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Quality of life in patients with slowly progressive neuromuscular disorders dependent on mechanical ventilation

Mathieu Delorme ^{1,2}, Christian Reveillere,³ Christian Devaux,² Sandrine Segovia-Kueny,² Frédéric Lofaso,^{1,4} Ghilas Boussaid^{1,2}

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¹UVSQ, ERPHAN, Université Paris-Saclay, Versailles, France

²Direction des Actions Médicales, AFM-Téléthon, Evry, France

³Département de Psychologie, EE QualiPsy, Université de Tours, Tours, France

⁴Unité des pathologies du sommeil, Service de physiologie - explorations fonctionnelles, Hôpital Raymond Poincaré, AP-HP, Garches, France

Correspondence to

Mr Mathieu Delorme, UVSQ, ERPHAN, Université Paris-Saclay, 78000, Versailles, France; mathieu.delorme.pt@gmail.com

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ABSTRACT

This cross-sectional study evaluated health-related quality of life (HRQoL) in patients with slowly progressive neuromuscular disorders dependent on mechanical ventilation (MV; ≥ 16 hours/day). 119 participants, with 9 years (25th–75th percentiles: 4–15 years) of MV dependence, were included. MV was applied via a tracheostomy in 80 participants (67.2%) and non-invasive interfaces in 39 participants (32.8%), including 28 participants (71.8%) with daytime mouthpiece ventilation. HRQoL was rated good or excellent by 81 participants (68.1%), independently from age, diagnosis or respiratory autonomy. On multivariate analysis, time since MV initiation, independence from family member(s), residence in a rural area, ability to go outdoors with MV and tracheostomy were associated with better HRQoL.

INTRODUCTION

Respiratory insufficiency is among the leading causes of death in patients with slowly progressive neuromuscular disorders (NMD).¹ The need for mechanical ventilation (MV) is, therefore, considered at early stages of disease evolution.^{2,3}

However, despite optimal ventilatory management, disease progression may lead to an increase in MV needs. The longer life expectancy is at the cost of MV dependence, which may impact health-related quality of life (HRQoL).

The primary objective of this study was to assess HRQoL in patients with slowly progressive NMD dependent on MV, either non-invasive (NIV) or invasive mechanical ventilation (IMV; via a tracheostomy). Our secondary objective was to identify factors associated with HRQoL in this population.

METHODS

Additional information about the methods is available in the online supplemental file 1.

Participants

Participants were identified via the AFM-Téléthon organisation. Adults who had slowly progressive NMD, were wheelchair bound or bedridden and were dependent on NIV or IMV (≥ 16 hours/day for at least the past year) were eligible. The following diseases were defined a priori as not eligible: amyotrophic lateral sclerosis, myasthenia, polyradiculoneuritis and metabolic neuropathies.

Data collection

Each participant completed a questionnaire collecting the variables listed in the online supplemental table 1, and two validated and standardised HRQoL questionnaires: the Severe Respiratory Insufficiency Questionnaire (SRI), developed for patients with chronic respiratory failure,⁴ and the Quality of Life in genetic Neuromuscular Disease Questionnaire (QoL-gNMD), developed for patients with slowly progressive NMD.⁵ Each HRQoL score presented hereafter ranges from 0 to 100, with higher scores indicating better HRQoL.

Statistical analysis

We built multivariate linear regression models to identify factors associated with the SRI summary scale (SRI-SS) and with scores on each of the three QoL-gNMD domains (impact of physical symptoms (QoL-gNMD-PS), self-perception (QoL-gNMD-SP) and activities and social participation (QoL-gNMD-ASP)). Each multivariate model was adjusted for age, diagnosis (dystrophinopathy vs other diseases) and factors associated with p values of <0.20 by univariate analysis and identified by stepwise selection.

We compared groups with IMV versus NIV using Student's t-test, the Mann-Whitney U test, the χ^2 test and Fisher's exact test, where appropriate. All tests were two-tailed and p values of <0.05 were considered significant.

RESULTS

Between December 2020 and December 2021, 119 participants distributed throughout France were enrolled. Online supplemental figure 1 is the flow chart. Table 1 reports the main features of the participants. Of the 39 patients (32.8%) on NIV, the most common daytime interface was mouthpiece (28 participants (71.8%)). Additional information about the results is provided in online supplemental file 1.

Health-related quality of life

As assessed based on the two general QoL-gNMD items, health status and HRQoL were 'good or excellent' for 75 participants (63.0%) and 81 participants (68.1%), respectively. Only 4 participants (3.4%) described their HRQoL as poor (online supplemental figure 2). The mean SRI-SS score was 56.1 ± 14.6 (range: 15.9–86.5). Consistently with the inclusion criteria, physical functioning was the most severely impaired SRI domain (18.9 ± 14.8 ;



Table 1 Main features of the study participants and in the groups with IMV (via a tracheostomy) versus NIV

	All patients (n=119)	NIV (n=39)	IMV (n=80)	P value
Features				
Age, years	37.0 (29.0–46.5)	35.0 (27.0–47.5)	37.5 (31.0–46.0)	0.439
Female sex, n (%)	35 (29.4)	9 (23.1)	26 (32.5)	0.290
BMI, kg/m ²	21.2 (16.5–24.7)	19.0 (15.4–24.2)	21.4 (18.0–25.3)	0.129
Diagnosis, n (%)				
Dystrophinopathy				
Duchenne muscular dystrophy	59 (49.6)	23 (59.0)	36 (45.0)	
Becker muscular dystrophy	2 (1.7)	–	2 (2.5)	
Limb girdle muscular dystrophy	15 (12.6)	11 (28.2)	4 (5.0)	
Congenital muscular dystrophy	9 (7.6)	2 (5.1)	7 (8.8)	
Facioscapulohumeral dystrophy	4 (3.4)	–	4 (5.0)	
Congenital myopathy	4 (3.4)	1 (2.6)	3 (3.8)	
Glycogen storage disease type 2	2 (1.7)	1 (2.6)	1 (1.3)	
Steinert myotonic dystrophy	1 (0.8)	–	1 (1.3)	
Spinal muscular atrophy				
SMA 1b	9 (7.6)	1 (2.6)	8 (10.0)	
SMA 2	7 (5.9)	–	7 (8.8)	
SMA 3	1 (0.8)	–	1 (1.3)	
Other neuromuscular disorder				
Myofibrillar myopathy	1 (0.8)	–	1 (1.3)	
Charcot-Marie-Tooth disease	1 (0.8)	–	1 (1.3)	
Inclusion body myositis	1 (0.8)	–	1 (1.3)	
Undetermined NMD	3 (2.5)	–	3 (3.8)	
Respiratory care				
Time since MV initiation, years	17.0 (10.0–21.0)	11.0 (8.3–16.8)	19.0 (11.5–23.0)	0.001
Time since onset of MV dependence*, years	9.0 (4.0–15.0)	6.0 (3.3–9.8)	11.0 (5.3–17.0)	<0.001
Respiratory autonomy, min	10.0 (2.0–90.0)	18.8 (3.3–113.0)	5.0 (1.5–60.0)	0.077
Requirement for airway clearance techniques, n (%)	80 (67.2)	20 (51.3)	60 (75.0)	0.010
Airway clearance sessions, n/week	3.0 (0.0–7.0)	0.0 (0.0–2.5)	7.0 (0.0–7.0)	<0.001
Functional autonomy				
Personal assistant every day, n (%)	83 (69.7)	24 (61.5)	59 (73.8)	0.173
In bed most of the time, n (%)	11 (9.2)	2 (5.1)	9 (11.3)	0.337
Can go outdoors with MV, n (%)				0.143
Yes, alone	20 (16.8)	9 (23.1)	11 (13.8)	
Yes, with a caregiver†	96 (80.7)	28 (71.8)	68 (85.0)	
No	3 (2.5)	2 (5.1)	1 (1.3)	
Assistance required during meals, n (%)	108 (90.8)	36 (92.3)	72 (90.0)	1.0
Duration of main meal, min	40.0 (30.0–60.0)	30.0 (30.0–52.5)	40.0 (30.0–60.0)	0.297
Place of residence, n (%)				
Urban area	66 (55.5)	28 (71.8)	38 (47.5)	0.005
Medico-social institution	18 (15.1)	3 (7.7)	15 (18.8)	0.114
Living arrangements, n (%)				
Lives alone or in shared accommodations‡	42 (35.3)	8 (20.5)	34 (42.5)	0.018
Lives with a partner	19 (16.0)	7 (17.9)	12 (15.0)	0.680
Lives with other family members	57 (47.9)	24 (61.5)	33 (41.3)	0.038
Other variables				
Interactions with healthcare workers\$, days/week	3.0 [0.0–7.0)	2.0 [2.0–4.0)	7.0 [3.0–7.0)	<0.001
Psychotropic medication, n (%)	24 (20.3)	3 (7.7)	21 (26.3)	0.017
Inclusion during a COVID-19 lockdown, n (%)	49 (41.2)	15 (38.5)	34 (42.5)	0.674
Level of education, n (%)				
Elementary school	10 (8.4)	1 (2.6)	9 (11.3)	0.094

Continued

Table 1 Continued

	All patients (n=119)	NIV (n=39)	IMV (n=80)	P value
Middle school	24 (20.2)	9 (23.1)	15 (18.8)	
High school	38 (31.9)	9 (23.1)	29 (36.3)	
University	46 (38.7)	20 (51.3)	26 (32.5)	
Professional activity, n (%)	9 (7.6)	4 (10.3)	5 (6.3)	0.438

Data are presented as n (%) or median (25th–75th percentiles).
*MV dependence was defined as daily ventilation ≥ 16 hours/day for at least the past year.
†Caregiver was defined as a family member, personal assistant or healthcare worker.
‡The patients in sharing arrangements did not require help from those living in the same home.
§Healthcare workers were defined as nurses or physiotherapists.
BMI, body mass index; IMV, invasive mechanical ventilation; MV, mechanical ventilation (non-invasive or invasive); NIV, non-invasive ventilation; NMD, neuromuscular disorder; SMA, spinal muscular atrophy.

range: 0–54.2). Of the QoL-gNMD domains, activities and social participation had the lowest mean score (43.1 ± 8.6 ; range: 24.2–62.7).

Figure 1 and online supplemental table 2 report the scores on the SRI subscales and QoL-gNMD domains, comparing groups with IMV versus NIV. The SRI-SS score was significantly better in the IMV group than in the NIV group (57.9 ± 13.6 and 52.2 ± 16.0 , respectively; $p=0.046$). This difference was mainly due to the lesser intensity of respiratory complaints (online supplemental figure 3) and anxiety in the IMV group. Scores for all three QoL-gNMD domains were better with IMV than with NIV, but this difference was not significant and should be interpreted with caution.

Factors associated with HRQoL

Online supplemental tables 1 and 3 report the results of the univariate analyses. HRQoL was not associated with age, overweight, diagnosis of dystrophinopathy versus other NMD, inclusion during a COVID-19 national lockdown, daily presence of a personal assistant, level of education or residence in a medical institution.

Table 2 shows the results of the multivariate analyses. A better SRI-SS score was associated with a longer time since MV initiation at study inclusion, residence in a rural area and living independently from a partner or other family member(s). A longer time since MV initiation, tracheostomy and ability to

go outdoors with MV were associated with better scores for the QoL-gNMD-PS, QoL-gNMD-SP and QoL-gNMD-ASP domains, respectively.

DISCUSSION

From these results, it should be emphasised that HRQoL was perceived as good or excellent by over two-thirds of MV-dependent patients with slowly progressive NMD. Although there is strong agreement that HRQoL is a major goal in patients treated with long-term MV,⁶ assessment of HRQoL in daily clinical practice can be far from optimal and should deserve greater attention.

Our results also highlight that, even though often viewed as a negative step, tracheostomy was not associated with poorer HRQoL. A 1993 report indicated a preference for NIV over tracheostomy for safety, comfort, speech, swallowing and appearance.⁷ Since then, however, long-term IMV techniques have improved considerably, allowing speech and eating during MV (as shown in online supplemental figure 3).^{8,9} Importantly, in our study, more than 80% of the participants ventilated through IMV did not necessitate to move into a medical institution, and two-fifths of them lived alone or shared accommodations compared with only one-fifth of patients using NIV. Tracheostomy was not a barrier to independence.

The limitations of our study include the occurrence during the

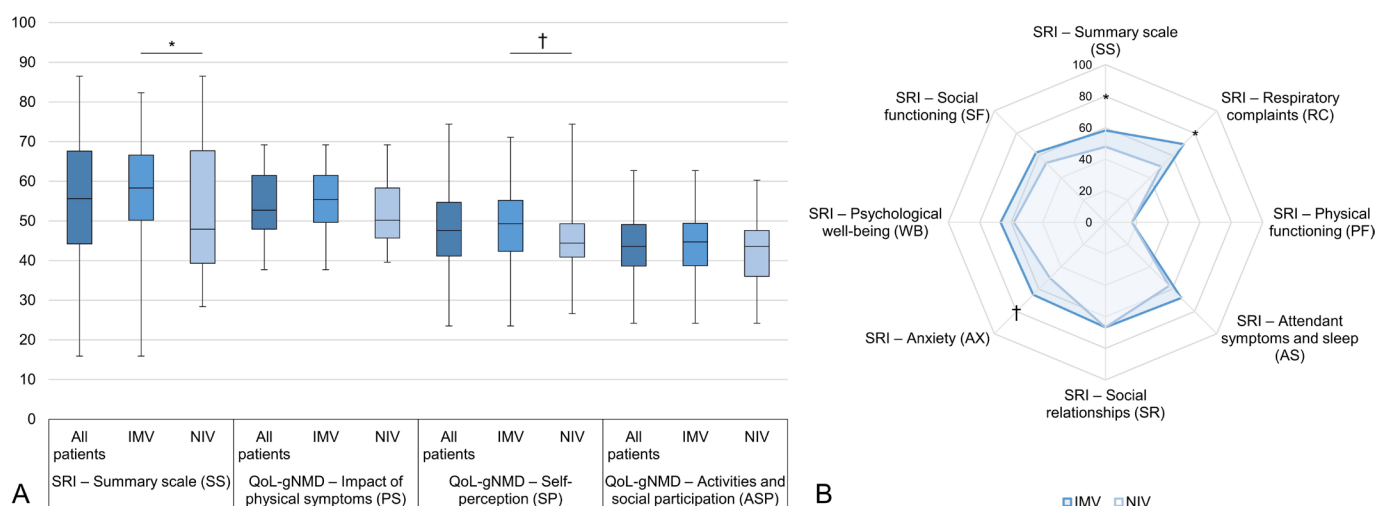


Figure 1 Scores on the SRI and QoL-gNMD. The scores for each domain can range from 0 to 100, with higher scores indicating better quality of life. The box plots represent quality-of-life scores for all participants and comparison between groups with IMV versus NIV (A). The radar plots represent the different domains of the SRI in the groups with IMV versus NIV (B). Comparison between groups with IMV versus NIV: * $p<0.05$; † $p<0.1$. See online supplemental table 2 for more information. IMV, invasive mechanical ventilation; NIV, non-invasive ventilation; QoL-gNMD, Quality of Life in genetic Neuromuscular Disease Questionnaire; SRI, Severe Respiratory Insufficiency Questionnaire

Table 2 Multivariate analyses

	SRI-SS			QoL-gNMD-PS			QoL-gNMD-SP			QoL-gNMD-ASP		
	β (95% CI)	P value	R ²	β (95% CI)	P value	R ²	β (95% CI)	P value	R ²	β (95% CI)	P value	R ²
NIV	-	-	0.21	-	-	0.05	-4.92 (-9.44 to -1.11)	0.017	0.06	-	-	0.10
Time since MV initiation, years	0.30 (0.03 to 0.57)	0.027		0.20 (0.01 to 0.33)	0.030		-	-		-	-	
Residence in an urban area	-5.57 (-10.46 to -0.67)	0.026		-	-		-	-		-	-	
Lives with a partner or other family member(s)	-10.32 (-15.36 to -5.28)	<0.0001		-	-		-	-		-	-	
Can go outdoors with MV*											0.003	
Yes, with a caregiver†	-	-		-	-		-	-		-3.88 (-8.01 to 0.00)	0.057	
No	-	-		-	-		-	-		-17.18 (-27.26 to -7.09)	0.001	

The data are estimates (β) and 95% CIs.

Positive values indicate that the variable was associated with better quality of life.

*Reference: yes, alone.

†Caregiver was defined as a family member, personal assistant or healthcare worker.

MV, mechanical ventilation (non-invasive or invasive); NIV, non-invasive ventilation; QoL-gNMD, Quality of Life in genetic Neuromuscular Disease Questionnaire; QoL-gNMD-ASP, Quality of Life in genetic Neuromuscular Disease Questionnaire-self-perception; activities and social participation; QoL-gNMD-PS, Quality of Life in genetic Neuromuscular Disease Questionnaire-impact of physical symptoms; QoL-gNMD-SP, Quality of Life in genetic Neuromuscular Disease Questionnaire-self-perception; SRI-SS, Severe Respiratory Insufficiency Summary Scale.

recruitment period of several COVID-19 national lockdowns, which may have decreased the willingness of patients to participate in a study on such a topic at a time when quality of life was constrained for everyone. This may have induced selection bias toward patients with better perceived HRQoL. Also, participants were recruited via a single organisation (AFM-Téléthon). However, this recruitment was carried out through 18 services all over France, which suggests a reasonable representativeness of the whole territory. Lastly, the cross-sectional study design precluded assessments of causality.

In conclusion, MV-dependent patients with slowly progressive NMD reported good HRQoL independently from age, diagnosis and respiratory autonomy. Participants with tracheostomy had less respiratory complaints, and a trend toward better self-perception and lower anxiety than participants with NIV, despite requiring a higher level of respiratory care. Additionally, other factors strongly associated with better HRQoL, such as not requiring help from family for daily needs and being able to go outdoors with MV, deserve consideration.¹⁰

Correction notice This article has been corrected since it was published Online First. The licence has been updated to open access CC BY-NC.

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Contributors All authors contributed substantially to conceive and design the study, revised the manuscript for important intellectual content, approved the final version, agree to be accountable for all aspects of the work and to ensure that questions related to the accuracy or integrity of any part of the work are appropriately investigated and resolved. MD, GB and FL contributed to acquire, analyse and interpret the data and to draft the manuscript.

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Competing interests MD reports grants from Fondation de France, AFM Téléthon and Fondation Garches, during the conduct of the study; personal fees from Air Liquide Medical Systems, Breas Medical AB, ResMed SAS and GSK; and non-financial support from L3 Medical, outside the submitted work. Other authors have nothing to disclose.

Patient consent for publication Not applicable.

Ethics approval This study involves human participants and was approved by Comité de Protection des Personnes Sud-Ouest et Outre Mer III on 29 July 2020 (ID: 2020-A00997-32). Participants gave informed consent to participate in the study before taking part.

Provenance and peer review Not commissioned; externally peer reviewed.

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ORCID iD

Mathieu Delorme <http://orcid.org/0000-0001-8506-2021>

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SUPPLEMENTAL MATERIAL

Quality-of-Life in Patients with Slowly Progressive Neuromuscular Disorders Dependent on Mechanical Ventilation.

Mathieu DELORME, PT, MSc^{1,2}, Christian REVEILLERE, PhD³, Christian DEVAUX,
PT², Sandrine SEGOVIA-KUENY, MD², Frederic LOFASO, MD, PhD^{1,4}, Ghilas
BOUSSAID, PT, PhD^{1,2}.

SUPPLEMENTAL METHODS

The study was approved by the local ethics committee (Comité de Protection des Personnes Sud-Ouest et Outre Mer III; July 29th, 2020; ID: 2020-A00997-32).

Written informed consent was obtained from all participants before study inclusion.

The study was registered on www.clinicaltrials.gov (NCT04652401).

Identification of participants was made via the *AFM-Téléthon*, which is a non-profit organization providing support to over 16 000 patients throughout France, including about 10% treated with home MV.[1]

Participants

Participants were eligible if they had slowly progressive NMD. The following diseases were defined *a priori* as not eligible: amyotrophic lateral sclerosis, myasthenia, polyradiculoneuritis, and metabolic neuropathies. Additionally, we did not include patients who lived outside metropolitan France, were under guardianship, had an unstable health condition (*e.g.*, a fever, an acute infection, or one or more organ failures), or had cognitive impairments precluding questionnaire completion.

Eligible participants were contacted by phone to confirm their eligibility at the time of enrolment and to collect their oral consent before study documents were mailed to them. Participants were included after receipt of their written informed consent.

Data collection

Participants were invited to anonymously complete the printed questionnaires, alone or with assistance, and to return them by mail to the research team.

The SRI investigates seven HRQoL domains: respiratory complaints (SRI-RC), physical functioning (SRI-PF), attendant symptoms and sleep (SRI-AS), social relationships (SRI-SR), anxiety (SRI-AX), psychological well-being (SRI-WB), and social functioning (SRI-SF). Each subscale and the average of the seven subscales (SRI Summary Scale, SRI-SS) can range from 0 (worst possible score) to 100 (best possible score).

The QoL-gNMD has two general items (health and quality-of-life) and 24 items classified into three domains: impact of physical symptoms (QoL-gNMD-PS), self-perception (QoL-gNMD-SP), and activities and social participation (QoL-gNMD-ASP). The score for each of the QoL-gNMD domains (which are not intended to be aggregated) can also range from 0 to 100, with higher scores indicating better HRQoL.

Statistical analyses

Continuous variables are described as mean \pm SD or median [25th–75th percentiles] depending on data distribution, and categorical variables as counts and percentages. For the construction of the multivariate models, all the variables listed in supplemental table 1 were tested by univariate analysis. Each multivariate model was then adjusted for age, diagnosis (dystrophinopathy versus other diseases), and factors associated with P values <0.20 by univariate analysis and identified by stepwise selection. Q–Q plots of residuals, residual-*versus*-fitted value plots, and residual-*versus*-leverage plots were constructed to test linearity assumptions.

We compared groups with IMV *versus* NIV using, for continuous variables, Student's t -test when distribution was normal and the Mann-Whitney U test otherwise, and for categorical variables, the χ^2 test if counts were above 5 and Fisher's exact test otherwise.

For most variables, less than 5% of values were missing. Duration of the main meal was unknown for 17 (14.3%) participants, including 16 fed *via* a gastrostomy. Frequency of airway clearance sessions was unknown for 9 (7.6%) participants. Missing data imputation according to Rubin's criteria (replacement by the median or most common value) did not change the findings.[2] Consequently, we report the analyses of complete cases.

The statistical analyses were performed with SAS[®] software version 9.4 for Windows (SAS Institute, Cary, NC).

SUPPLEMENTAL RESULTS

The participants enrolled in this study were followed by 18 different regional services of the *AFM-Téléthon* distributed throughout France.

Participants receiving invasive mechanical ventilation (IMV; n=80) had been on MV for a mean of 18.7 ± 9.2 years and had undergone tracheostomy 14.5 ± 10.3 years before study inclusion. More specifically, 40 participants (50% of this group) were started on NIV, and then switched to IMV after a mean of 8.3 ± 5.7 years of NIV. Tracheostomy was performed as an emergency procedure in 15 (37.5%) of them. These patients had been ventilated invasively for a mean of 10.6 ± 8.1 years at study inclusion. Thirty-nine (48.8%) participants had never experienced NIV, and 25 (64.1%) of them had undergone tracheostomy in an emergency context. Thirty-six participants (45.0 % of the IMV group) were started on ventilation directly with invasive ventilation *via* tracheostomy. Three (3.8%) participants had tracheostomy before starting mechanical ventilation (respectively, 1, 3 and 4 years before starting MV). One participant data is missing. These patients had been ventilated invasively for a mean of 18.3 ± 10.6 years at study inclusion.

Among all the participants included, the initiation of home MV required 4 (6.7%) participants to move to another home, 4 (6.7%) to a medical institution, and 1 (0.8%) to another type of institution. The remaining 110 (92.4%) participants did not change their place of residence. Of the 53 participants who were employed or students at the time of MV initiation, 27 (50.9%) stated that MV required no change in these activities, 11 (20.8%) that their work schedule had to be modified, and 15 (28.3%) that they became unable to work or study.

Of the 39 (32.8%) patients on NIV, the most common daytime interface was mouthpiece ventilation (MPV; 28 [71.8%] participants), followed by nasal masks (8 [20.5%] participants) and nasal pillows (3 [7.7%] participants). Among participants using daytime MPV, 17 (60.7%) used a nasal mask at night; 7 (25%) used nasal pillows; and 4 (14.3%) used a facemask.

Supplemental table 3 reports the results of the univariate analyses for the variables entered into the multivariate models, including details of the effects of each variable for each SRI sub-scale. Despite requiring a higher level of respiratory care (requirement for airway clearance techniques, frequency of airway clearance sessions, interactions with healthcare workers [table 1]), participants with IMV reported fewer respiratory complaints than participants with NIV (supplemental table 2). Of note, breathing difficulties during speech or eating appeared less pronounced in participants with IMV compared to participants with NIV (supplemental figure 3).

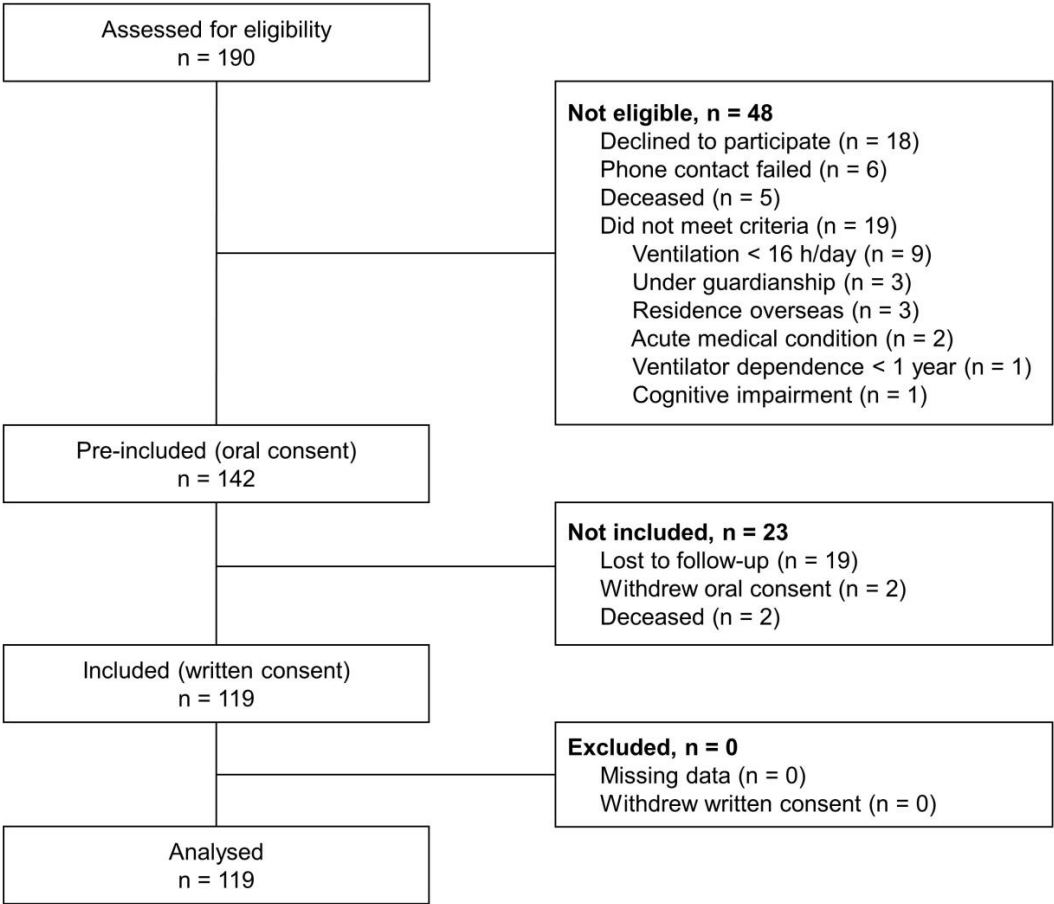
Fifty-nine (49.6%) participants had a diagnosis of Duchenne muscular dystrophy (DMD). Supplemental tables 4 and 5 respectively show the participants characteristics and HRQoL scores in the group with DMD versus other diagnoses. No significant difference in HRQoL was found between patients with DMD *versus* others.

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SUPPLEMENTAL FIGURES

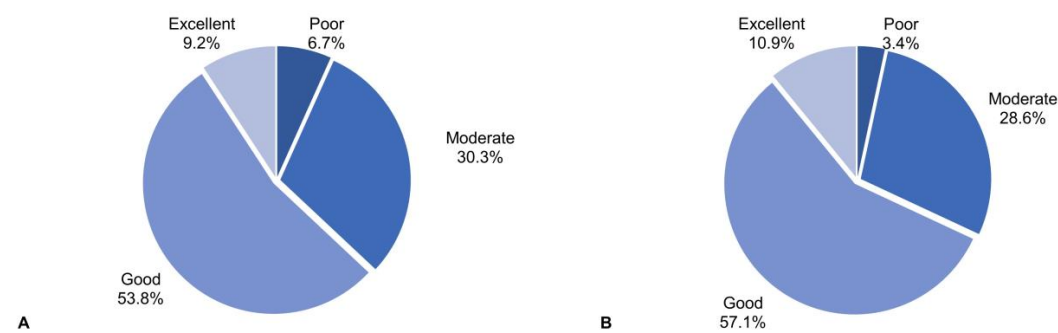
Supplemental figure 1. Patient flow diagram



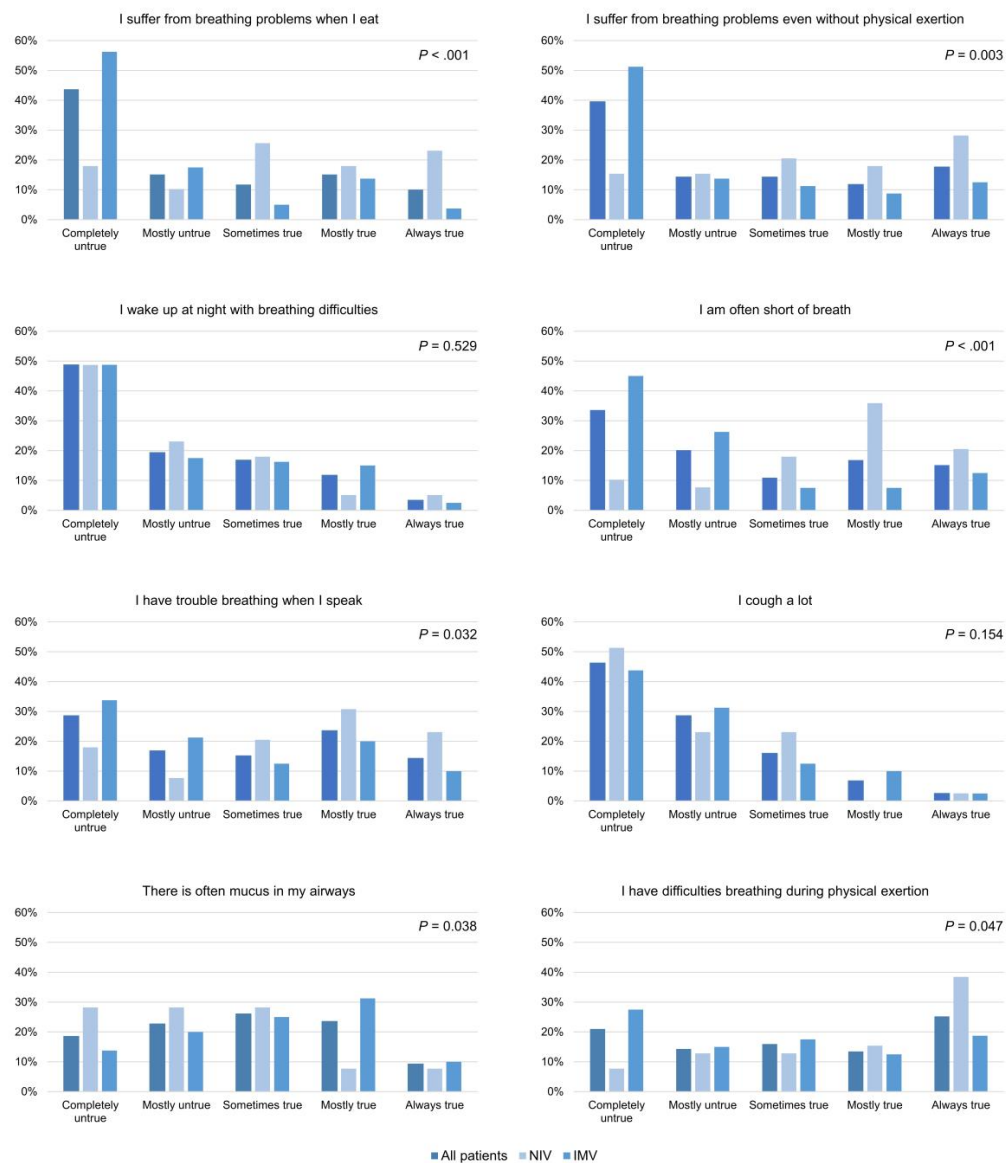
Supplemental figure 2. Perception of general health status and quality-of-life.

The data are derived from the two general items of the QoL-gNMD questionnaire.

A, health; B, quality-of-life.



Supplemental figure 3. Detailed responses to the SRI questionnaire questions related to the "Respiratory Complaints" domain and comparison between groups with invasive mechanical ventilation (IMV) versus non-invasive ventilation (NIV).



Supplemental table 1. Univariate analyses

		SRI-SS			QoL-gNMD-PS			QoL-gNMD-SP			QoL-gNMD-ASP		
	n	β	SE	P value	β	SE	P value	β	SE	P value	β	SE	P value
Continuous variables													
Age, y†	119	0.001	0.10	0.994*	0.08	0.06	0.169*	0.04	0.07	0.537*	-0.08	0.06	0.182*
Duration of main meal, min	102	-0.01	0.08	0.919	-0.06	0.04	0.196*	-0.08	0.05	0.131*	0.004	0.05	0.931
Respiratory autonomy, min	117	-0.01	0.01	0.650	-0.02	0.01	0.027*	-0.01	0.01	0.485	-0.001	0.01	0.869
Time since MV initiation, y	117	0.34	0.15	0.023*	0.17	0.08	0.034*	0.11	0.10	0.294	0.09	0.09	0.309
Time since onset of MV dependence‡, y	116	0.32	0.16	0.049	0.12	0.09	0.179	0.11	0.11	0.319	0.12	0.10	0.202
Interaction with healthcare worker§, days/week	118	-0.09	0.53	0.865	-0.57	0.29	0.051*	-0.16	0.34	0.635	-0.34	0.31	0.274
Categorical variables													
Female sex	119	-2.27	2.95	0.443	-2.75	1.61	0.090	1.07	1.96	0.586	0.55	1.73	0.753
Overweight**	119	-0.21	3.17	0.948	-1.18	1.74	0.500	-0.82	2.11	0.699	-1.74	1.85	0.350
NIV	119	-5.73	2.84	0.046*	-2.28	1.56	0.147*	-3.64	1.88	0.055*	0.84	1.68	0.617
In bed most of the time††	119	-4.83	4.83	0.319	-0.13	2.56	0.961	-1.91	3.09	0.537	-7.74	2.63	0.004
Can go outdoors with MV	119			0.156			0.217			0.495			0.003
Yes, alone		1*			1*			1*			1*		
Yes, with a caregiver‡‡		-4.96	3.56	0.166	-2.46	1.97	0.214	-1.84	2.40	0.444	-4.00	2.02	0.050*
No		-18.09	10.74	0.095*	-7.84	4.96	0.116*	-6.69	6.04	0.270	-17.18	5.09	0.001*
Residence in an urban area	116	-5.41	2.67	0.045*	-0.96	1.52	0.529	-1.39	1.74	0.425	-0.93	1.60	0.560
Inclusion during a COVID-19 national lockdown	119	-0.32	2.75	0.908	0.74	1.50	0.625	-1.49	1.81	0.412	0.86	1.60	0.592
Requirement for airway clearance techniques	119	0.56	2.89	0.845	-0.32	1.58	0.841	2.25	1.90	0.238	-1.17	1.68	0.487
Airway clearance sessions everyday	110	0.92	2.83	0.746	2.10	1.55	0.176*	1.89	1.88	0.315	-0.58	1.66	0.730
Psychotropic medication	118	-3.04	3.36	0.368	-0.78	1.85	0.673	-3.21	2.22	0.152*	-2.96	1.94	0.130*

		SRI-SS			QoL-gNMD-PS			QoL-gNMD-SP			QoL-gNMD-ASP		
	n	β	SE	P-value	β	SE	P-value	β	SE	P-value	β	SE	P-value
Diagnosis other than dystrophinopathy†	119	-0.50	2.70	0.854*	-1.03	1.48	0.489*	-0.80	1.79	0.654*	0.71	1.58	0.652*
Lives with partner or other family member(s)	119	-9.93	2.66	< .001*	-1.58	1.54	0.307	-3.85	1.84	0.039*	-3.61	1.62	0.028*
Level of education	118			0.857			0.721			0.470			0.943
Elementary school		4.41	5.15	0.394	0.70	2.84	0.805	3.45	3.41	0.313	1.47	3.04	0.630
Middle school		0.25	3.72	0.947	0.16	2.05	0.939	2.91	2.46	0.238	0.76	2.19	0.728
High school		0.97	3.25	0.765	1.94	1.78	0.278	2.78	2.14	0.196	-0.12	1.91	0.950
Lives in a medical institution	119	2.48	3.75	0.509	-2.09	2.06	0.312	0.72	2.50	0.775	1.21	2.20	0.583
Personal assistant every day	117	3.07	2.92	0.294	-1.01	1.61	0.533	-2.17	1.94	0.265	-0.19	1.72	0.910
Professional activity	119	6.55	5.05	0.197*	-4.49	2.77	0.108*	-2.86	3.36	0.399	2.23	2.98	0.456
Assistance required during meals	116	1.49	5.42	0.783	0.40	2.97	0.894	-0.08	3.57	0.982	-0.60	3.19	0.851

The data are estimate (β) and standard error (SE).

Positive values indicate an association of the variable with better quality-of-life.

* Variable included in the multivariate model ($P < 0.20$ by univariate analysis)

† Age and diagnosis (dystrophinopathy vs. other) were included by default in the multivariate models.

‡ Time since onset of MV dependence was not included in the multivariate model because it interacted with time since MV initiation.

§ Healthcare workers were defined as nurses or physiotherapists.

|| Sex was not included in the multivariate model because it interacted with diagnosis of dystrophinopathy.

** Overweight was defined as body mass index $> 25 \text{ kg.m}^{-2}$

†† In bed most of the time was not included in the multivariate model because it interacted with the ability to go outdoors.

‡‡ Caregiver was defined as a family member, personal assistant, or healthcare worker.

SRI-SS, severe respiratory insufficiency summary-scale; QoL-gNMD, quality-of-life in genetic neuromuscular disease questionnaire; QoL-gNMD-PS, impact of physical symptoms; QoL-gNMD-SP, self-perception; QoL-gNMD-ASP, activities and social participation; MV, mechanical ventilation (non-invasive or invasive); NIV, non-invasive ventilation

Supplemental table 2. Comparison of health-related quality-of-life (HRQoL) between groups with invasive mechanical ventilation (IMV) versus non-invasive ventilation (NIV).

	All patients (n=119)	NIV (n=39)	IMV (n=80)	P value
SRI				
Respiratory complaints (RC)	62.9±21.7	53.4±20.3	67.6±21.0	<.001
Physical functioning (PF)	18.9±14.8	17.4±15.0	19.6±14.8	0.449
Attendant symptoms and sleep (AS)	63.2±21.2	59.0±23.2	65.3±20.0	0.133
Social relationships (SR)	67.6±19.1	66.6±19.1	68.0±19.1	0.709
Anxiety (AX)	58.9±25.1	52.7±29.0	61.9±22.5	0.061
Psychological well-being (WB)	64.3±20.8	63.6±21.2	64.6±20.7	0.813
Social functioning (SF)	57.7±20.8	55.8±20.5	58.5±21.1	0.506
Summary scale (SS)	56.1±14.6	52.2±16.0	57.9±13.6	0.046
QoL-gNMD				
Health – general, n (%)				0.114
Poor	8 (6.7)	5 (12.8)	3 (3.8)	
Moderate	36 (30.3)	12 (30.8)	24 (30.0)	
Good	64 (53.8)	21 (53.8)	43 (53.8)	
Excellent	11 (9.2)	1 (2.6)	10 (12.5)	
Quality-of-life – general, n (%)				0.413
Poor	4 (3.4)	1 (2.6)	3 (3.8)	
Moderate	34 (28.6)	15 (38.5)	19 (23.8)	
Good	68 (57.1)	19 (48.7)	49 (61.3)	
Excellent	13 (10.9)	4 (10.3)	9 (11.3)	
Impact of physical symptoms (PS)	54.2±8.1	52.6±8.3	54.9±7.9	0.147
Self-perception (SP)	47.6±9.7	45.2±9.6	48.8±9.6	0.055
Activities and social participation (ASP)	43.1±8.6	42.6±8.3	43.4±8.7	0.617

The data are mean±SD.

For each variable, higher values indicate better quality-of-life.

NIV, non-invasive ventilation; IMV, invasive mechanical ventilation (via a tracheostomy); SRI, severe respiratory insufficiency questionnaire; QoL-gNMD, quality-of-life in genetic neuromuscular disease questionnaire.

Supplemental table 3. Univariate analyses for variables entered into the multivariate models

	Non-invasive ventilation		Time since MV initiation, y		Residence in an urban area		Lives with a partner or other family member(s)		Can go outdoors with MV*		
									Yes, with a caregiver†	No	
	β (SE)	P-value	β (SE)	P-value	β (SE)	P-value	β (SE)	P-value	β (SE)	β (SE)	P-value
SRI											
Respiratory complaints (RC)	-14.22 (4.06)	0.001	0.45 (0.22)	0.047	-12.43 (3.93)	0.002	-4.68 (4.17)	0.264	-4.00 (5.37)	-11.94 (13.52)	0.604
Physical functioning (PF)	-2.20 (2.90)	0.449	0.07 (0.15)	0.654	-0.88 (2.80)	0.754	-11.55 (2.65)	<.0001	-4.30 (3.61)	-17.83 (9.10)	0.129
Attendant symptoms and sleep (AS)	-6.24 (4.13)	0.133	0.35 (0.21)	0.106	-6.64 (3.94)	0.095	-5.73 (4.06)	0.161	-0.50 (5.26)	-1.76 (13.26)	0.990
Social relationships (SR)	-1.40 (3.73)	0.709	0.35 (0.20)	0.072	-5.31 (3.52)	0.134	-10.57 (3.54)	0.003	-6.98 (4.67)	2.71 (11.76)	0.248
Anxiety (AX)	-9.16 (4.84)	0.061	0.77 (0.25)	0.003	-5.84 (4.67)	0.214	-15.17 (4.62)	0.001	-8.40 (6.17)	-2.83 (15.53)	0.383
Psychological well-being (WB)	-0.97 (4.07)	0.813	0.17 (0.21)	0.440	-6.43 (3.84)	0.097	-7.05 (3.95)	0.077	-4.24 (5.12)	-12.43 (12.91)	0.545
Social functioning (SF)	-2.74 (4.11)	0.506	0.14 (0.22)	0.522	1.42 (3.93)	0.718	-13.20 (3.83)	<.001	-6.32 (5.10)	-23.55 (15.38)	0.220
Summary scale (SS)	-5.73 (2.84)	0.046	0.34 (0.15)	0.023	-5.41 (2.67)	0.045	-9.93 (2.66)	<.001	-4.96 (3.56)	-18.09 (10.74)	0.156
QoL-gNMD											
Impact of physical symptoms	-2.28 (1.56)	0.147	0.17 (0.08)	0.034	-0.96 (1.52)	0.529	-1.58 (1.54)	0.307	-2.46 (1.97)	-7.84 (4.96)	0.217
Self-perception	-3.64 (1.88)	0.055	0.11 (0.10)	0.294	-1.39 (1.74)	0.425	-3.85 (1.84)	0.039	-1.84 (2.40)	-6.69 (6.04)	0.495
Activities and social participation	-0.84 (1.68)	0.617	0.09 (0.09)	0.309	-0.93 (1.6)	0.560	-3.61 (1.62)	0.028	-4.00 (2.02)	-17.18 (5.09)	0.003

The data are estimate (β) and standard error (SE).
Positive values indicate that the variable was associated with better quality-of-life.

* Reference: Yes, alone.
† Caregiver was defined as a family member, personal assistant, or healthcare worker.

SRI-SS, severe respiratory insufficiency summary-scale; QoL-gNMD, quality-of-life in genetic neuromuscular disease questionnaire; QoL-gNMD-PS, impact of physical symptoms; QoL-gNMD-SP, self-perception; QoL-gNMD-ASP, activities and social participation; MV, mechanical ventilation (non-invasive or invasive).

Supplemental table 4. Main features of the study participants in the groups with Duchenne muscular dystrophy versus other diagnoses.

	Duchenne muscular dystrophy (n=59)	Other diagnoses (n=60)	P value
Features			
Age, y	34.0 [28.0–41.0]	43.0 [33.8–54.3]	<.001
Female sex, n (%)	2 (3.4)	33 (55.0)	<.001
BMI, kg.m ⁻²	19.1 [16.3–22.8]	22.0 [18.0–26.1]	0.044
Respiratory care			
Time since MV initiation, y	13.5 [8.3–19.0]	20.0 [13.5–24.0]	<.001
Time since onset of MV dependence*, y	9.0 [3.0–13.0]	10.0 [5.0–20.0]	0.034
Respiratory autonomy, min	5.0 [1.0–60.0]	21.3 [3.0–120.0]	0.009
NIV, n (%)	23 (39.0)	16 (26.7)	0.152
Requirement for airway clearance techniques, n (%)	45 (76.3)	35 (58.3)	0.037
Airway clearance sessions, n/week	2.8 [0.0–7.0]	4.5 [0.0–7.0]	0.680
Functional autonomy			
Personal assistant every day, n (%)	42 (71.2)	41 (68.3)	0.728
In bed most of the time, n (%)	3 (5.1)	8 (13.3)	0.294
Can go outdoors with MV, n (%)			0.850
Yes, alone	10 (16.9)	10 (16.7)	
Yes, with a caregiver†	48 (81.4)	48 (80.0)	
No	1 (1.7)	2 (3.3)	
Assistance required during meals, n (%)	56 (94.9)	52 (86.7)	0.083
Duration of main meal, min	42.5 [30.0–60.0]	37.5 [30.0–60.0]	0.482
Place of residence, n (%)			
Urban area	31 (52.5)	35 (58.3)	0.591
Medico-social institution	9 (15.3)	9 (15.0)	0.904
Living arrangements, n (%)			
Lives alone or in shared accommodations‡	21 (35.6)	21 (35.0)	0.891
Lives with a partner	3 (5.0)	16 (26.7)	0.002
Lives with other family members	34 (57.6)	23 (38.3)	0.027
Other variables			
Interactions with healthcare workers§, days/week	4.0 [2.0–7.0]	6.0 [2.0–7.0]	0.387
Psychotropic medication, n (%)	11 (18.6)	13 (21.7)	0.716
Inclusion during a COVID-19 lockdown, n (%)	19 (32.3)	30 (50.0)	0.049
Level of education, n (%)			0.780
Elementary school	5 (8.5)	5 (8.3)	
Middle school	10 (16.9)	14 (23.3)	
High school	18 (30.5)	20 (33.3)	
University	25 (42.4)	21 (35.0)	
Professional activity, n (%)	2 (3.4)	7 (11.7)	0.163

Data are presented as n (%) or median [25th–75th percentiles].

* MV dependence was defined as daily ventilation ≥16 hours/day for at least the past year.

† Caregiver was defined as a family member, personal assistant, or healthcare worker.

‡ The patients in sharing arrangements did not require help from those living in the same home.

§ Healthcare workers were defined as nurses or physiotherapists.

BMI, body mass index; MV, mechanical ventilation (non-invasive or invasive); NIV, non-invasive ventilation.

Supplemental table 5. Comparison of health-related quality-of-life (HRQoL) between groups with Duchenne muscular dystrophy versus other diagnoses.

	Duchenne muscular dystrophy (n=59)	Other diagnoses (n=60)	P value
SRI			
Respiratory complaints (RC)	64.3 (19.4)	61.6 (23.9)	0.498
Physical functioning (PF)	18.7 (14.4)	19.1 (15.3)	0.875
Attendant symptoms and sleep (AS)	66.0 (18.5)	60.5 (23.5)	0.161
Social relationships (SR)	64.4 (19.1)	70.7 (18.6)	0.074
Anxiety (AX)	60.3 (22.2)	57.5 (27.7)	0.544
Psychological well-being (WB)	63.0 (20.2)	65.4 (21.5)	0.530
Social functioning (SF)	57.3 (20.2)	58.0 (21.6)	0.859
Summary scale (SS)	56.1 (13.8)	56.1 (15.4)	0.982
QoL-gNMD			
Health – general, n (%)			0.106
Poor	5	3	
Moderate	14	22	
Good	37	27	
Excellent	3	8	
Quality-of-life – general, n (%)			0.814
Poor	3	1	
Moderate	16	18	
Good	34	34	
Excellent	6	7	
Impact of physical symptoms (PS)	54.4 (7.6)	53.9 (8.6)	0.720
Self-perception (SP)	47.8 (9.3)	47.5 (10.2)	0.865
Activities and social participation (ASP)	42.9 (8.3)	43.4 (8.9)	0.759

The data are mean±SD.

For each variable, higher values indicate better quality-of-life.

NIV, non-invasive ventilation; IMV, invasive mechanical ventilation (via a tracheostomy); SRI, severe respiratory insufficiency questionnaire; QoL-gNMD, quality-of-life in genetic neuromuscular disease questionnaire.