forms of spinal dysraphism as well as the symptoms of spina bifida aperta, especially of myelomeningocele (MMC) are discussed. The view on three major disablement concepts is described and the conceptual framework that we have chosen for our research is defined. The impact of spina bifida on the four domains of the 'disablement process' such as 'pathology', 'impairments', 'functional limitations' and 'disability', as well as the possible

impact on HRQL is discussed. The aims and outline of the thesis are formulated.

In Chapter 2, we investigated outcome in 177 children with spina bifida, aged 1-18 years. In this cross-sectional study, we compared outcome in children with MMC, (n = 122) to those with other types of spina bifida (OTSB, n = 55) regarding 'pathology' (presence of associated central nervous system abnormalities and lesion level), 'impairments' (mental status, range of motion and muscle strength), 'functional limitations' (ambulation level, scores of functional skills) and 'disability' (caregiver assistance), as well as perceived competence and HRQL. Additionally, within the MMC group we compared outcome in children with and without hydrocephalus. We also investigated associations between 'impairments', 'functional limitations', 'disability', perceived competence and quality of life. Ambulation level was statistically significantly associated with muscle strength (the higher the muscle strength, the higher the ambulation level), as well as with lesion level (the lower the lesion level, the higher the ambulation level). Furthermore, we concluded that children with MMC, especially those with shunted hydrocephalus, appeared to be significantly more impaired, to have more functional limitations and lower scores on HRQL compared to patients with other types of spina bifida. However, perceived competence in MMC patients did not differ from those with OTSB who had less impairments and more functional abilities. Moreover, HRQL appeared to be significantly associated with ambulation level, functional abilities and mental status, whereas perceived competence showed no association with most of these variables. In our opinion, health care professionals should not mainly focus their attention on more disabled patients with MMC with severe disabilities, especially regarding psychosocial support. This kind of support might be needed in children with OTSB and minor disabilities as well.

In Chapter 3, we investigated which factors of the 'disability process' domains were the most important determinants for functional independence regarding self-care and mobility in 122 children with MMC. Our main interest regarding HRQL was to explore which functional abilities were the most important determinants for quality of life. Our findings showed that parameters on the level of impairments were significantly associated with disability. From our study it is suggested that lesion level, mental ability, having no contractures and good muscle strength of lower extremities particularly determine independence in self-care in children with MMC. All these factors are mutually dependent. Mental ability, having no contractures and good muscle strength of lower extremities were the most important determinants for functional independence in mobility. With regard to HRQL, we found that being independent in mobility appeared to contribute more to HRQL than being independent in self-care or being wheelchair-dependent. This information is of clinical significance in planning a comprehensive and realistic rehabilitation program.

In Chapter 4, we investigated muscle strength, ambulation level, motor performance and functional outcome regarding self-care, mobility and social function (measured with the Pediatric Evaluation Disability Inventory (PEDI)) in two groups of children with sacral level spina bifida (MMC versus lipomyelomeningocele (LMMC)). Both groups were compared with each other and with reference values of the normal population regarding motor performance and functional outcome. In the MMC group (n = 30), all children had hydrocephalus and Chiari II malformation. In the LMMC group (n = 14), none of the children had these central nervous system abnormalities. We concluded that children with sacral level paralysis had more than minor gait disturbances. In both groups, gross motor and functional problems were seen, whereas fine motor problems only occurred in the MMC group with shunted hydrocephalus and Chiari II malformation, and in none of the children with LMMC without these central nervous system abnormalities. In the MMC group, mus-

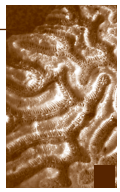
cle weakness and motor problems, mainly concerning balance, were more severe compared to the LMMC group, but functional abilities (ambulation level and PEDI-scores) did not differ significantly between the groups. Both groups needed caregiver assistance for a prolonged period, especially regarding bladder and bowel management. These findings need special attention, particularly in children who attend regular schools. Periodic monitoring is warranted, so that appropriate intervention can be started when needed.

In Chapter 5, we prospectively studied the long-term effects of neurosurgical untethering on neurosegmental motor level and ambulation level in 44 patients with spinal dysraphism, aged 1-18 years (MMC, n = 16; LMMC, n = 9; other types of spina dysraphism, n = 19). Motor and ambulation levels were assessed before, and three times after surgery with a mean duration of follow-up of 7.1 years. We also tried to gain insight into risk factors in deterioration of ambulation level and in the occurrence of retethering. This long-term follow-up study shows that after neurosurgical untethering, neurosegmental motor level and ambulatory status remained stable in the great majority of patients. Late deterioration (≥ 4 years after surgery) of neurosegmental motor level and ambulatory status occurred mainly in patients with (lipo)myelomeningocele. Obesity and retethering were strongly associated with the deterioration of ambulation level. In this study, retethering was mostly seen in patients with LMMC, although the presence of lipomas itself was not a statistically significant factor. Careful multidisciplinary follow-up is necessary to monitor the development of neurological changes that can be used to determine the timing of surgical untethering.

In Chapter 6, we prospectively studied the functional consequences of surgical correction of spinal deformities in 10 children with MMC. Progressive scoliosis often results in instability of the trunk that might endanger sitting balance, ambulation and activities of daily life (ADL). Therefore, the main goal of surgical intervention is prevention of an

unbalanced spine and to maintain or improve sitting balance, mobility and ADL. In this study, ambulation level, functional skills and caregiver assistance (PEDI-scores) regarding self-care and mobility were measured before, and three times after surgery at 6, 12 and 18 months respectively. After spinal fusion magnitude of curvature decreased significantly, whereas pelvic tilt and trunk decompensation did not. Furthermore, we found that within the first six months after spinal fusion more caregiver assistance was needed in self-care and mobility compared to pre-surgery, especially with regard to dressing upper and lower body, bowel and bladder management, and transfers. It took about 12 months to recover to pre-surgery level of functioning, while small improvement was seen afterwards. After spinal fusion, ambulation became difficult in most of the patients who were able to ambulate (mainly exercise walkers) prior to surgery. These findings are important for health care professionals in order to inform and prepare the patients and their parents properly for a planned spinal fusion.

In Chapter 7, a summary, the general discussion and conclusions as well as directions for future research are given.



GENERAL DISCUSSION

Now that the data have been analyzed and interpreted in this thesis, it is time to update the discussion with our current knowledge on interrelationships between 'impairments', 'functional limitations', 'disability' and 'HRQL' in childhood spina bifida. What are the implications of our results? What does it imply for the knowledge of individuals with spina bifida and their parents, and for health care professionals working with them? What are the implications for physical therapy treatment strategies and future research?

Functional prognosis

Spina bifida is one of the major causes of locomotor dysfunction in children. One of the first questions that most parents have is: 'will my child be able to walk'? Therefore, factors associated with ambulation have been studied extensively in the past [6-13]. There is a large disparity in reports of ambulatory outcome across neurosegmental lesion groups [6,7,9]. This might be due to lack of homogeneity in patient groups and/or due to the cross-sectional nature of most studies. Nevertheless, lesion level is mostly used for predicting ambulation level in children with spina bifida but other determining known 'pathology' factors are hydrocephalus, orthopedic problems, obesity, age, etc [10,13]. As far as we know, there is only one study that focused on the relation between muscle strength and ambulation [9]. McDonald et al. [9] studied 291 children with MMC and found m. iliopsoas strength to be the best predictor of ambulation, with the m. quadriceps, m. tibialis anterior and mm. gluteï also contributing significantly. Therefore, they recommended the use of specific patterns of lower-extremity muscle strength for predicting ultimate walking ability and establishing ambulatory goals.

In our studies, we also found ambulation level to be related to muscle strength (Chapter 2). All community walkers in our study had muscle strength grade 5 of hip flexor muscles, grade 4-5 of hip abductor muscles and grade 3 or more of ankle dorso-flexors. In Chapter 3 we studied determinants for independence in mobility, as mobility encompasses more than just ambulation. Mobility has been defined as the "individual's ability to move around effectively in his or her surroundings, with or without assistance or physical aids "[14]. Regarding independence in mobility as measured with PEDI-scores on caregiver assistance, we found lesion level to be of less importance than lower-extremity muscle strength. We found that independence in mobility appeared to be significantly related to strength of hip flexor muscles, hip abductor muscles, hip extensor muscles, knee extensor muscles and ankle dorso-flexors. These are the same muscle groups as found by McDonald et al. [9] in relation to ambulation. Therefore, we believe that muscle group-patterns rather than lesion level should be used for setting relevant and realistic goals regarding ambulation and mobility. Moreover, with regard to HRQL, we found that being wheelchair-dependent was of less importance than being independent in mobility. Therefore, walking itself should not be the primary focus for all patients with spina bifida. Parents and therapist would be better to focus "on realistic goals rather than to attempt the impossible" [2]. Instead, an independence-oriented approach would be more appropriate. The process of learning to move around without assistance should start early in childhood to make it easier for children to mature into well-functioning adults as far as mobility is concerned. Facilitation of the optimal level of independence in mobility, implicates that early wheelchair training and transfer training in the actual environment might have the highest priority for a child with low muscle strength (grade 0-3) of m. iliopsoas and m. quadriceps. On the other hand, optimizing running or jumping skills, might only be a realistic goal for children with good strength (≥ 4) of hip abductor and ankle dorso flexor muscles. In Chapter 4 we studied muscle strength and motor performance in 44 children with sacral level paralysis (30 with MMC and

14 with LMMC). 'Normal' ambulation (without the use of aids) was strongly associated with strength of hip abductor muscle and ankle dorso-flexor muscles. In both groups, muscle weakness was most severe in hip extensor and calf muscles. Balance problems in actions such as hopping, jumping and standing on one leg, were seen in 90% of the children with MMC compared to half of the children with LMMC. The link between balance problems and its relation to limitations in regular sporting activities needs further investigation.

Outcome measures in Spina Bifida

Due to the multitude of body systems affected, the potential impact of spina bifida on individual development and adaptation, and the intensive involvement of the family, children with spina bifida require a holistic approach [15]. Traditionally, health care and physical therapy treatment focused on 'impairments' rather than on the person's function in his or her environment. Nowadays, there is a shift towards evaluation of both capability and performance in the environment. These aspects are incorporated into the PEDI as developed by Hayley et al. [16]. Recently, the Dutch adaptation of the PEDI was investigated [17-18], and it appeared to be a reliable and feasible instrument to discriminate between children with and without disabilities, and to evaluate changes in functional status. Therefore, in this thesis the Dutch PEDI was the instrument of choice, used to evaluate the amount of 'functional limitations' and 'disability' in different types of spina bifida (Chapter 2, 4). Furthermore, it was our main outcome measure for determinants of functional independence and HRQL (Chapter 3). We also used the PEDI to evaluate changes in functional abilities after surgical correction of spinal deformities (Chapter 6) as it provides important information on the level of independence as reflected in the amount of caregiver assistance needed for daily skills, before and after this major surgery.

Recently, the PEDI has been used in other series to evaluate functional abilities of children with spina bifida [19-21]. We found that PEDI-scores in young children (≤ 7.5 years) were below the mean, but still within normal ranges, with the exception of scores on mobility. In older children (> 7.5 years) scores were deviant in all domains (Chapter 3, Table 1; Chapter 4, Table 4). This is in agreement with other studies. Dahl et al. [20] studied self-care skills in 35 children with MMC and found that 60% needed moderate to maximum caregiver assistance. They found low intelligence, poor executive functions, hypotonia above the cele level and non-ambulancy, to be risk factors for poor self-care. In our study on children with MMC as well as those with OTSB, we found that 64% (113/177) of our patients needed some kind of caregiver assistance (Chapter 2, Table 4). Most help was needed in self-care, especially in bowel and bladder management, even in non-MMC patients. In our study on determinants for independence in self-care in 122 children with MMC, mental status and motor function below the cele level were found to be the most important determinants (Chapter 3, Table 2).

Although the PEDI is primarily designed for functional evaluation of children aged between 0.5 and 7.5 years, it can be used in older children as well if their performance falls below that expected of non-disabled children. As this was the case in all our studies (Chapter 2, Table 4; Chapter 4, Table 4; Chapter 6, Figure 1 and 2), we believe that the PEDI is a very useful instrument for evaluating improvement in both capability and performance in children with spina bifida. To obtain a realistic picture of the functional skills and the amount of caregiver assistance required, it is important to observe changes in PEDI-scores on item level instead of domain level only. This addresses the need for the development of a disease specific PEDI-profile for children with spina bifida, using the Rasch rating scale methodology [17,19]. This will be the next step in our future research on spina bifida.

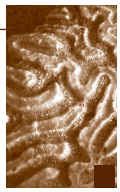
When evaluating the efficacy of intervention strategies from the individual's (and parent's) perspective, elements of emotional well-being, competence and life satisfaction should also be included. These

aspects are included in the field of HRQL. HRQL has only recently become a variable of interest in spina bifida research [15,23-26]. Both generic and condition specific instruments exist in literature and there is a debate as to which is preferable. The decision is directed by the research question. It is known that children with spina bifida and their families do not view their health as negatively as their physicians do [4,5]. This suggests that HRQL might be best understood from the perspective of the individuals themselves. Therefore, in this thesis we opted for the 'Spina Bifida Health Related Quality of Life Questionnaire' as developed by Parkin et al. [23]. This disease specific-instrument measures the perception of health from the viewpoint of children (5-12 years) and adolescents (13-20 years) themselves.

From recent literature there is growing evidence that physical functioning of patients with spina bifida is related to HRQL, but on the other hand there appears to be no significant relation between HRQL and 'pathology' parameters such as lesion level or hydrocephalus [15,24-26]. Apart from the physical functioning, other factors such as personal and family factors seem to contribute significantly to HRQL. Kirpalani et al. [24] found that physical functioning explained a significant proportion of variance in HRQL (31 to 47%), although parental hope explained an additional 19 to 24% of the variance. Pit-Ten Cate et al. [25] showed that children with spina bifida had lower scores on HRQL with regard to self-care, incontinence and mobility compared to children with hydrocephalus alone. They found that severity of the condition and family resources independently predicted 32% of the variance in HRQL. Sawin et al. [15] studied factors associated with HRQL in 60 adolescents with spina bifida, and found HRQL to be related to personal factors (such as stress, hope, future expectations) and family factors (such as family satisfaction, family activity, and family overprotection). Padua et al. [26] found lower scores on the physical aspects of HRQL in children who are more severely disabled. This is in agreement with our findings in Chapter 2, as we found lower scores in HRQL in patients with a greater amount of disability. HRQL appeared to be positively related to ambulation level and func-

tional abilities (PEDI-scores) in children and adolescents (Table 6). In Chapter 3, we tried to find out which functional abilities were most important determinants regarding HRQL in individuals with spina bifida, in order to set relevant and realistic goals for physical therapy treatment. We found that being independent in mobility appeared to contribute more to HRQL than other functional abilities such as being independent in self-care or being wheelchair-dependent.

Spina bifida is a complex disorder that affects almost all aspects of an individual's life. The consequences of spina bifida can be displayed using a 'disablement' framework. The use of such a conceptual model is the basic architecture on which research, policy and clinical care are built [27]. In this thesis we used the 'disablement process' as advocated by Verbrugge and Jette [27] to guide us in our research, instead of the ICIDH model that was often used in European rehabilitation research [14]. The ICIDH concept was primarily designed as a classification model for coding and manipulating data on the consequences of health conditions. Moreover, the model did not differentiate between 'functional limitations' (individual capability) and 'disability' (actual performance in response to the environment), which are important issues in pediatric disability research [27-28]. Both aspects are reflected in the PEDI. Recently, the International Classification of Functioning, Disability and Health (ICF) designed by the WHO in 2001, has been advised [30]. In contrast to the ICIDH, the ICF also incorporates qualifiers of capacity and performance as reflected in the level of activity and participation, respectively (Chapter 1, Figure 1). For future research on disability in children with spina bifida, the ICF could be a model to choose.



Treatment approaches

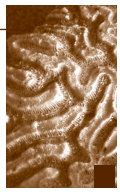
Physical therapy is a common intervention for children with movement disorders. However, the efficacy of physical therapy intervention has been questioned. In the past, physical therapy focused primarily on the child's impairments, such as normalization of muscle tone, increasing range of motion and increasing muscle strength, in the hope that this would automatically result in improvement of skills and functions of daily living [31]. Although we found impairment parameters to be significantly associated with disability in our study on determinants for functional independence (Chapter 3), this does not automatically mean that stretching and strengthening exercises of individual upper and lower extremity muscles are the best way to improve function.

Over the last years, there is growing evidence that treatment based on the function appeared to be more effective than the impairment-oriented treatment, although research in this field is mostly focused on children with cerebral palsy [31-35]. The so-called 'Functional Therapy Programs' are based on the more recent theories on motor development and motor control, and the dynamic systems approach, rather than on the reflex-hierarchical and neuromaturational theories [30] where changes in motor behavior are seen as a result of maturation. In the system models, motor development and coordination are assumed to emerge from a dynamical interaction of many subsystems in a task-specific context [31,35,36]. These models implicate that therapeutic interventions should focus on problematic function rather than on the movement patterns for the sake of the movement's alone. The child should have the opportunity to actively explore the environment in order to find out the best strategy to solve a particular functional problem.

Another recent shift in therapy intervention is the attention for parental participation. This means that the parents have an active role in deciding the goals and content of physical therapy, rather than that they are expected to carry out a treatment regime at home that is pre-

scribed by the therapists. Ketelaar et al. [34] compared the efficacy of 'functional physical therapy' versus 'regular physical therapy' on motor abilities of children with cerebral palsy. In the functional therapy program, parents were actively involved in goal setting. They found that functional skills in daily situations, as measured by the PEDI, improved more in the 'functional physical therapy' group compared to the 'regular physical therapy' group. As the functional treatment program is a systematic way of trying to solve a child's functional problem, this approach is not restricted to treatment of children with cerebral palsy, and could therefore be used in children with spina bifida as well. In the research of Ketelaar et al. [34], goal setting was limited to one discipline, as children received physical therapy only. It has not yet been investigated whether the functional programs are applicable to situations where collaborative problem-definition and collaborative goal setting become more difficult [34].

As many children with spina bifida encounter problems in physical functioning, cognitive abilities as well as in psychosocial adjustment, most young (pre-school) children are treated in a rehabilitation center. Moreover, many school-aged children receive their physical and occupational therapy in the context of the special school they attend, where parental participation is minimal. For these children and their families, a study into a family centered functional therapy with collaborative goal setting from different disciplines could be beneficial. Long-term follow-up remains important, as physical and intellectual disabilities often become more manifest when these children grow older. Furthermore, associations between disabilities and HRQL found in children, could change in time and might even be totally different from adulthood. For future research, a close collaboration between behavioral and medical researchers is needed, which will facilitate the search for better ways to prevent disability. In January 2003 such a multidisciplinary network for Childhood Disability Research in Utrecht, the Netherlands (NetChild) has been launched with a focus on maximizing the level of activities and quality of life of children with disabilities and their families. To try to understand the



'disablement process' in children, research programs in 'childhood disability' should focus on both generic and disease specific components of functioning throughout different underlying diseases or disorders, e.g. both neurological disorders (cerebral palsy, spina bifida, neuromuscular disorders) as non-neurological disorders (juvenile idiopathic arthritis, musculoskeletal disorders).

For children with spina bifida, a careful multidisciplinary follow-up is of utmost importance to monitor the development of neurological changes due to associated central nervous system such as spinal tethered cord, that is often a threat to their functional abilities. Kaufman et al. [37] studied the effects of disbanding a multidisciplinary clinic on health care of spina bifida patients. They concluded that it is unrealistic to expect the continuation of effective care by merely maintaining the individual services of the disbanded clinic, without some coordination of care.

CONCLUSIONS

The following conclusions can be drawn from this thesis:

1 Parameters on the level of 'impairments' are statistically significantly associated with 'disability'.

2 Ambulation is significantly positively associated with muscle strength of hip abductors and ankle dorso-flexors.

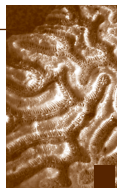
3 Good muscle strength, having no contractures and mental ability appears to be much more important for daily life functioning of children with spina bifida than other medical indicators.

4 With regard to independence in mobility, good muscle strength, especially of knee extensors, having no contractures, and good mental ability, appears to contribute more to independence than lesion level and shunt status.

5 Being independent in mobility appears to contribute more to HRQL than being independent in self-care or being wheelchair-dependent.

6 In young children (≤ 7.5 years) with spina bifida, PEDI-scores were below the mean, but still within normal ranges with the exception of scores on mobility, whereas in older children (> 7.5 years) scores were deviant in all domains.

7 Most children with sacral level paralysis have gross motor problems, more severe in myelomeningocele than in lipomyelomenigocele. In both groups caregiver assistance was needed for a prolonged period, especially regarding bladder and bowel management.



8 Although patients with myelomeningocele were more disabled than those with other types of spina bifida, perceived competence in the latter group was not better.

9 Psycho-social support might be needed in children with other types of spina bifida and minor disabilities as well as in those with myelomeningocele with severe disabilities.

10 After neurosurgical untethering, late deterioration of motor and ambulation level occurs in a minority of patients. Deterioration and retethering is mainly seen in patients with (lipo)myelomeningocele and not in patients with other types of spinal dysraphism.

11 Magnitude of curvature significantly decreases after spinal fusion.

12 Within the first six months after spinal fusion, more caregiver assistance is needed in self-care and mobility. It takes about 12 months to recover to pre-surgery level, while a small improvement is seen afterwards.

13 After spinal fusion, ambulation can become difficult in patients who are exercise walkers prior to surgery.

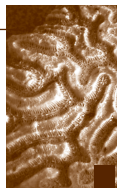
DIRECTIONS FOR FUTURE RESEARCH

1 Longitudinal cohort studies in spina bifida in different age groups are indicated to study prospectively the determinants and dynamics of the interrelationships within the different domains of disablement, such as 'impairments', 'functional limitations/activities', 'disability/participation' and personal and environmental factors.

2 A longitudinal cohort study is necessary to evaluate the efficacy of current physical therapy treatment strategies, with special emphasis on prevention of contractures and on muscle strength, and its relation to independence in mobility. In this study, stratification according to muscle patterns is necessary. Furthermore, a comparison has to be made between the traditional approach that focuses on the impairment in order to improve function, versus the functional approach where the intervention is primarily focused on training a specific ability that might result in improved muscle function and/or range of motion as well.

3 Development of a disease specific functional status profile for children with spina bifida (a disease specific PEDI-profile). The Rasch rating scale methodology can be used to observe changes in PEDI-scores on item level instead of on domain level only. A disease specific PEDI-profile indicates the individual needs of a child compared to his/her peers with the same disease.

4 Before using the 'Spina Bifida Health Related Quality of Life Questionnaire' to evaluate efficacy of treatment strategies, a Dutch adaptation, as done with the PEDI, should be established.



REFERENCES

1 Hunt GM. Open spina bifida: outcome for a complete cohort treated unselectively and followed into adulthood. Dev Med Child Neurol 1990; 32(2):108-118.

2 Hunt GM, Poulton A. Open spina bifida: a complete cohort reviewed 25 years after closure. Dev Med Child Neurol 1995; 37(1):19-29.

3 Hunt GM, Oakeshott P, Kerry S. Link between the CFS shunt and achievement in adults with spina bifida. J Neurol Neurosur Psychiatry 1999; 67(5):591-595.

4 McCormick MC, Charney EB, Stemmler MM. Assessing the impact of a child with spina bifida on the family. Dev Med Child Neurol 1986; 28(1):53-61.

5 Rasmussen FR, Lie HR, Hagelsteen JH, Lagergren J, Börjeson MC, Lagerkvist B, Kohler L. Nordic children with myelomeningocele. Parents' assessment of the handicap and physicians' classification of the disabilities. Acta Paediatr 1993; 82(3):276-280.

6 Hoffer M, Feiwell, Perry J, Bonnet C. Functional ambulation in patients with myelomeningocele. J Bone Joint Surg Am 1973; 55(1):137-148.

7 Asher M, Olson J. Factors affecting the ambulatory status of patients with spina bifida cystica. J Bone Joint Surg Am 1983; 65(3):350-356.

8 Banta JV, Casey JM, Bedell L, Morgan J. Long-term ambulation in spina bifida. Dev Med Child Neurol 1983; 25:110.

9 McDonald CM, Jaffe KM, Mosca VS, Shurtleff DB. Ambulatory outcome of children with myelomeningocele: effect of lower-extremity muscle strength. Dev Med Child Neurol 1991; 33(6):482-490.

10 Liopis ID, Munoz MB, Agullo EM, Martines AL, Aymerich VG, Valero JVF. Ambulation in patients with myelomeningocele: a study of 1500 patients. Paraplegia 1993; 31:28-32.

11 Swank M, Dias LS. Walking ability in spina bifida patients: a model for predicting future ambulatory status based on sitting balance and motor level. J Pediatr Orthop 1994; 14(6):715-718.

12 Dudgeon BJ, Jaffa KM, Shurtleff DB. Variations in midlumbar myelomeningocele: Implications for ambulation. Pediatr Phys Ther 1991; 4:57-62.

13 Findley TW, Agre JC, Habek RV, Schmalz R, Birkebak RR, McNally MC. Ambulation in the adolescent with myelomeningocele I; early childhood predictors. Arch Phys Med Rehabil 1987; 68(8):518-522.

14 The International Classification of Impairments, Disabilities and Handicaps -a manual relating to the consequences of disease. Geneva: World Health Organization; 1980.

15 Sawin KJ, Brei TJ, Buran CF, Fastenau PS. Factors associated with quality of life in adolescents with spina bifida. J Holist Nurs 2002; 20(3):279-304.

16 Hayley SM, Coster WJ, Ludlow LH, Haltiwanger JT, Andrellos PJ. 'Pediatric Evaluation of Disability Inventory' (PEDI). Development, standardization and administration manual. Boston, MA: New England Central Center Inc. and PEDI Research group; 1992.

17 Custers JWH. Pediatric Evaluation of Disability Inventory, the Dutch adaptation [Dissertation]. Utrecht (the Netherlands): University Utrecht; 2001.

18 Custers JWH, Wassenberg-Severijnen JE, Net J van der, Vermeer A, Hart HT, Helders PJM. Dutch adaptation and content validity of the 'Pediatric Evaluation of Disability Inventory'. Disabil Rehabil 2002; 24(5):250-258.

19 Glass CAW, Verhelst ND. Tests of fit for polytomous Rasch models. Rasch models. Foundation, recent developments and applications. New York: Springer; 1995:325-352.

20 Dahl M, Ahlsten G, Butler A, Norrlin S, Strinnholm M, Winberg A. Self-care skills in young children with myelomeningocele. Eur J Pediatr Surg 2000; 10 (Suppl 1):52-53.

21 Tsai PY, Yang TF, Chan RC, Huang PH, Wong TT. Functional investigation in children with spina bifida - measured by the Pediatric Evaluation of Disability Inventory (PEDI). Child's Nerv Syst 2002; 18:48-53.

22 Norrlin S, Strinnholm M, Carlson M, Dahl M. Factors of significance for mobility in children with myelomeningocele. Acta Pediatr 2003; 92(2):204-210.

23 Parkin PC, Kirpalani HM, Rosenbaum PL, Fehlings DL, Van Nie A, Willan AR, King D. Development of a health-related quality of life instrument for use in children with spina bifida. Qual Life Res 1997; 6:123-132.

24 Kirpalani HM, Parkin PC, Willan AR, Fehlings DL, Rosenbaum PL, King D, Van Nie AJ. Quality of life in spina bifida: importance of parental hope. Arch Dis Child 2000; 83(4):293-297.

25 Pit-Ten Cate IM, Kennedy C, Stevenson J. Disability and quality of life in spina bifida and hydrocephalus. Dev Med Child Neurol 2002; 44:317-322.

26 Padua L, Rendeli C, Rabini A, Girardi E, Tonali P, Salvaggio E. Health-related quality of life and disability in young patients with spina bifida. Arch Phys Med Rehabil 2002; 83(10):1384-1388.

27 Verbrugge LM, Jette AM. The disablement process. Soc Sci Med. 1994; 38(1):1-14.

28 Jette AM. Physical disablement concepts for physical therapy research and practice. Phys Ther 1994; 74(5): 380-386.

29 Jette AM, Keysor JJ. Disability models: Implications for arthritis exercise and physical activity interventions. Arthritis Rheum 2000; 49(1):114-120.

30 ICF International Classification of Functioning, Disability and Health. Geneva: World Health Organization; 2001.

31 Helders PJM, Engelbert RHH, Gulmans VAM, Net J van der. Paediatric rehabilitation. Disab Rehabil 2001; 23(11):497-500.

32 Stuberg W, Harbourne R. Theoretical practice in peadiatric physical therapy: past, present and future considerations. Paediatr Phys Ther 1994; 6:119-125.

33 Law M, Darrah J, Pollock N, King G, Rosenbaum P, Russell D, Palisano R, Harris S, Armstrong R, Watt J. Family-centered functional therapy for children with cerebral palsy: an emerging practice model. The Haworth Press; 1998: 83-101.

34 Ketelaar M, Vermeer A, Hart H 't, Petegem-van Beek E, Helders PJM. Effects of a functional therapy program on motor abilities of children with cerebral palsy. Phys Ther 2001; 81(9):1534-1545.

35 Jansen LMC, Ketelaar M, Vermeer A. Parental experience of participation in physical therapy for children with physical disabilities. Dev Med Child Neurol 2003; 45(1):58-69.

36 Thelen E, Smith LB. A dynamic system approach to the development of cognition and action. Cambridge, Mass: MIT Press; 1995.

37 Kaufman BA, Terbock A, Ito J, Klosterman A, Park TS. Disbanding a multidisciplinary clinic: effects on the health care of myelomeningocele patients. Pediatr Neurosurg 1994; 21 (1):36-44.

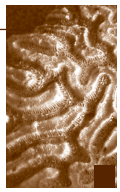
NEDERLANDSE SAMENVATTING

(DUTCH SUMMARY)

MAGC Schoenmakers

Door verbeteringen binnen de medische zorg bereiken steeds meer kinderen met spina bifida de volwassen leeftijd. De invloed van deze chronische aandoening op het functioneren van een individu kan zichtbaar gemaakt worden aan de hand van het zogenaamde 'disablement' model. De term 'disablement' verwijst daarbij naar de gevolgen van een aandoening op het lichamelijke, persoonlijke en maatschappelijke functioneren. Het 'disablement' model onderscheidt verschillende domeinen, zoals 'ziekte' ('pathology'), 'stoornissen' ('impairments'), 'beperkingen in vaardigheden' ('functional limitiations') en 'beperkingen in activiteiten van het dagelijkse leven' ('disability'). De meeste studies betreffende spina bifida beperken zich tot een à twee domeinen, zoals de relatie tussen de aandoening en het fysieke functioneren, de cognitieve mogelijkheden of de psychosociale aspecten. Uit deze studies is gebleken het dat het hebben van spina bifida een enorme invloed heeft op de gezondheid van een individu. Er is echter nog weinig bekend over met gezondheid samenhangende kwaliteit van leven ('KvL').

Het belangrijkste doel van dit proefschrift is om meer inzicht te verkrijgen van in de onderlinge relaties tussen de verschillende domeinen van het 'disablement' proces, bij kinderen met spina bifda. Door het verkregen inzicht in de onderlinge relaties tussen 'ziekte', 'stoornissen', 'beperkingen in vaardigheden en activiteiten' en 'KvL', kan beter richting gegeven worden aan therapiedoelstellingen. Tot voor kort was de fysiotherapeutische behandeling van kinderen met spina bifida voornamelijk gericht op het niveau van 'stoornissen', zoals bijvoorbeeld het verbeteren van de spierkracht, het verbeteren van de spierspanning en het bestrijden van contracturen, met als doel het functioneren van deze kinderen te optimaliseren. Het is echter nog onvoldoende bekend hoe deze 'stoornissen' zich verhouden tot zelfredzaamheid en 'KvL'. Omdat fysiotherapeutische begeleiding voor zowel kinderen als hun ouders vaak langdurig en intensief is, is het belangrijk om te weten welke factoren medebepalend zijn voor de mate van zelfredzaamheid. Alleen door kennis te hebben van deze determinanten kunnen realistische therapiedoelstellingen geformuleerd worden.



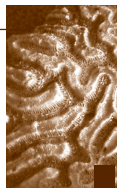
Inleiding

In hoofdstuk 1 wordt een korte inleiding gegeven over de verschillende uitingsvormen van spina bifida. Ook worden in het kort de ontwikkelingen binnen de medisch zorg in de afgelopen decennia belicht. De symptomen van spina bifida occulta (verborgen spina bifida) en spina bifida aperta (open spina bifida), met name de meningomyelocele (MMC), worden besproken. MMC is de meest ernstige vorm van spina bifida, waarbij er aanlegstoornissen zijn van zowel de hersenen als het ruggenmerg. Hierdoor kunnen er naast motorische problemen ook cognitieve functiestoornissen zijn. Bij de occulte vormen van spina bifida betreft het meestal alleen aanlegstoornissen van het ruggenmerg die leiden tot motorische en/of incontinentieproblemen. Verder worden de drie belangrijkste 'disablement' concepten kort besproken, terwijl dieper ingegaan wordt op het model dat wij als uitgangspunt gekozen hebben voor dit proefschrift. De mogelijke gevolgen van spina bifida worden belicht binnen de verschillende domeinen, zoals 'ziekte', 'stoornissen', 'beperkingen in vaardigheden en activiteiten van het dagelijkse leven' en de invloed op 'KvL'. Tenslotte worden de doelstellingen van dit proefschrift geformuleerd.

'Disablement' proces

In hoofdstuk 2 beschrijven we de klinische karakteristieken van 177 kinderen met spina bifida, in de leeftijd van 1-18 jaar. In deze cross-sectionele studie zijn twee groepen kinderen met elkaar vergeleken; kinderen met een MMC (n = 122) versus kinderen met een overige vorm van spina bifida (OVSB, n = 55). Het betreft: a) parameters op het niveau van 'ziekte' ('pathology'), zoals de aanwezigheid van geassocieerde afwijkingen aan het centrale zenuwstelsel en het laesieniveau; b) parameters op het niveau van 'stoornissen' ('impairments'), zoals de mentale status, de bewegingsmogelijkheid

van de gewrichten en de spierkracht; c) parameters op het niveau van 'beperkingen in vaardigheden' ('functional limitations') zoals ambulantieniveau, scores betreffende functionele vaardigheden gemeten met de 'Pediatric Evaluation of Disability Inventory' (PEDI) en d) parameters op het niveau van 'disability', zoals de mate van ouderondersteuning die nodig is bij deze functionele vaardigheden, eveneens gemeten met de PEDI. Ook scores betreffende het zelfbeeld en de 'KvL' zijn binnen de twee genoemde groepen met elkaar vergeleken. Binnen de MMC groep is gekeken naar de verschillen in scores tussen kinderen met en zonder hydrocephalus. Daarnaast hebben we de onderlinge relaties bestudeerd tussen 'ziekte', 'stoornissen', 'beperkingen in vaardigheden en activiteiten', zelfbeeld en 'KvL'. Het ambulantieniveau was statistisch significant geassocieerd met de spierkracht in de onderste extremiteiten (hoe hoger de spierkracht, des te hoger het ambulantieniveau) en met het laesieniveau (hoe lager het laesieniveau, des te hoger het ambulantieniveau). Wij vonden dat kinderen met een MMC die tevens een hydrocephalus hadden, significant meer stoornissen hadden en meer beperkingen in hun vaardigheden en activiteiten, dan met kinderen met een OVSB. Bovendien scoorden kinderen met een MMC in combinatie met een hydrocephalus significant lager betreffende hun 'KvL'. Desalniettemin waren de scores betreffende het zelfbeeld niet significant verschillend tussen beide groepen. 'KvL' was significant positief geassocieerd met ambulantie, functionele vaardigheden en mentale status, terwijl scores betreffende het zelfbeeld vrijwel geen significante associaties vertoonden met deze variabelen. Wij zijn van mening dat hulpverleners hun aandacht niet hoofdzakelijk moeten richten op kinderen met de meest ernstige vorm van spina bifida (MMC), vooral waar het hun psychosociale begeleiding betreft. Naar ons idee is psychosociale ondersteuning voor kinderen met een OVSB wellicht net zo hard nodig als voor kinderen met een MMC.



Determinanten voor zelfredzaamheid en kwaliteit van leven.

In hoofdstuk 3 is bij 122 kinderen met een MMC onderzocht welke factoren van het 'disability' proces determinanten zijn voor hun zelfredzaamheid, vooral betreffende hun zelfverzorging en het zich kunnen verplaatsen. Met betrekking tot 'KvL' wilden we weten welke functionele vaardigheden het meest bepalend zijn voor 'KvL' (goede ambulantie, goede handfunctie, zelfstandigheid op het gebied van zelfverzorging, het zich kunnen verplaatsen, en/of sociaal functioneren). Uit onze bevindingen blijkt er dat bij kinderen met een MMC een significante relatie bestaat tussen parameters op het 'impairment' niveau en parameters op het 'disability' niveau. Zelfstandigheid op het gebied van de zelfverzorging was sterk geassocieerd met het laesieniveau (onder L3), de mentale status (IQ ≥ 80), de afwezigheid van contracturen en goede spierkracht in de benen (vooral van de knie-extensoren). Deze factoren waren afhankelijk van elkaar. De belangrijkste determinanten voor het zich zelfstandig kunnen verplaatsen waren de mentale status, de afwezigheid van contracturen en een goede spierkracht in de benen. Voor de 'KvL' is het zich onafhankelijk kunnen verplaatsen van groter belang dan zelfredzaamheid op het gebied van de zelfverzorging of het al dan niet rolstoelgebonden zijn. Deze informatie is belangrijk voor het opstellen van realistische behandeldoelen.

Sacrale spina bifida.

In hoofdstuk 4 zijn in twee groepen kinderen met een sacrale spina bifida (MMC versus lipomeningomyelocele (LMMC)) de spierkracht, het ambulantieniveau, de motorische vaardigheden en de functionele vaardigheden betreffende zelfverzorging, het zich kunnen verplaatsen en sociaal functioneren onderzocht. Vervolgens zijn beide groepen zijn met elkaar vergeleken. Daarnaast zijn de scores van de motorische en de functionele vaardigheden vergeleken met referen-

tiewaarden van de normale populatie. Alle kinderen in de MMC groep (n = 30) hadden een gedraineerde hydrocephalus en een Chiari II malformatie. Geen van de kinderen in de LMMC groep (n = 14) had deze bijkomende afwijkingen aan het centraal zenuwstelsel (CZS). Wij constateerden dat er bij kinderen met een sacrale spina bifida meer aan de hand was dan lopen met een 'schoonheidsfoutje'. Hoewel zij over het algemeen goed ambulant waren, zagen we in beide groepen problemen op het gebied van de grove motoriek en de functionele vaardigheden. Problemen met de fijne motoriek deden zich alleen voor in de MMC groep. Spierzwakte en motorische problemen, vooral met betrekking tot het evenwicht, waren het meest ernstig in de MMC groep in vergelijking met de LMMC groep. Wat betreft het ambulantieniveau en de functionele vaardigheden (PEDI-scores) verschilden de beide groepen niet significant van elkaar. Beide groepen waren langer aangewezen op de hulp van derden dan leeftijdsgenoten, vooral op het gebied van continentie. Beide groepen hadden langdurig hulp of ouderondersteuning nodig bij de zelfverzorging, vooral waar het catheriseren en colonspoelen betrof. Goede voorlichting en specifieke begeleiding op deze fronten zijn noodzakelijk om te voorkomen dat kinderen met een sacrale spina bifida, juist zij die regulier onderwijs volgen, zich een buitenbeentje gaan voelen.

De invloed van 'untethering' op laesie- en ambulantieniveau.

In hoofdstuk 5 zijn bij 44 patiënten in de leeftijd van 1 - 18 jaar (MMC, n = 16; LMMC, n = 9; OVSB, n = 19) op prospectieve wijze, de lange termijn gevolgen bestudeerd die een ontkluisterende ruggenmergoperatie ('untethering') heeft op het laesieniveau en op het ambulantieniveau. Het laesie- en ambulatieniveau zijn direct vóór, en drie keer na de operatie onderzocht (met een gemiddelde follow-up duur van 7 jaar en 1 maand). Wij wilden ook inzicht krijgen in de risicofactoren voor zowel het achteruitgaan in ambulantie als het optreden van

recidief verklevingen ('retethering'). De resultaten van dit onderzoek toonden aan dat na een ontkluistering van het ruggenmerg bij de meerderheid van de patiënten zowel het laesieniveau als het ambulantieniveau stabiel bleven. Achteruitgang op de lange termijn (langer dan 4 jaar na de operatie) werd alleen gezien bij patiënten met een (lipo)meningomyelocele. Overgewicht en het ontstaan van recidief verklevingen waren significante risicofactoren voor achteruitgang in het ambulantieniveau. Het optreden van 'retethering' werd voornamelijk gezien bij patiënten met een LMMC, hoewel de aanwezigheid van een lipoom op zichzelf geen statistisch significante risicofactor was. Zorgvuldige multidisciplinaire controle is noodzakelijk om tijdig het ontstaan van neurologische veranderingen te detecteren, waarop de indicatie voor chirurgische interventie bepaald kan worden.

De invloed van operatieve wervelkolomfixatie op ambulantie en functionele vaardigheden.

In hoofdstuk 6 zijn bij 10 kinderen met een MMC, op prospectieve wijze, de functionele gevolgen van een operatieve correctie van standafwijkingen aan de wervelkolom bestudeerd. Een progressieve scoliose leidt over het algemeen tot instabiliteit van de romp, wat weer negatieve gevolgen kan hebben voor de zitbalans, de functionele vaardigheden en de ambulantie. Een operatieve fixatie c.q. stabilisatie van de wervelkolom heeft tot doel deze functies zoveel mogelijk te behouden, dan wel te optimaliseren. In dit onderzoek zijn het ambulantieniveau en de functionele vaardigheden direct vóór, en drie keer na de operatie onderzocht. De functionele vaardigheden op het gebied van de zelfverzorging en het zich kunnen verplaatsen, evenals de mate van hulp c.q. ouderondersteuning die bij deze vaardigheden nodig zijn, zijn gemeten met behulp van de PEDI. Na de operatie was de ernst van de scoliose (de grootte van de primaire bocht) significant afgenomen. De mate van bekkenscheefstand en de mate van rompde-

viatie veranderden niet significant. In de eerste 6 maanden na de operatie (waarvan de eerste drie maanden immobilisatie in een rompcorset) hadden de kinderen significant meer hulp nodig bij hun zelfverzorging en het zich kunnen verplaatsen in vergelijking met vóór de operatie. Deze hulp was vooral nodig bij het aan- en uitkleden van zowel het boven- als onderlichaam, bij catheriseren en colonspoelen en bij het maken van transfers. Na 12 maanden waren de scores terug op het preoperatieve niveau en na een periode van anderhalf jaar werd zelfs een stijging gezien ten opzichte dit niveau. Na de operatieve fixatie van de wervelkolom was lopen niet meer goed mogelijk voor die kinderen hier die voor de operatie vaak alleen in een 'therapiesituatie' konden lopen. Deze informatie is van belang voor hulpverleners teneinde ouders van kinderen bij wie een wervelkolomfixatie wordt overwogen, adequaat te kunnen informeren en begeleiden.

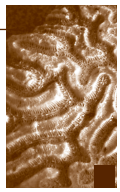
Conclusies

Op basis van de onderzoeksresultaten kunnen de volgende conclusies worden getrokken:

1 Bij kinderen met spina bifida zijn parameters op het niveau van 'stoornissen' ('impairments') statistisch significant gecorreleerd met 'beperkingen in activiteiten van het dagelijkse leven' ('disability').

2 Het ambulantieniveau vertoont een positieve statistisch significante samenhang met de spierkracht van de heupabductoren en de voetheffers.

3 Voor de zelfredzaamheid zijn een goede spierkracht, de afwezigheid van contracturen en goede mentale mogelijkheden belangrijker dan andere ziektekenmerken van spina bifida.



4 Een goede spierkracht (vooral van de knie-extensoren), de afwezigheid van contracturen en de mentale mogelijkheden blijken een belangrijkere bijdrage te leveren aan de zelfredzaamheid ten aanzien van zich kunnen verplaatsen, dan het laesieniveau of het al dan niet aanwezig zijn van een (gedraineerde) hydrocephalus.

5 Onafhankelijk zijn ten aanzien van zich kunnen verplaatsen draagt meer bij aan de 'kwaliteit van leven', dan het al dan niet rolstoelgebonden zijn of onafhankelijkheid op het gebied van de zelfverzorging.

6 Jonge kinderen met spina bifida (£ dan 7.5 jaar) scoren op de verschillende domeinen van de PEDI binnen de grenzen van normaal, maar onder het gemiddelde. Een uitzondering vormen de scores op het gebied van zich kunnen verplaatsen, waarop lager dan normaal wordt gescoord. Bij oudere kinderen (> 7.5 jaar) zijn de scores op alle domeinen afwijkend.

7 De meeste kinderen met een lage (sacrale) spina bifida hebben problemen met grof motorische vaardigheden; kinderen met een meningomyelocele meer dan kinderen met een lipomeningomyelocele. Beide groepen hebben meer ouderondersteuning nodig bij hun zelfverzorging dan leeftijdsgenoten.

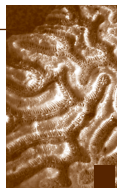
8 Hoewel kinderen met de meest ernstige vorm van spina bifida (MMC) significant meer beperkingen hebben dan kinderen met overige vormen van spina bifida, zijn scores betreffende het zelfbeeld niet significant verschillend tussen beide groepen.

9 sychosociale ondersteuning is voor kinderen met overige vormen van spina bifida waarschijnlijk net zo hard nodig als voor kinderen met een meningomyelocele.

10 Een operatieve ontkluistering van het ruggenmerg voorkomt bij de meeste patiënten progressieve neurologische uitval en achteruitgang in ambulantie. Achteruitgang in ambulantie en het optreden van 'retethering' worden voornamelijk gezien bij patiënten met een (lipo)meningomyelocele en niet bij patiënten met overige vormen van spina bifida.11 De grootte van de primaire bocht van een scoliose vermindert significant na een operatieve fixatie c.q. stabilisatie van de wervelkolom.

12 In de eerste 6 maanden na de operatie is significant meer hulp nodig op het gebied van de zelfverzorging en het zich kunnen verplaatsen. Na 12 maanden zijn de scores terug op het preoperatieve niveau en na 18 maanden is er een verbetering te constateren ten opzichte van de preoperatieve situatie.

13 Na een operatieve fixatie van de wervelkolom is lopen niet meer mogelijk voor dié kinderen die daarvoor alleen in een 'therapiesituatie' konden lopen.



AANBEVELINGEN VOOR TOEKOMSTIG ONDERZOEK

1 Om inzicht te krijgen in de dynamiek van de onderlinge relaties tussen de verschillende domeinen van het 'disablement' proces bij kinderen met spina bifida, zijn longitudinale cohortstudies van verschillende leeftijdsgroepen noodzakelijk. Het is belangrijk om de parameters op deze verschillende domeinen prospectief te bestuderen, waarbij de huidige 'International Classification of Functioning, Disability and Health (ICF) uitgangspunt kan zijn.

2 Longitudinale cohortstudies zijn noodzakelijk om de effectiviteit van de huidige fysiotherapeutische interventies te beoordelen, wat betreft preventie contractuurvorming en het verbeteren van de spierfunctie in relatie tot het vergroten van de zelfredzaamheid op het gebied van het zich kunnen verplaatsen. In deze toekomstige studies is stratificatie op grond van de spierfunctie gewenst, in tegenstelling tot stratificatie op grond van het laesieniveau. Daarnaast is het van belang om te bestuderen welke therapievorm bij kinderen met spina bifida de meest effectieve vorm is om functieverbetering te bewerkstelligen; de traditionele vorm van therapie, waarbij 'stoornissen' (contractuurpreventie en spierkrachtverbetering) het uitgangspunt zijn om functie te verbeteren of 'functionele fysiotherapie', waarbij het trainen van een specifieke vaardigheid het primaire uitgangspunt is. Deze vorm kan eveneens resulteren in verbetering van de spierfunctie en/of afname van contracturen.

3 Het ontwikkelen van een ziektespecifiek (PEDI-)profiel van functionele vaardigheden is noodzakelijk. Met behulp van Rasch analyses kunnen binnen de PEDI veranderingen op itemniveau bestudeerd worden, in plaats van op domeinniveau. Een ziekte-

specifiek PEDI-profiel geeft inzicht in de behoeften van een kind, in vergelijking met zijn of haar leeftijdsgenoten die dezelfde aandoening hebben.

4 Adaptatie van de Nederlandse vertaling van de 'Spina Bifida Health Related Quality of Life Questionnaire' is noodzakelijk, voordat deze gebruikt kan worden voor het evalueren van therapeutische interventies.

DANKWOORD

Een proefschrift komt nooit tot stand zonder hulp van anderen. Bij de afronding ervan wil ik een aantal van hen in het bijzonder bedanken:

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Promotor, Prof. dr. AHJ Prevo, beste Arie, ik dank je voor je waardevolle bijdrage bij de verschillende onderdelen van dit manuscript.

Copromotor, Dr. VAM Gulmans, beste Vincent, met jouw analytische vermogen en rustige benadering heb jij mijn onrustige brein door de grote brei van data heen geloodst. Je vertrek uit het UMC Utrecht was een logische stap in je carrière. Het feit dat je mijn begeleiding daarna gecontinueerd hebt, was niet zo vanzelfsprekend. Ik had geen betere procesbegeleider kunnen hebben. Met het gereed komen van dit proefschrift komt er een einde aan onze samenwerking, maar niet aan de vriendschap die gaande het traject is ontstaan.

Copromotor, Dr. RHJM Gooskens, beste Rob, jij staat bekend om je grote betrokkenheid bij alles wat met spina bifida te maken heeft. De wijze waarop jij de ouders van pasgeboren patiëntjes informeert over de ernst van deze aandoening, dwingt respect af. Onze vele stimulerende discussies zijn vormend geweest voor mij. Ik hoop nog lang met je samen te mogen werken binnen het spina bifida team en op het Sylvia Tóth Centrum.

Ik wil alle kinderen en hun ouders die aan het onderzoek hebben meegedaan bedanken voor de medewerking en het geduld waarmee de vele vragenlijsten beantwoord zijn. Veel dank ben ik verschuldigd

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Drs. Femke Kooijmans en Sonja van de Pol. Jullie vriendschap is mij erg tot steun geweest en ik vind het geweldig dat jullie mij op 9 september ter zijde willen staan. Femke, de wijze waarop jij de patiëntenzorg op afdeling Kikker gecontinueerd hebt, is fantastisch geweest.

Mijn collegae van de afdeling kinderfysiotherapie hebben allen op hun eigen wijze bijgedragen aan dit proefschrift. Dr. Janjaap van der Net, Dr. Raoul Engelbert, Dr. Jan Custers, Dr. Tim Takken, jullie zijn mij voorgegaan in de wetenschappelijke vorming. Extra dank ben ik verschuldigd aan mijn kamergenoten, Drs. Ron van Empelen, Rian Eijsermans, Anneke de Klerk, Femke, Maartje Wormgoor en Patrick

van der Torre. Als ik jullie moet geloven is dit proefschrift kreunend tot stand gekomen. Tijdens de momenten van opperste concentratie achter de computer vergeet ik kennelijk om rustig door te ademen. Maar zolang mijn gekreun niet het aantal decibellen overschrijdt dat toptennissers produceren, is er geen sprake van geluidshinder. Annemiek Apeldoorn, Sonja Raaff en Carla van Rooijen, jullie hebben zelfs een steekwagentje aangeschaft om de vele statussen van het medische archief naar mijn werkplek te vervoeren. En dan te bedenken dat de meeste kinderen met spina bifida minstens twee dossiers hebben! Dr. Jan Willem Gorter, jij 'roomt-in' op onze afdeling en daar moet je het stellen met een 'flex-plek'. Vanaf die plaats zijn prikkelende discussies tot stand gekomen. Ik bewonder je gedrevenheid en hoop in de toekomst met je samen te werken aan nieuwe spina bifida onderzoeksprojecten.

Mijn collegae van het spina bifida team, Dr. Pieter Dik, Dr. Rob Gooskens, Dr. Patrick Hanlo, Dr. Teau de Jong, Madelon Kleingeld, Carla Nesselaar, Dr. Hans Pruijs en Drs. Marja van Tol, bedank ik voor de prettige samenwerking in de afgelopen jaren. Ik denk hierbij ook aan diegene, zonder wie de werkgroep nooit tot stand gekomen zou zijn: Dr. Wim Keessen, Prof. dr. Jan van Gool en Drs. Hesther van Wieringen. Henny Huttinga, jou wil ik met name bedanken. Jij hebt ervoor gezorgd dat alle afspraken voor mijn onderzoek samen vielen met de controles op de spina bifida poli, waardoor de patiënten niet extra hoefden te reizen.

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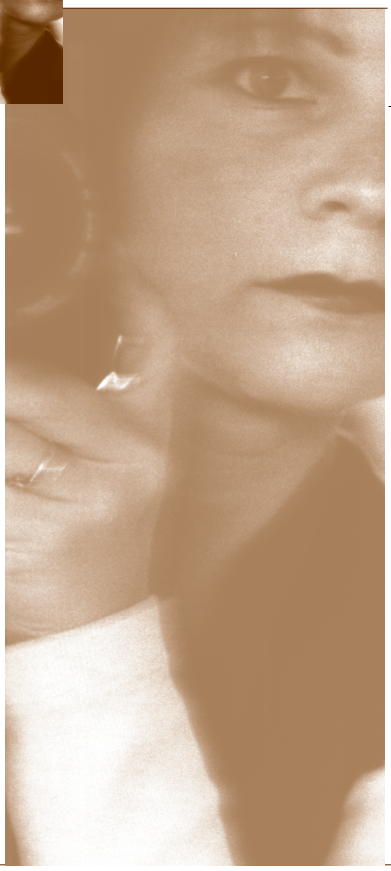
Curriculum Vitae



De auteur is op 8 december 1959 te Kaatsheuvel geboren. Zij behaalde in 1977 haar diploma aan de Maris Stella - HAVO te Dongen. Van 1977 tot 1981 werd de opleiding fysiotherapie gevolgd aan de Stichting Academie voor Fysiotherapie West-Brabant te Breda.
Van 1981 tot 1985 was zij werkzaam in een particuliere praktijk voor fysiotherapie te Breda. In deze periode volgde zij diverse applicatiecursussen op het gebied van neurologische oefenmethoden (1983) en orthopedische afwijkingen (1984).
In 1985 werd zij hoofd van de afdeling fysiotherapie van 'de Merwebolder', een instituut voor meervoudig gehandicapten te Sliedrecht, waar zij tot augustus 1990 werkzaam was. In deze periode (van 1986 tot 1989) volgde zij de 3-jarige Post Academiale Scholing Kinderfysiotherapie aan de Hogeschool Midden Nederland te Utrecht.
Vanaf augustus 1990 tot heden is zij werkzaam op de afdeling kinderfysiotherapie van het Wilhelmina Kinderziekenhuis, Universitair Medisch Centrum Utrecht (hoofd: Prof. dr. PJM Helders) en vanaf die tijd is zij lid van het spina bifida team. In 1993 volgde een studiebezoek aan de 'Division of Congenital Defects, Children's Hospital and Medical Center' in Seattle (hoofd: Prof. DB Shurtleff).
Specifieke wetenschappelijke scholing vond plaats via de basis module 'Onderzoeksmethodiek en Statistiek' van de Hogeschool Midden Nederland te Utrecht (1992) en via de Open Universiteit. Daar behaalde zij de certificaten 'Methoden en Technieken van Sociaalwetenschappelijk onderzoek 1 & 2' (1995, 1997) en in 1999 werd het certificaat 'Statistiek 1' behaald. In januari 1999 werd gestart met het onderzoek dat deel uitmaakt van dit proefschrift.
De auteur is getrouwd met Rob Heijne den Bak.

LIST OF PUBLICATIONS

Engelbert RHH, Schoenmakers MAGC. Cave distale diplegie. Ned Tijdschr Fysioth 1990; 100 (11):291-293.

Engelbert RHH, Schoenmakers MAGC, Net J van der, Keessen W, Helders PJM. Mijn dochtertje wil niet meer op haar beentjes staan. Fysiopraxis 1992; 14:2-4.

Engelbert RHH, Net J van der, Schoenmakers MAGC, Keessen W, Helders PJM. Twee kinderen met discitis: casuïstische mededeling. Ned Tijdschr Geneeskd 1993; 137(32):1614-1616.

Empelen R van, Schoenmakers MAGC, Net J van der, Helders PJM. Tonus onderzoek bij kinderen: objectivering door gebruik van een meetinstrument. Fysiopraxis 1994; 15:20-23.

Engelbert RHH, Schoenmakers MAGC, Haastert IC van. Loopstoeltjes: Panacee of Plaag? Ned Tijdschr Kindergeneeskd 1994; 62 (6): 277-279.

Vanpaemel GADM, Schoenmakers MAGC, Nesslerooij BPM, Pruijs JEH, Helders PJM. Multiple Congenital Contractures. J Pediatr Orthop (part B) 1997; 6:172-178.

Empelen R van, Schoenmakers MAGC, Zee DC van der. Biofeedbacktraining ter verbetering van anale incontinentie na een traumatische ruptuur van de sfincter ani. Fysiopraxis 1998; 6:11-14.

Schoenmakers MAGC, Gulmans VAM, Bax NMA, Helders PJM. Physiotherapy as an adjuvant to the surgical treatment of the anterior chest wall deformities. Eur J Pediatr Surg 2000; 35(10):1440-1443.

Schoenmakers MAGC, Gulmans VAM, Helders PJM, Berg HM van den. Motor performance and disability in Dutch children with haemophilia: a comparison with their healthy peers. Haemophilia 2001; 7:293-298.

Schoenmakers MAGC, Gooskens RHJM, Gulmans VAM, Uiterwaal CSPM, Prevo AJH, Helders PJM. Disablement process in children with spina bifida: impairments, functional limitations, disability, perceived competence and quality of life. Accepted for publication in Disability and Rehabilitation 2003.

Schoenmakers MAGC, Gooskens RHJM, Gulmans VAM, Uiterwaal CSPM, Prevo AJH, Helders PJM. Determinants of functional independence and quality of life in children with spina bifida: a physical therapy perspective. Submitted for publication.

Schoenmakers MAGC, Gulmans VAM, Gooskens RHJM, Helders PJM. Sacral myelomeningocele: more than minor gait-disturbances. Accepted for publication in Clinical Rehabilitation 2003.

Schoenmakers MAGC, Gooskens RHJM, Gulmans VAM, Hanlo PW, Vandertop WP, Uiterwaal CSPM, Helders PJM. Long-term outcome of neurosurgical untethering on neurosegmental motor level and ambulatory status. Dev Med Child Neurol 2003; 45:551-555.

Schoenmakers MAGC, Gulmans VAM, Gooskens RHJM, Pruijs JEH, Helders PJM. Spinal fusion in children with spina bifida: influence on ambulation level and functional abilities. Submitted for publication.

Chapters in books

Schoenmakers MAGC, Janssen G. Perifeer neurologische aandoeningen: Fysiotherapie bij kinderen met spina bifida. In: Leerboek Kinderfysiotherapie, onder redactie van Empelen R van, Nijhuis-van der Sanden R, Hartman A (ISBN 90 352 2271 7). Maarssen: Elsevier gezondheidszorg, 2000; 365-382.

Engelbert RHH, Schoenmakers MAGC, Vlimmeren L van. Kinderorthopedie: Kinderfysiotherapie in relatie tot de kinderorthopedie. In: Leerboek Kinderfysiotherapie, onder redactie van Empelen R van, Nijhuis- van der Sanden R, Hartman A (ISBN 90 352 2271 7). Maarssen: Elsevier gezondheidszorg, 2000; 251-271.

Schoenmakers MAGC, Vanpaemel GADM, Pruijs JEH. Arthrogryposis Multiplex Congenita: Multipele Congenitale Contracturen. In: Kinderrevalidatie, onder redactie van Meihuizen - de Regt MJ, Moor JMH de, Mulders AHM (ISBN 90 232 3799 4). Assen: Van Gorcum, derde herziene druk, 2003; 464-473.

Abstracts

Net J van der, Engelbert RHH, Schoenmakers MAGC, Keessen W. Physiotherapy following total hip replacement; a multiple case report. In: Proceedings of the Second International Workshop of Physiotherapy in Juvenile Chronic Arthritis, Garmisch-Partenkirchen (D) 1992:105.

Vanpaemel GADM, Schoenmakers MAGC, Nesselrooij BPM van, Keessen W. A pilot study into Arthrogryposis Multiplex Congenita. In: Proceedings of the First International Arthrogryposis Group Conference, Bristol (U.K.), 1993:18-26.

Schoenmakers MAGC, Engelbert RHH, Keessen W, Helders PJM. A quality assessment on chest fysiotherapy following spondylodesis according to Luque. In: Proceedings of the NIVEL-international conference, Den Bosch, 1994.

Schoenmakers MAGC, Bax NMA, Helders PJM. Preoperatieve fysiotherapeutische behandeling bij chirurgische correctie van thoraxwanddeformiteiten: een noodzaak? In: Proceedings Chirurgendagen, Nederlandse Vereniging voor Heelkunde, Veldhoven, 1995: 33.

Schoenmakers MAGC, Engelbert RHH, Helders PJM. Rugklachten op de kinderleeftijd; kinderfysiotherapeutische kanttekeningen. In: Proceedings Jaarcongres Fysiotherapie, Den Haag, 1995.

Schoenmakers MAGC, Vanpaemel GADM, Nesselrooij BPM, Pruijs JEH, Helders PJM. Multipele Congenitale Contracturen; een cross-sectionele studie van achtentwintig kinderen. In: Proceedings congres Nederlandse Vereniging voor Fysiotherapie in de Kinder- en Jeugdgezondheids zorg, Veldhoven, 1997: 76-77.

Custers JWH, Wassenberg J, Schoenmakers MAGC, Net J van der, Hoijtink H, Helders PJM. Betrouwbaarheidsstudies bij de ontwikkeling van de Nederlandse versie van de PEDI. In: Proceedings Jaarcongres Fysiotherapie, Den Haag, 1999:106.

Schoenmakers MAGC, Gulmans VAM, Gooskens RHJM, Helders PJM. Sacral myelomeningocele: more than minor gait-disturbances. In: Proceedings of the Uppsala meeting of the Society for Research into Hydrocephalus and Spina Bifida, 2001. Eur J Pediatr Surg 2001; 11 (Suppl I): S65.

Schoenmakers MAGC, Gooskens RHJM, Gulmans VAM, Hanlo PW, Vandertop WP, Uiterwaal CSPM, Helders PJM. Long-term outcome of neurosurgical untethering on neurosegmental motor level and ambulatory status. In: Proceedings of the Leuven meeting of the Society for Research into Hydrocephalus and Spina Bifida, 2002. Eur J Pediatr Surg 2002; 12 (Suppl I):S60.

Schoenmakers MAGC. Determinanten voor zelfredzaamheid en kwaliteit van leven bij kinderen met spina bifida. In: Proceedings congres Nederlandse Vereniging voor Fysiotherapie in de Kinder- en Jeugdgezondheids zorg, Veldhoven, 2003: 52.