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RESEARCH PAPER



# Psychological well-being in adults with spinal muscular atrophy: the contribution of participation and psychological needs

Maarten J. Fischer<sup>a</sup>, Fay-Lynn Asselman<sup>b</sup>, Esther T. Kruitwagen-van Reenen<sup>a</sup>, Marjolein Verhoef<sup>a</sup>, Renske I. Wadman<sup>b</sup>, Johanna M. A. Visser-Meily<sup>a</sup>, W. Ludo van der Pol<sup>b</sup> and Carin D. Schröder<sup>a</sup>

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## ABSTRACT

**Purpose:** Patients with spinal muscular atrophy (SMA) suffer from slowly progressive weakness of axial, respiratory and proximal muscles, leading to restrictions in activity and participation. This study aims to investigate patients' level of psychological well-being, using the International Classification of Functioning model and self-determination theory as theoretical frameworks.

**Materials and methods:** In this cross-sectional study, adults with SMA were invited to complete a questionnaire. Instruments to assess psychological well-being included the Satisfaction with Life Scale, the Rosenberg Self-Esteem Scale and the Positive and Negative Affect Scale. Hierarchical lineal regression analyses were performed to investigate the contribution of participation (International Classification of Functioning model) and satisfaction of the need for autonomy, competence and relatedness (self-determination theory) to well-being.

**Results:** Ninety-two respondents (67%) returned the questionnaire. Levels of psychological well-being were comparable to that of healthy reference samples. Well-being was unrelated to sociodemographic variables or illness characteristics. By contrast, well-being was closely related to respondents' satisfaction with participation, and their sense of autonomy, competence and relatedness.

**Conclusions:** This study illustrates the relevance of psychological needs for understanding well-being of individuals with SMA. Supporting patients in meeting their psychological needs should become an objective of person-centred care for this population.

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## KEYWORDS

International Classification of Functioning; participation; self-determination theory; spinal muscular atrophy; well-being

## ► IMPLICATIONS FOR REHABILITATION


- Spinal muscular atrophy is a rare inherited disease, characterized by slowly progressive muscle weakness.
- Psychological well-being, including satisfaction with life, self-esteem and emotional functioning of adults with spinal muscular atrophy appears very comparable with that of healthy reference samples.
- In line with the International Classification of Functioning framework, well-being in adults with spinal muscular atrophy may be improved by increasing their (satisfaction with) participation.
- Moreover, clinical assessment and management should focus on optimizing patients' satisfaction with their basic psychological needs (autonomy, competence, relatedness), as this is strongly related to indices of psychological well-being.

## Introduction

Proximal spinal muscular atrophy (SMA) is an autosomal recessive motor neuron disorder caused by the homozygous loss of function of the survival motor neuron (SMN) 1 gene [1]. Prevalence of SMA is estimated to be 1–2 per 100 000 persons, whereas the incidence lies around 8–10 per 100 000 live births [2]. SMA is characterized by progressive muscle weakness that predominates in axial, respiratory and proximal muscle groups. Four different types of SMA are distinguished, depending on age of diagnosis and achievement of motor milestones (Box 1). Natural history studies have suggested that the arrest in motor development is

followed by functional decline over longer periods of time [3]. Life expectancy is reduced for early-onset SMA, but may be unaffected for patients diagnosed after the age of 18 months [4]. The striking variation in SMA severity is primarily caused by differences in gene copy number of the human backup SMN 2 gene, that allows production of residual amounts of the crucial SMN protein. Recently, significant advancements have been made in medical interventions for patients with SMA. Treatment strategies that increase SMN protein levels have been successful in children aged 12 years and younger [5–8]. Both the Food and Drug Administration and European Medicines Agency have approved the use of antisense oligonucleotide Spinraza and this treatment

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 Supplemental data for this article can be accessed [here](#).

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has become available in the USA, large parts of Europe and an increasing number of other countries [9]. Nonetheless, the mainstay of clinical management focuses on prevention and treatment of complications regarding respiratory, nutritional, and orthopedic functioning [10,11].

**Box 1** . Discriminating criteria of SMA types I–IV.

SMA type	Age of onset	Maximal motor milestone	Motor ability
I	<6 months	None	Unable to sit or roll
II	6–18 months	Sitting	Unable to walk
III	>18 months	Walking	May lose ability to walk
IV	>30 years	Normal motor development	Mild motor impairment

To date, limited quantitative research has focused on SMA patients' subjective well-being. A recent study among Dutch patients with SMA showed that, compared with a healthy reference population, individuals with SMA perceive their Health Related Quality of Life (HRQoL) as very similar to reference values of the general population, except for the domain of physical functioning [12]. By contrast, a large American study suggested that perceived HRQoL of respondents with SMA was lower than for healthy controls on all domains [13]. However, differences in study sample and research methodology make these studies difficult to compare. Due to the limited body of research, little is known about determinants of well-being in patients with SMA. For instance, studies that investigated the impact of illness severity on HRQoL in patients with SMA have reported contradictory findings [12–15].

In order to support patients in living a fulfilling life in spite of their physical disadvantages, theory-driven research is vital to understand factors that contribute to or threaten patients' well-being. Post and colleagues have proposed a framework of quality of life (QoL) that extends the well-known International Classification of Functioning, Disability and Health (ICF model) with the concept of general well-being [16–18]. Central tenet of this framework is that consequences of a disease or condition can be experienced on the level of body functions, activities and (societal) participation. Supported by empirical data [16,18], this framework proposes that patients' well-being is linked most directly to the level of participation, which in turn is affected by the impact of the illness on a person's bodily functions and activities.

Alternatively, the self-determination theory [19] provides a more universal theoretical framework about human well-being, acknowledging the notion that well-being encompasses, but is not identical to, health status [20,21]. According to the self-determination theory, three basic psychological needs underlie human motivation: the need for autonomy, relatedness, and competence. Autonomy refers to the sense of being able to act from personal interests and values. Relatedness concerns the feeling of being connected with, respected and valued by others. Finally, competence is the sense of control over one's actions and the environment. Satisfaction of these needs is thought to contribute to the experience of well-being [19], and studies have demonstrated that variations in the satisfaction of these three needs correspond with fluctuations in subjective well-being [22,23].

No studies have previously investigated the influence of psychological need satisfaction on well-being in patients with neuromuscular disorders. Therefore, the aim of this study is to examine psychological well-being among patients with SMA, and to investigate the relationship of well-being with participation and psychological need satisfaction. Based on the superordinate QoL

model of Post et al. [16] and the self-determination theory [19], we hypothesize that higher levels of participation and psychological need satisfaction are associated with better psychological well-being. Additionally, difficulties in participation because of SMA are likely to have a negative impact on the experience of autonomy, relatedness, and competence, which in turn may reduce patients' subjective well-being. Therefore, we expect that need satisfaction will add to the explanation of well-being, after taking into account the individual's level of participation.

## Methods

### Subjects and procedure

This study was approved by our hospital's Medical Ethics Committee. Eligible patients were adults (18 years and older) with a genetically confirmed diagnosis of SMA. We identified a total of 138 adults in the Dutch national SMA registry who met the inclusion criteria. Patients in this registry previously had participated in a national cross-sectional cohort study that focused on motor function and muscle strength [3]. In this study, patients had provided informed consent to use their data for future research. We informed eligible patients by e-mail about the study. One week after receiving the study information, patients were contacted and asked whether they would like to participate. Patients who gave informed consent were directed to an online questionnaire. A paper and pencil version of the questionnaire was also available for those who preferred this option. A reminder was sent out to all patients who had started but not completed the questionnaire, one week after they had given informed consent. All data were collected between 1 November 2017 and 1 December 2017. Additional clinical characteristics (SMA-type and motor function (Hammersmith Functional Motor Scale-Expanded)) were obtained from the SMA database [3].

### Questionnaire content

#### Psychological well-being

In order to obtain a comprehensive impression of respondents' psychological well-being, we selected three main outcome variables for this study: global satisfaction with life, self-esteem, and emotional functioning. The Satisfaction with Life Scale (SWLS) is a five-item questionnaire that assesses a person's global satisfaction with life [24]. Items are formulated in the form of statements, e.g. "In most ways my life is close to ideal." Respondents indicate on a seven-point scale the degree to which they agree or disagree with this statement. The items are summed to produce one single score for satisfaction with life (range 5–35), with higher scores indicating a greater satisfaction with life.

Patients' self-esteem was assessed with the Rosenberg Self-Esteem Scale (RSES) [25]. The RSES is a widely applied instrument consisting of 10 statements regarding an individual's self-esteem. All statements are answered on a 0–3 scale with higher scores representing higher global self-esteem. All items are summed to produce one single index of self-esteem, ranging from 0 to 30.

Emotional functioning was assessed with the Positive and Negative Affect Scale (PANAS) [26]. The PANAS consists of two subscales, Positive Affect and Negative Affect, each consisting of 10 items. Respondents indicate on a five-point scale the degree to which they experience positive (e.g. pride, enthusiasm) and negative feelings (e.g. guilt, shame). The two subscales are calculated by summing the respective items. Scores range from 10 to 50 for each subscale.

To provide context for the levels of psychological well-being reported by adults with SMA, we will also report data from other studies investigating these three psychological variables among healthy Dutch adults. Comparison studies were identified that consisted of a large sample size ( $N > 200$ ), and that had included a Dutch speaking population, aged 18 and older [25,27–31].

### Participation

Participation was measured with the 32-item Utrecht Scale for Evaluation of Rehabilitation-Participation (USER-P), designed to assess both objective and subjective parameters of participation [32]. The USER-P includes three subscales: frequency of participation activities, participation restrictions, and satisfaction with participation. Frequency of participation is divided into two parts: frequency of vocational activities and household chores (four items), and frequency of leisure and social activities (seven items). An aggregated frequency score is calculated by summing the frequency of activities of each part and then calculating the mean score of these two parts. Participation restrictions are assessed by asking the respondent to indicate the level of restriction as a result of their illness in 11 different areas of participation, such as work or social activities. Finally, satisfaction with participation is determined by asking the respondent to indicate his or her satisfaction with 10 aspects of life. All scores for the three USER-P scales are transformed into a 0–100 scale, with higher scores indicating better participation (e.g. higher frequency, less restrictions, higher satisfaction).

### Basic psychological needs

Satisfaction of the need for autonomy, relatedness and competence was assessed using the Basic Psychological Needs Satisfaction and Frustration Scale (BPNSFS) [33]. The BPNSFS consists of 24 items. The satisfaction or frustration of the basic needs is assessed with eight items per need domain. Three composite scores for autonomy, competence, and relatedness were calculated by combining the need satisfaction and reversed need frustration items of each separate need into one mean score. Scores for the three needs range from 1 to 5 with higher scores indicating higher satisfaction of basic needs [34].

### Statistical analyses

First, descriptive analyses were carried out to provide an overview of the sociodemographic and clinical characteristics of the study sample. Furthermore, means and standard deviations were calculated to summarize details concerning respondents' participation, needs satisfaction, and psychological well-being. Cronbach's alpha was used to investigate internal consistency of questionnaire subscales. Next, linear bivariate correlation analyses were used to investigate the association between patients' participation, basic needs and psychological well-being. Finally, four hierarchical regression analyses were performed to examine the contribution of participation and basic need satisfaction to each of the outcome variables. In the first step, the domains of the USER-P (frequency, restrictions, and satisfaction) were entered (forced entry) in the regression analysis. In the second step, satisfaction of basic needs was added. For each regression analysis, sociodemographic or clinical covariates were included as control variable whenever these variables demonstrated a significant univariate relation ( $p < 0.05$ ) with the outcome variable. Variables that were considered as possible confounders were: current age, age at diagnosis, sex, educational level, relational status, SMA type, motor function

Table 1. Sociodemographic and medical characteristics of respondents ( $N = 92$ ).

	N (%)
Age, M (SD)	40.3 (14.5)
Age at diagnosis, M (SD)	9.3 (11.6)
Sex (female)	55 (60%)
Country of birth	
The Netherlands	84 (91%)
Other	8 (9%)
Education	
Special education	7 (8%)
Regular primary school	1 (1%)
Regular secondary education	17 (18%)
Regular secondary voc. training	21 (23%)
Regular higher voc. training/university	46 (50%)
Employment	
Working fulltime	15 (16%)
Working part-time	30 (33%)
Disability compensation/sick leave	15 (16%)
No job (yet)	20 (22%)
Lost job/dismissed	1 (1%)
Student	11 (12%)
Annual gross income (Euro)	
Not answered	55 (60%)
<20 000	10 (11%)
20 000–35 000	15 (16%)
35 000–50 000	7 (8%)
50 000–75 000	3 (3%)
>75 000	2 (2%)
Living situation	
Independent without support	45 (49%)
Independent with structural support	28 (30%)
With (foster) parents	18 (20%)
Other	1 (1%)
Siblings	
No, single child in family	8 (9%)
One or more younger brothers/sisters	52 (57%)
One or more older brothers/sisters	50 (54%)
Having sibling(s) with SMA	35 (39%)
Having a partner	45 (50%)
Having child(ren)	18 (20%)
SMA subtype	
I	3 (3%)
II	37 (40%)
IIIa	21 (23%)
IIIb	26 (29%)
IV	4 (4%)
HFMSE (range 0–66), M (SD)	15.8 (20.8)
Wheelchair use	
No	14 (15%)
Manually operated wheelchair	12 (13%)
Electric wheelchair	72 (79%)
Ventilatory support	
No	71 (78%)
Not yet, but will soon	1 (1%)
Night time ventilation	14 (15%)
Tracheostomy	5 (6%)
Previous scoliosis surgery	44 (48%)

HFMSE: Hammersmith Functional Motor Scale-Expanded.

(Hammersmith Functional Motor Scale-Expanded) and use of ventilatory support.

## Results

Ninety-two patients ( $92/138 = 67\%$ ) completed the questionnaire. An overview of demographic and medical variables is presented in Table 1. Current age of the respondents ranged from 18 to 73 ( $m = 40.3$ ) years, whereas the age at diagnosis varied from 0 to 47 ( $m = 9.3$ ) years. Nearly, half of the respondents were living independently without professional support. Half of the patients were engaged in an intimate relationship and 20% had one or more children. Roughly half of the respondents (56%) were diagnosed

with SMA type IIIa or IIIb. Whereas most patients used an electric wheelchair, only a minority (21%) had (nocturnal) ventilatory support.

### Participation in vocational and social activities

Internal consistency and mean scores for the USER-Participation questionnaire are summarized in Table 2. Whereas the restrictions and satisfaction scales demonstrated good internal consistency, Cronbach's alpha values for the frequency scales were suboptimal. Regarding work and other duties, respondents spent most time on paid work. Eighteen percent ( $n = 16$ ) of our sample worked more than 36 h a week. Sixty percent ( $n = 54$ ) indicated to spend between 1 and 8 h a week on household chores. With regard to social and leisure activities, online or telephone contact with other people was the most frequently performed activity. More than 80% ( $n = 75$ ) of the respondents indicated to engage in telephone/online contact more than 18 times a month. Other frequently performed activities were (in descending order of frequency) leisure activities at home (such as reading, gaming), visiting friends and family, and receiving visits from family and friends.

Participants reported to experience most restrictions in doing household chores (67%), followed by outdoor physical activity or exercise (57%), and going out (e.g. visiting a theater, dining out) (43%). Respondents felt unable to perform these activities, or only when assisted by other people. In spite of these restrictions, most participants were very satisfied with their level of participation. Highest satisfaction was experienced with regard to the social relationships with partner, family and friends. Over 85% of respondents were (very) satisfied with their participation within these domains. Additionally, respondents gave high satisfaction scores for work or study, and leisure activities at home with more than 80% indicating to be (very) satisfied.

Table 2. Participation and Psychological Needs Satisfaction of adults with SMA ( $N = 88-90$ ).

	Cronbach's $\alpha$	$M$ (SD)
Participation (USER-P) (range 0-100)		
Frequency (total score)		35.7 (8.9)
Work, household	-0.49	21.5 (11.4)
Leisure, social	0.52	49.8 (11.9)
Restrictions	0.89	68.9 (20.4)
Satisfaction with participation	0.82	76.6 (18.0)
Psychological Needs Satisfaction (BPNSFS) (range 1-5)		
Autonomy	0.82	4.0 (0.7)
Relatedness	0.76	4.6 (0.5)
Competence	0.76	4.1 (0.6)

USER-P: Utrecht Scale for Evaluation of Rehabilitation-Participation; BPNSFS: Basic Psychological Needs Satisfaction and Frustration Scale.

### Basic Psychological Needs Satisfaction

Respondents scored high on the degree to which they saw their basic psychological needs fulfilled (Table 2). Mean scores for autonomy, relatedness, and competence varied between 4.0 and 4.6 on a five-point scale. Only a minority of the participants reported that their needs for autonomy (17%), relatedness (1%), and competence (7%) were not met, as indicated by mean satisfaction scores below the midpoint of the scale.

### Psychological well-being in comparison with reference samples

Results for psychological well-being were compared with results from other studies consisting of healthy Dutch adults (a description of the reference samples is provided in Supplementary Table 1). Despite the physical limitations of their disease, respondents were quite satisfied with their life in general (Table 3). Mean scores were very similar to those obtained in two other Dutch studies consisting of healthy adults [27,31]. Nonetheless, nearly 17% ( $n = 15$ ) of our sample were dissatisfied with their lives. In addition, respondents in our study had high self-esteem scores. Interestingly, our sample of adults with SMA appeared to score even higher than two other samples of Dutch adults [25,30]. Finally, with regard to emotional functioning, respondents in our sample reported relatively high levels of positive affect, whereas scores for negative affect were comparable with those found in adult reference samples [28,29].

### Participation and need satisfaction in relation to psychological well-being

Frequency, restrictions, and satisfaction scales of the USER-P questionnaire showed weak to moderate positive intercorrelations ( $r = 0.14-0.35$ ) (Table 4). The three scales of the BPNSFS and the indices of psychological well-being demonstrated moderate to strong associations.

Whereas the experienced restrictions in participation were largely unrelated to respondents' psychological well-being, the level of satisfaction with participation showed significant relations with all measures of psychological well-being (Table 4). In addition, satisfaction of the needs for autonomy, relatedness and competence, demonstrated significant correlations with nearly all indices of well-being.

### Hierarchical regression of psychological well-being on participation and need satisfaction

Four regression analyses were performed to examine the contribution of participation and satisfaction of psychological needs to the explanation of variance in satisfaction with life, self-esteem, and emotional functioning (positive and negative affect). Exploratory analyses showed that sociodemographic (e.g. age, education) and clinical variables (time since diagnosis, SMA

Table 3. Psychological well-being of adults with SMA ( $N = 87$ ).

	SMA sample		Comparison studies	
	$\alpha$	$M$ (SD)	$M$ (SD)	$M$ (SD)
Satisfaction with life (SWLS) (range 5-35)	0.87	26.2 (6.5)	26.2 (5.7) [27]	25.7 (5.6) [31]
Self-esteem (RSES) (range 0-30)	0.75	25.0 (3.9)	21.6 (4.5) [30]	20.9 (4.4) [25]
Affective state (PANAS) (range 10-50)				
Positive affect	0.85	38.8 (5.4)	32.1 (6.3) [29]	34.1 (5.1) [28]
Negative affect	0.84	17.5 (5.6)	17.0 (5.7)-19.2 (7.0) [29]	22.5 (6.5) [28]

SWLS: Satisfaction with Life Scale; RSES: Rosenberg Self-Esteem Scale; PANAS: Positive and Negative Affect Scale.

Table 4. Pearson's correlation between indices of participation, basic need satisfaction and psychological well-being ( $N = 87$ ).

	USER-P restriction	USER-P satisfaction	BPNSFS autonomy	BPNSFS relatedness	BPNSFS competence	Satisfaction with life	Self-esteem	Positive affect	Negative affect
USER-P Frequency	0.29**	0.35***	0.22*	0.15	0.31**	0.22*	0.24*	0.36***	-0.19
USER-P Restrictions	1	0.14	0.23*	0.13	0.13	0.04	0.01	-0.04	-0.28**
USER-P Satisfaction		1	0.49***	0.26*	0.33**	0.43***	0.34***	0.33**	-0.26*
BPNSFS Autonomy			1	0.47***	0.65***	0.51***	0.47***	0.50***	-0.44***
BPNSFS Relatedness				1	0.49***	0.17	0.35***	0.34***	-0.47***
BPNSFS Competence					1	0.41***	0.66***	0.69***	-0.44***
SWLS Satisfaction with life						1	0.43***	0.44***	-0.36***
RSES Self-esteem							1	0.62***	-0.57***
PANAS Positive affect								1	-0.32**
PANAS Negative affect									1

USER-P: Utrecht Scale for Evaluation of Rehabilitation-Participation; BPNSFS: Basic Psychological Needs Satisfaction and Frustration Scale; SWLS: Satisfaction with Life Scale; RSES: Rosenberg Self-Esteem Scale; PANAS: Positive and Negative Affect Scale.

\* $p \leq 0.05$ .

\*\* $p \leq 0.01$ .

\*\*\* $p \leq 0.001$ .

Table 5. Multivariate analyses investigating the contribution of participation and need satisfaction to psychological well-being ( $N = 85$ ).

	Satisfaction with life		Self-esteem		Positive affect		Negative affect		
	Model 1 Std. B	Model 2 Std. B	Model 1 Std. B	Model 2 Std. B	Model 1 Std. B	Model 2 Std. B	Model "0" Std. B	Model 1 Std. B	Model 2 Std. B
Step "0"									
Sex (M)	n.a.		n.a.		n.a.		-0.23*	-0.20*	-0.21*
$\Delta R^2$							0.05		
$\Delta F$							4.72*		
Step 1									
USER-P Freq.	0.15	0.10	0.19	0.02	0.34**	0.17*		-0.11	-0.04
USER-P Restr.	-0.03	-0.10	-0.08	-0.13	-0.18	-0.24**		-0.23*	-0.17
USER-P Sat.	0.38***	0.21	0.29**	0.12	0.25*	0.05	-	-0.21	-0.06
$\Delta R^2$	0.20		0.15		0.21			0.15	
$\Delta F$	6.79***		4.61**		7.16***			4.82**	
Step 2									
Autonomy		0.37**		0.04		0.14			-0.07
Relatedness		-0.14		0.03		0.02			-0.32**
Competence	-	0.15	-	0.60***	-	0.57***	-	-	-0.16
$\Delta R^2$		0.13		0.33		0.36			0.18
$\Delta F$		5.11**		16.23***		21.23***			7.67***
Model									
$R^2$	0.20	0.33	0.15	0.47	0.21	0.57	0.05	0.20	0.38
Adjusted $R^2$	0.17	0.28	0.11	0.43	0.18	0.53	0.04	0.16	0.33
Overall $F$	6.79***	6.47***	4.61**	11.72***	7.16**	16.87***	4.72*	4.96***	6.83***

USER-P Freq: USER-P frequency scale; USER-P Restr: USER-P restrictions scale; USER-P Sat: USER-P satisfaction with participation scale.

\* $p \leq 0.05$ .

\*\* $p \leq 0.01$ .

\*\*\* $p \leq 0.001$ .

subtype, motor function) were generally unrelated to respondents' psychological well-being (data not shown). The only exception was a significant relation found between female sex and negative affect ( $t(85)=2.32$ ,  $p=0.02$ ). Sex was therefore included as a covariate only in the regression analysis for negative affect.

With regard to satisfaction with life, we found the USER-Participation domain of satisfaction with participation to show the strongest association with SWL scores in the first block (Table 5). Next, satisfaction regarding the need for autonomy, relatedness and competence were added to the equation. This step added 13% to explanation of variance in SWL ( $p < 0.01$ ) with satisfaction with autonomy remaining as the only significant independent variable in the final model (Std  $\beta = 0.37$ ,  $p = 0.009$ ).

Self-esteem of adults with SMA was also significantly related to respondents' satisfaction with participation (Table 5). However, when respondents' satisfaction with basic psychological needs were added in the second step of the analysis, the contribution of

USER-Participation variables was no longer significant. In the final model, self-esteem was most strongly related to respondents' sense of competence (Std  $\beta = 0.60$ ,  $p < 0.001$ ).

Regarding emotional functioning (PANAS), results showed that the Frequency and Satisfaction scales of the USER-Participation questionnaire both were significantly related to positive affect (Table 5). Adding the three basic need domains to the regression increased the amount of variance explained significantly ( $\Delta R^2=0.36$ ,  $p < 0.001$ ). In the final model, the sense of competence, participation frequency and restrictions in participation remained as significant independent variables, whereas satisfaction with participation was no longer significant.

The last regression analysis focused on negative affect (Table 5). As mentioned above, sex was entered as control variable, before the USER-Participation scales were added (Step "0"). In the first step, the USER-P Restrictions scale showed a significant relation to negative affect (less restrictions being related to lower

negative affect). However, this variable was no longer significant when the basic needs were added to the regression in the second step. In the final model, female sex and lower sense of relatedness remained as significant predictors of negative affect.

## Discussion

The present paper adds to the limited body of knowledge concerning psychological well-being of adults with SMA [9]. Our results indicate that adults with SMA report a level of psychological well-being that is very similar to well-being reported by reference samples consisting of healthy adults. Importantly, this study showed that approximately 30–50% of variance in psychological well-being is explained by patients' societal participation and satisfaction of basic psychological needs (autonomy, competence, and relatedness).

At first sight, the high levels of well-being reported in this study seem at odds with outcome of a large multicenter study conducted in the US among 125 juvenile patients with SMA (<18 years) who reported relatively poor QoL, compared with 1100 sex and aged matched controls [13]. We think that the methodological approach of our study, in addition to the sociodemographic differences (i.e. children versus adults), may explain the different outcomes. The conceptual approach of our study was intentionally different from the ones used in previous studies that have used the concept of HRQoL as an operationalization of SMA patients' well-being [12,13,35]. HRQoL instruments, such as the PedsQL and the SF-36, that were used in those studies predominantly assess negative aspects, for instance physical symptoms and activity limitations, as an indication of the individual's QoL. Furthermore, when focusing on HRQoL, the effect of non-medical variables on a person's sense of well-being is usually underestimated [36]. This predominantly deficit-centered medical view on well-being contrasts to more recent views on health that consider health as the ability to adapt and self-manage "in the face of social, emotional and physical challenges" [37,38]. Therefore, we deliberately chose to use global satisfaction with life as an indication of a person's QoL, as proposed by Moons et al. [39]. Similarly, the other outcome variables in this study, i.e. self-esteem and emotional functioning, were chosen as they make no explicit reference to health, illness, or disability, allowing for a relatively unbiased comparison with reference groups. Our results clearly illustrate that there is a negligible relation between objective health indicators, such as motor function, and psychological well-being.

Post et al. have suggested that well-being and mental health are closely related to a person's level of participation. The USER-P was used to assess participation frequency, restrictions and satisfaction. The low internal consistency of the participation frequency scale, especially the part on vocational activities and household chores, demands further consideration. An explanation for this finding is that the activities in this subscale are mutually exclusive. Even without the limitations of a particular illness, people only have a fixed amount of time. Spending more time on one activity (e.g. full-time employment), leaves less time for another activity (e.g. studying). Negative correlations between the items and poor internal consistency of the scale can therefore be expected [32]. Nonetheless, results from the present investigation are in line with previous patient studies by showing an association between indices of subjective well-being and different aspects of participation, particularly with respect to the individual's satisfaction with his or her participation [18,40–42]. Including this subjective, evaluative dimension, as an addition to the

"objective" and observable aspects of physical impairment, and limitations in activities and participation, is likely to improve our understanding of an individual's sense of well-being [17,43].

In addition to the satisfaction with participation, the present study also assessed satisfaction with respect to the need for autonomy, competence, and relatedness as predictors of well-being, as outlined by the self-determination theory [19]. Our findings indicate that satisfaction of these three needs is strongly related to patients' well-being, even when the contribution of patients' participation has been partialled out. Adding the basic need variables in the regression increased the Adjusted R-square values in all four analyses, and improved the model fit for three of the four outcomes. To the best of our knowledge, this study is the first to include satisfaction of basic psychological needs to explain variations in well-being among patients with neuromuscular disorders. Although the self-determination theory has not been widely applied within medical research, several previous quantitative studies have illustrated the hypothesized relation of psychological needs with life satisfaction and emotional health among various patient groups [44–46]. For people with SMA in particular, strong support for the significance of these psychological needs has been provided by qualitative studies, in which patients make explicit reference to the influence of satisfaction or frustration of their need for autonomy (e.g. limited independence, living an autonomous life), relatedness (e.g. social development, stigma) and competence (e.g. self-control, self-confidence, being a productive member of society) on their psychological well-being [47–49].

## Limitations

Several limitations must be acknowledged. First, although the response rate in this investigation was satisfactory, we cannot rule out the possibility that patients who had a less optimistic view on life were more reluctant to participate in this study, leading to an overestimation of psychological health of adults with SMA. Overestimation also may have been caused by the fact that the respondents in our sample constitute a highly educated group. Educational level has generally been found to be positively related to well-being [50]. In our study, 50% of the sample had completed higher education (higher vocational training or university). By comparison, in 2017, only 30% of the Dutch population aged 15–75 had completed higher education (Statistics Netherlands). This may have introduced bias in our comparison with the health reference groups. In fact, our sample may have been different from the reference samples with regard to other sociodemographic characteristics, which disallows us to draw definite conclusions about the well-being of adults with SMA and health adults. Second, our study has included only adults with SMA, an understudied subgroup compared with children with SMA [9]. It is likely that reports of children about their well-being and the association with participation and basic need satisfaction are different from adults, limiting the generalizability of our results. Finally, our study has used a cross-sectional methodology, disallowing us to draw conclusions about the direction of causality. Arguably, patients with higher sense of well-being will be more motivated to be active and to become take part in social activities.

## Implications for research

This study suggests that well-being of adults with SMA is very comparable to values found in the normal population. To further

our understanding, a larger study is needed that compares well-being of a healthy norm group with that of several patient groups with various neuromuscular conditions (e.g. skeletal muscular disorders, motor neuron disorders, neuromuscular junction disorders).

Follow-up studies with a longitudinal design are warranted to investigate the temporal stability of SMA patients' well-being and to explore whether fluctuations are related to variations in participation and satisfaction of psychological needs. Diary studies may be useful in this regard [22,23]. In addition, qualitative studies are required to provide in-depth information about how and when basic psychological needs of people with SMA are threatened or preserved. This information is valuable for the development of psychological interventions to support patients in achieving their desired levels of autonomy, competence and relatedness.

Our study has underscored the relevance of the satisfaction of psychological needs for adult patients with SMA. As the importance of need and values may change in the development from childhood to adulthood, a separate study among children with SMA is called for, to determine the relative importance of basic needs for their psychological well-being. Finally, as noted previously, the progressive nature of SMA has long been considered inevitable. In light of the recent developments regarding pharmaceutical treatment options for SMA, a replication of this investigation may demonstrate how these advancements provide hope and increase confidence for the future among this population, which in turn may heighten their psychological well-being.

### **Implications for treatment**

Our results point to the importance of supporting patients in meeting their basic psychological needs, both within and outside of the medical domain. Importantly, paying attention to basic psychological needs should be a continuous concern throughout the trajectory of care [51]. Several studies have compared the impact of "autonomy supportive" healthcare climates on patients' need satisfaction [52]. Autonomy supportive healthcare professionals take on a patient-centered approach and promote partnership and collaboration with their patients by active listening and seeking patient input, exploring patients' values, cognitions and emotions, providing information on disease, treatment and self-management in an understandable manner, and respecting patients' preference and choice, even if this does not correspond to professional recommendation [53–55].

With regard to the need for competence, techniques and interventions originating from Bandura's work on personal efficacy [56] can be valuable. First, the sense of competence is thought to be enhanced when individuals experience success in achieving personally relevant goals. To increase chances of success, therapists may aid patients by setting realistic goals, providing clear instructions and encouragement, and teaching problem solving skills to overcome potential barriers. Competence may also be enhanced by "vicarious learning" [56]. Patients' confidence and persistence is expected to increase by observing and learning from peers, either directly (e.g. (online) peer contact) or indirectly (e.g. vignettes, video testimonials).

Relatedness support requires healthcare professionals to invest in developing a solid working alliance with their patients, characterized by unconditional positive regard and empathy with patient and family values, needs and concerns [57]. Professional support and attitudes of healthcare professionals have been shown to be one of the most relevant environmental factors

contributing to QoL among people with slowly progressive neuromuscular diseases, including SMA [58].

### **Conclusions**

The present study illustrated the importance of satisfaction of basic psychological needs as described by the self-determination theory, in understanding subjective well-being in adults with SMA. We suggest that, in addition to supporting patients in preserving their participation, clinicians should be attentive throughout the illness trajectory to optimize autonomy, competence and relatedness, in order to improve patients' psychological well-being.

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