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Understanding Adherence to Noninvasive Ventilation in Youth with Duchenne Muscular Dystrophy

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Abstract

Duchenne muscular dystrophy (DMD) is an X-linked, progressive neuromuscular disorder that results in chronic respiratory insufficiency and subsequently failure requiring non-invasive ventilation (NIV). Adherence to NIV in neuromuscular disorders and related barriers are poorly described. The aim of the current study was to assess NIV adherence, adherence barriers, and identify psychosocial predictors of adherence in young boys with early DMD-related sleep disordered breathing and recommended nocturnal NIV.

This cross-sectional study included 42 youth with DMD with prescribed nocturnal NIV, and their caregivers. Caregivers and youth completed questionnaires assessing adherence barriers, psychosocial symptoms (e.g. anxiety and depressive symptoms), and stress. Medical information pertinent to cardiopulmonary health and neurologic status at both enrollment and initiation of NIV was reviewed.

Adherence to NIV, defined as percent days used and days used ≥ 4 hours/day was 56.1±38.7% and 46.2±40.6%, respectively. Average duration of use on days worn was 5.61±4.23 hours. NIV usage was correlated with the severity of obstructive sleep apnea but not cardiopulmonary variables. Mask discomfort was the most commonly reported adherence barrier followed by behavioral barriers (e.g., refusing to use). Multiple regression analyses revealed that internalizing behaviors (e.g., anxiety and depressive symptoms) and total adherence barriers significantly predicted NIV adherence.

Adherence to NIV in DMD is poor and similar to other pediatric chronic diseases. Our data suggest interventions targeting adherence barriers and patient internalizing symptoms may improve adherence to NIV in DMD.

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Keywords

Adherence; Barriers; Bilevel Positive Airway Pressure; Noninvasive Ventilation; Duchenne Muscular Dystrophy; Children

Introduction

Duchenne muscular dystrophy (DMD) is an X-linked, progressive neuromuscular disease that affects one in 3,500 male births[1]. Initially, the proximal striated muscles manifest weakness, however, all striated muscles, including the heart and diaphragm eventually succumb. Current treatments are largely symptom-based and have lengthened survival from the second to the third decade of life[2]. The impact of ventilator support is notable, lengthening survival from 19 years in those not on support, to 27 years[3]. Ultimately, the cause of death for DMD patients is frequently related to progressive cardiorespiratory failure.

DMD is characterized by gradual dependence on noninvasive ventilation (NIV) that becomes life-sustaining in the advanced stages. The current American Academy of Sleep Medicine (AASM) definition of hypoventilation has limitations in the neuromuscular population. Sleep-disordered breathing in DMD evolves over years from mild obstructive sleep apnea (OSA) with or without subtle hypoventilation to chronic respiratory failure. Sleep disordered breathing is reported as early as 12 years in boys with DMD on glucocorticoids[4]. Boys with DMD and mild sleep-disordered breathing in the early stages of NIV usage experience minimal, if any, daytime sequelae of untreated sleep-disordered breathing. Thus, nonadherence to NIV should be suspected. The progression of respiratory muscle weakness culminates into full dependence on mechanical ventilation, a stage where nonadherence has catastrophic consequences. There are limited data pertaining to NIV adherence in children with neuromuscular disease.

In contrast, several pediatric studies have examined adherence to positive airway pressure (PAP) therapies for obstructive sleep apnea. PAP adherence is often arbitrarily operationalized as ≥ 3 hours[5,6] or ≥ 4 hours per night[7–10]. A recent review found that the use of PAP therapy ranged 3.8–7.0 hours per night[6,9] with the majority of children not using their machines nightly[5]. Thus, estimates may reflect the machine being turned on but not necessarily with effective pressure delivered[9]. Self-report also overestimates usage compared to objective measurement[6,11]. These data highlight the need for objective measurement of adherence.

An important next step in the field of NIV and PAP therapy adherence is the identification of barriers to device use; this information can lead to the development of evidence-based interventions for children with neuromuscular disease and DMD, in particular. To date, only one study has examined adherence barriers in four patients with neuromuscular disease[8]. Adherence based on objective and self-reported data was 75%. The most prevalent adherence barriers were minimal symptom relief, negative attitude toward NIV, and prior experience of alleviated symptoms of an illness after an intervention. Non-adherent youth also had a tendency to challenge caregiver authority[8]. While these data provide some initial

evidence of NIV adherence barriers, no studies have been conducted solely in youth with DMD.

Existing studies have identified physical, behavioral, familial, and psychosocial factors that affect adherence for youth using PAP therapy. Prior studies on physical factors did not find a relationship between adherence and sleep-disordered breathing severity, therapeutic pressure, and PAP therapy type (continuous or bilevel)[5–7,9,12,13]. Behavioral, familial, and psychosocial domains appear more influential in PAP therapy adherence. Both parents and children have identified embarrassment, forgetting, simply refusing to use, illness, choosing to ignore obstructive sleep apnea, and refusing to transport the machine out of the home as reasons for nonadherence[14]. Mask style[7] and younger age at initiation also correlate with adherence[7,9,15]. Among adolescents, better adherence was associated with more organized households and an authoritative parenting style that allowed for informed autonomy for the adolescent[15]. These factors are highly relevant in boys with DMD as NIV initiation commonly occurs in early adolescence.

Embedded within the parent-child interaction is the degree of stress experienced by caregivers[16–19]. Families of children with neuromuscular disease experience greater stress than those other pediatric conditons[20] due to caregiver[17,18,21–23] and child factors[16,21,23]. Boys with DMD often have impaired verbal IQ related to underlying disease pathology,[24–26] which in turn contributes to behavior problems and family stress[26]. Despite this, caregivers view their role in providing daily care for their dependent sons as extremely important and fulfilling[27].

The current study sought to identify rates of adherence and barriers to NIV use in boys with DMD and assess medical, psychosocial, and demographic predictors of adherence at a single outpatient clinic visit. First, absolute adherence rates, defined as usage ≥ 4 hours per night, duration of NIV, and percent of days used by electronic download were examined. Consistent with other pediatric conditions,[28] adherence was hypothesized to be 50%. Adherence was hypothesized to be higher in patients with more advanced neuromuscular disease, irrespective of sleep disordered breathing severity. The second aim was to examine barriers to NIV (e.g. mask discomfort, disbelief in the need for NIV, belief that NIV signals disease progression in DMD, and requiring assistance with NIV). The final aim was to identify psychosocial predictors of adherence. Child age, parental stress, and child mental health were hypothesized to be significant predictors of treatment non-adherence.

Methods

Inclusion criteria for this cross-sectional study were as follows: 1) confirmed diagnosis of DMD, 2) children and adolescents age 8 to 18 years, 3) previously prescribed nocturnal NIV and 4) ability to read and speak English due to availability of questionnaires. Participants with tracheostomy dependence were excluded. This cohort was prescribed NIV due to obstructive sleep apnea with hypoventilation as defined by the American Academy of Sleep Medicine (AASM).

Procedures

The study was approved by the Institutional Review Board. Participants were recruited from the Comprehensive Neuromuscular Center at Cincinnati Children's Hospital Medical Center. Study eligibility was determined by review of medical records prior to a clinic visit. During the clinic visit, informed consent/child assent were obtained. Caregiver and patients then completed a battery of questionnaires. Youth with reading difficulties were aided by the research assistant. If cognitive ability precluded questionnaire completion, only caregiver measures were given. Objective adherence was measured via electronic download from NIV machines at the current clinic visit. Medical chart review was completed following the clinic visit.

Measures

Demographics Form.—Caregivers completed a form assessing basic demographic information about the child (i.e. sex, date of birth, and race/ethnicity).

Stress Index for Parents of Adolescents (SIPA).—The SIPA is measure of parenting stress in parents of adolescents ages 11–19. For the purpose of the present study, the four domains used (Life Restrictions, Relationship with Spouse/Partner, Social Alienation, Incompetence/Guilt) were reported on Total Parental Stress. Coefficient alpha for the parent domain was 0.94[29]. Clinically significant scores are considered ≥ 90th percentile and at-risk scores are 85–89th percentiles.

Children's Yale-Brown Obsessive-Compulsive Scale-Parent Report.—The CY-BOCS-PR is a 10 item parent-report questionnaire that assesses distress and impairment caused by obsessions (5 items) and compulsions (5 items) in the youth. Higher scores represent more severe impairment: subclinical (0–7), mild (8–15), moderate (16–23), severe (24–31), and extreme (32–40). Internal consistency coefficients ranged from 0.70–0.86 for the subscales (i.e., obsession, compulsion, total)[30].

Children's Florida Obsessive Compulsive Inventory (CFOCI).—The CFOCI is a 17 item self-report screener for pediatric obsessive-compulsive disorder, in which children endorse whether they experienced symptoms in the last month. Two subscales (i.e., obsessions and compulsions) and a total score are reported. Higher scores denote more symptoms. Internal consistency is adequate at 0.76[31].

Behavior Assessment Schedule for Children:2 (BASC-2).—Caregivers and children completed the parent-) and self-reported BASC-2, which are objective assessments of behavioral difficulties in children and adolescents. Several composite scores can be calculated. For this study, we used the internalizing (e.g., anxiety, depression) and externalizing (e.g., attention problems, aggression, hyperactivity) standardized T-scores. T-scores < 60 denote normative functioning, T-scores 60–69 denote at-risk behaviors that may warrant clinical attention, and T-scores >70 are considered clinically significant and represent high levels of maladjustment.

Adherence and Barriers to DMD Treatment.—The patient and caregiver completed an adherence barriers checklist, which included physical (e.g., mask discomfort, pressure intolerance) and behavioral items (e.g., inconvenience, reminder of progression of disease), reflecting their experiences at the current clinic visit, not at NIV initiation.

Objective Adherence Data: Objective adherence data pertaining to the current visit were downloaded from the NIV machines using manufacturer-specific proprietary software. These are universally used in sleep clinics to assess adherence. Data analyzed included days with/without usage, percent days used, average duration of usage on the nights NIV was worn, and percent days with usage ≥ 4 hours.

Medical Chart Review: Electronic medical chart review was conducted by the PI (pulmonology), and included the child's current age, age at loss of ambulation, age at recommended NIV initiation, apnea hypopnea index (AHI) and obstructive apnea/hypopnea index (OAHI) at diagnostic polysomnography, completion of NIV titration polysomnography, steroid usage, and the number of current prescribed medications excluding those administered "as needed".

Current motor function was assessed by multiple scales. The functional mobility score ranges from 1–6, with 6 representing ambulatory on all surfaces and 1 being wheelchair dependent. The performance of the upper limb scale was utilized for nonambulatory subjects. Three levels are assessed: shoulder, middle, and distal mobility for a maximum combined score of 74. High scores signified more mobility and internal consistency is excellent at 0.96[32].

Pulmonary function test (PFT) items included body mass index (BMI), forced vital capacity percent predicted (FVC%) and absolute value FVC. The timepoints were at NIV initiation and the present visit when adherence, barriers, and questionnaires were being assessed. PFT reference values are from C-NHANES III and ATS criteria for acceptability and repeatability are utilized[33].

Cardiac imaging data included left ventricular ejection fraction (LVEF) by echo or cardiac MRI, and presence of late gadolinium enhancement (LGE) as an indicator of myocardial scarring. Cardiorespiratory variables were collected +/− six months surrounding the date when NIV was initiated and the current visit.

Statistical analyses

Descriptive statistics (e.g., means/sd, frequencies) were calculated for adherence rates, barriers, and predictor variables. Independent t-tests were used to assess subgroup differences on NIV adherence: LGE and NIV titration polysomnography study completed. Multiple regression analyses were used to identify the individual, family, medical and sociodemographic predictors on NIV adherence data. Specifically, four separate regression models were examined in which the outcome variables included 1) NIV percent usage ≥ 4 hours and 2) percent days NIV use. Predictor variables were separated by child and caregiver-report, including child age, total prescribed medications, BASC Externalizing Problems T Score (parent report), BASC Internalizing Problems T Score (child and parent

report), BASC Emotional Symptoms Index (child), CFOCI (child), CYBOCS Total Score (parent), SIPA Index of Total Parenting Stress percentile (parent), and total barriers (child and parent). All analyses were conducted with SPSS v23 with statistical significance considered as $p < 0.05$.

Results

Demographic, medical, and adherence data are summarized in Table 1. Percent days NIV used was 56.1±38.7% of 287±309 total days. Average usage on these days was 5.61±4.23 hours. The percent days used ≥ 4 hours was 46.2±40.6%.

Youth with DMD reported 2.7±2.8 adherence barriers and caregivers reported 1.7±1.8 adherence barriers to NIV (Table 2). The most common barriers reported by children and caregivers were uncomfortable mask, poor sleep with NIV, and refusing to use the machine. Parent-reported obsessive-compulsive symptoms in the youth were clinically elevated in over half the sample (Table 3); however, parenting stress and parent and child-reported internalizing and externalizing symptoms were low.

Relationship between Adherence, Medical/Demographic, and Psychosocial Variables

The OAHI at diagnostic polysomnography was associated with adherence (i.e., percent of days NIV was used ≥ 4 hours). Additionally, duration of NIV usage on days used was associated with the OAHI (r=0.37, p<0.05). The change in absolute FVC from initiation of NIV to current clinic visit was associated with percent days of NIV use. No other cardiorespiratory variables were associated with percent of days of NIV use or percent of days NIV was used ≥ 4 hours (Table 4). No group differences were found on the adherence variables for LGE (percent days used: $t(27)=-0.33$, p=ns and percent days used ≥ 4 hours: $t(26)=-0.39$, p=ns), or completion of a titration polysomnogram (percent days used: $t(38)=-0.64$, p=ns and percent days used ≥ 4 hours: $t(37)=-0.37$, p=ns).

A higher number of caregiver adherence barriers were associated with lower adherence (percent days used ≥ 4 hours adherence; $r=-0.45$; p=0.002 and percent days used; $r=-0.44$; p=0.003). Caregiver-reported internalizing problems was associated with percent days used adherence (r=0.38; p=0.01). Total barriers reported by children was associated with worse adherence for both percent days used ≥ 4 hours ($r=-0.60$; p<0.001) and percent days used ($r=-0.65$; p<0.001). Child reported internalizing problems was also associated with adherence by percent days used ≥ 4 hours (r=0.32; p=0.035).

Predictors of Adherence

NIV percent usage ≥ 4 hours.—Multiple regression analyses for the caregiver variables explained 12.2% of the variance, $F(7, 29)=1.72$, however p=ns. Total caregiver barriers was the only significant predictor of adherence (beta=−0.43, p<0.05) (Table 5). A separate multiple regression analysis revealed that child variables explained 51.6% of the variance in NIV percent usage ≥ 4 hours, $F(6, 30)=7.39$, p<0.001. Total child barriers (beta=−0.65, p<0.001) and BASC internalizing problems (beta=0.88, p<0.01) were significant predictors of NIV percent usage > 4 hours (Table 5).

Percent days NIV use.—Multiple regression for the caregiver variables explained 25.1% of the variance, $F(7, 30)=2.77$, $p=0.024$, with total caregiver barriers (beta=−0.38, $p<0.05$) and BASC Internalizing Problems T Score (0.53, $p<0.05$) as significant predictors (Table 5). For the child model, 54.9% of the variance was explained in percent days NIV use, $F(6, 31) = 8.52$, $p<0.001$. Total child barriers (beta=−0.71, $p<0.001$) and BASC internalizing problems (beta=0.58, $p<0.05$) were identified as significant predictors (Table 5).

Discussion

This study contributes to the existing DMD literature by examining objective NIV adherence rates, adherence barriers, and identifying key psychosocial factors that contribute to NIV non-adherence. Although the minimal level of NIV adherence needed to achieve a therapeutic benefit is unknown, our data indicated that adherence to NIV was suboptimal at 56.1% of days used, and 46.2% of days used ≥4 hours in patients with DMD on glucocorticoids and preserved lung function. However, the average usage on days used of 5.61 hours is comparable to other studies evaluating adherence to PAP therapy in obstructive sleep apnea among children with chronic conditions[6,7,14]. Not surprisingly, our adherence rates are consistent with the pediatric adherence literature[28].

The relationship between NIV adherence and disease severity was assessed with proxies of cardiovascular and pulmonary health (e.g., FVC%, LGE, LVEF, and change in FVC% and absolute FVC). Our results indicated that percent days used correlated to absolute FVC change; adherence was otherwise not related to other measures of cardiopulmonary function. This finding may be explained by the fact that 98% of our patients were prescribed glucocorticoid therapy, a standard of care that delays the onset of progressive muscle weakness and subsequent cardiorespiratory failure[34]. Our cohort demonstrated clinical stability and preserved lung function with an average FVC% of 80% at 15 years of age. Patients with such preserved lung function but with reduced physical abilities are unlikely to challenge their respiratory reserve, and therefore remain asymptomatic during the day. Without daytime sequelae, NIV adherence may be lower.

Contrary to our hypothesis, the severity of sleep-disordered breathing did correlate with NIV adherence. Youth with an elevated OAHI were more likely to use their NIV longer (e.g., more hours) on a nightly basis. However, elevated OAHI did not correlate with the percent of nights NIV was used. One explanation for longer nightly use may be improved daytime symptoms of sleep disordered breathing when using NIV. Intuitively, a patient should feel best on the optimal therapeutic positive pressure as determined by a titration polysomnogram. Yet, having completed a titration polysomnogram was not associated with improved adherence. The empiric pressures initially chosen to desensitize a patient prior to titration may partially treat OSA. This may create a gradual continuum of symptom resolution, mirroring the insidious onset of OSA symptoms.

Mask discomfort was the most reported barrier by caregivers and their children. Sleep disruption, which was also endorsed, may have been related to mask discomfort, leak, and pressure intolerance. In addition, simple refusal, forgetting, and inconvenience of using NIV were identified as behavioral barriers to NIV adherence. These barriers are not unique to our

patient population[7,14,35]. Although we anticipated that not wanting others to know about the machine and the machine serving as a reminder of disease progression would be reported as NIV barriers[14], few of the parent-child dyads endorsed these barriers. This may be due to patients manifesting and coping with other physical aspects of the disease with an altered gait, wheelchair dependence, and/or Cushingoid facies due to chronic glucocorticoids.

Another key contribution of the current study findings is assessment of the psychosocial functioning of boys with DMD and their caregivers. Overall, boys with DMD did not exhibit significant internalizing (e.g., anxiety, depression) or externalizing problems compared to age matched normative data, with the exception of obsessive-compulsive tendencies. The psychosocial literature suggests better parental adjustment and more family involvement decreases behavioral symptoms in youth with DMD[19,21]. Familial stress can also significantly impact depression and psychosocial functioning in this population. Although caregiver stress remains elevated[18–20,27], it can improve over time[16] even as their youth's disease progresses. Our results may have been influenced by the broad referral base with patients travelling long distances to clinic, lending bias toward organized families with greater resources.

In order to develop interventions to improve NIV adherence, determination of modifiable predictors is necessary to identify intervention targets. Our results indicate that more adherence barriers and child internalizing symptoms (e.g. anxiety, depression) predicted lower NIV adherence. These variables are amenable to evidence-based adherence interventions and can serve as a first step in developing strategies to improve NIV adherence in boys with DMD. For example, follow-up phone calls to assess and remotely address adherence barriers have been efficacious in children with physical and cognitive delays[7]. The same effort could double as a screen for youth who may need more intensive behavioral therapy provided by a psychologist. Such intervention has been shown to be effective in increasing NIV usage from 1.72 to 8.58 hours/night after a single 90 minute session[36]. Unpublished data from our center reveals improved adherence to CPAP therapy consolidated efforts from the interdisciplinary clinical team that comprises a sleep medicine physician and psychologist, nurse, respiratory therapist, and dietician. A similar model may be beneficial in the neuromuscular population.

It is imperative to note that these results should not be generalized to patients with more advanced DMD that experience diurnal respiratory failure. The question of when to initiate NIV in the evolution of sleep disordered breathing in patients with neuromuscular disease is paramount but difficult to answer. Waiting to obtain a polysomnogram until the FVC% is 60% is felt among pediatric neuromuscular pulmonologists to be too late. Sleep disordered breathing in young boys with DMD with preserved lung function is well described. Further, alveolar hypoventilation in neuromuscular disease has not been clearly defined. The AASM definition of hypoventilation takes its origin from adult OSA pathology and fails to take into consideration compensatory physiologic mechanisms to maintain eucapnia at the expense of sleep quality. Recent literature suggests obtaining a diagnostic polysomnogram with any symptoms of sleep disordered breathing and treating with bilevel pressure support with a backup rate[37].

This study represents an important contribution to the DMD literature but carries some limitations. First, the sample size was small due to the rare nature of the disease, making it difficult to power for larger scale analyses. Second, participants completed questionnaires during their routine interdisciplinary clinic visits that often span 4–5 hours. Thus, at times, children and their caregivers completed the measures in the same room, potentially influencing each other's responses. Thirdly, the questionnaires limited the age of enrolled patients to those with less advanced disease. Consequently, our ability to find an association between adherence and disease severity was inherently compromised. This could conversely be considered a strength as adherence had more potential to be low thus rendering opportunity for improvement with future interventions. Lastly, the downloads of NIV adherence did not have a standardized timespan as patients in this stage of their disease from farther distances, have less frequent clinic follow up. Future prospective studies should monitor adherence as patients traverse the evolution of DMD. Similarly, multisite trials examining adherence patterns in centers that initiate NIV at different clinical time points would enable researchers to include a range of disease severity.

This study provides insight into factors influencing adherence to NIV therapy among boys with DMD and will help direct future intervention efforts. While physical features of NIV were noted as barriers, behavioral issues, which are amenable to intervention, may be critical in improving adherence. Our study demonstrates the importance of a multi-disciplinary approach to encouraging NIV adherence with physician providers, respiratory therapists, nurses, and psychologists. Meta analyses suggest that multicomponent interventions providing education and behavioral strategies, including technology, can yield adherence improvements[38–40]. Testing these types of interventions in research or even clinical practice can potentially impact the progression of disease for boys with DMD.

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Table 1.

Demographics and Medical Information (n=42)

Variable	M (SD) or %
Years of Age at Study Enrollment (Current Visit)	15.1 (2.2)
Years of Age at Loss of Ambulation	11.9 (2.8)
Years at Recommended Non-Invasive Ventilation (NIV) Initiation (n=41)	12.2 (1.9)
Race	
• White	95%
• Asian	2.5%
• Biracial	2.5%
Ethnicity	
• Hispanic	5%
• Non-Hispanic	95%
Ambulatory Status	
• Ambulatory	38%
• Non-ambulatory	62%
Mobility Score	
• Functional Mobility Score	4.57 (1.9)
• Performance of Upper Limb (n=22)	22.5 (10.5)
Diagnostic Polysomnogram (n=40)	
• Apnea Hypopnea Index	6.7 (4.6)
• Obstructive Apnea/Hypopnea Index	5.8 (4.4)
NIV Titration Study Completed	71%
Steroids prescribed	98%
Total Prescribed Medications (excluding PRN)	10.6 (3.2)
At time of Noninvasive Ventilation Initiation	
Forced Vital Capacity % Reference (n=41)	94.4 (22.7)
Body Mass Index (n=41)	24.6 (5.7)
Left Ventricular Ejection Fraction % (n=36)	59.9 (7.8)
Presence of Late Gadolinium Enhancement If Cardiac MRI Completed (n=28)	39%
At Current Visit	
Forced Vital Capacity % Reference (n=41)	79.9 (29.4)
BMI (n=41)	26.5 (5.5)
Left Ventricular Ejection Fraction % (n=38)	56.7 (6.4)
Presence of Late Gadolinium Enhancement If Cardiac MRI Completed (n=29)	55%
Decline from NIV Initiation to Current Visit	
Forced Vital Capacity % Reference (n=40)	14.0 (22.3)
Forced Vital Capacity Absolute Value (Liters) (n=40)	0.014 (0.41)

Variable	M (SD) or %
Adherence and Barriers Data to NIV	
Days with Device Usage (N=39)	175.8 (239.6)
Days without Device Usage (N=34)	96.74 (126.2)
Percent Days Used (N=40)	56.1 (38.7)
Average Usage Per Day (N=40) in Hours	5.61 (4.23)
Percent Days Used ≥ 4 Hours (n=39)	46.2 (40.6)
Average Number of Adherence Barriers Endorsed	
• Caregivers	1.7 (1.8)
• Children	2.7 (2.8)

Table 2.

Percent of Participants who Endorsed Adherence Barriers

Barrier	Child (n=40)	Caregiver (n=42)
Forgets to Use the Machine	32.5%	5%
He Doesn't Need the Machine	10%	0%
Don't Understand How Machine Works	7.5%	2%
Don't Want Others to Know About Machine	10%	Not Applicable
Don't Want to Transport	15%	5%
Can't Afford the Machine	2.5%	2%
Using Machine Prevents Sleeping Well	32.5%	19%
Machine is Loud	12.5%	9.5%
Refuses to Use Machine	22.5%	36%
Using Machine is Inconvenient	22.5%	14%
Reminder That Disease is Progressing	7.5%	7%
Mask is Uncomfortable	40%	36%
Air Pressure is Uncomfortable	15%	9.5%
Feels Suffocating	10%	9.5%
Claustrophobic With Mask On	10%	5%
Mask Difficult to Put On	10%	2%
Symptoms Not Better With Usage	7.5%	5%

Table 3.

Caregiver and Child Psychosocial Data

Variable	Mean (SD)	% Clinically Elevated
Caregiver (n=42)		
Children's Yale-brown Obsessive Compulsive Scale	8.69 (7.94)	52%
Stress Index for Parents of Adolescents (n=40)	48.2 (16.3)	2.5%
BASC: Externalizing Problems T Score	51.2 (9.5)	2%
BASC: Internalizing Problems T Score	55.0 (9.9)	5%
Child (n=40)		
Children's Florida Obsessive Compulsive Inventory	2.33 (2.93)	Not Applicable
BASC: Emotional Symptoms Index T Score	45.9 (8.2)	0% (5% At-risk)
BASC: Internalizing Problems T Score	44.8 (8.0)	0% (5% At-risk)

Table 4.

Correlations between Adherence and Child and Caregiver Variables of Interest

Variable	Percent days used ≥ 4 hours per night	Percent days used
Medical Variables	N=39 †	N=40 ‡
Forced Vital Capacity % Predicted Current Visit	−0.13	−0.15
Forced Vital Capacity % Predicted Decline	−0.02	0.03
Forced Vital Capacity Absolute Value Decline	0.26	0.36 *
Left Ventricular Ejection Fraction Current Visit	0.13	0.05
Apnea Hypopnea Index	0.26	0.18
Obstructive Apnea/Hypopnea Index	0.34 *	0.27
Caregiver Variables	n=37	n=38
Child Age in Years	−0.12	−0.15
Children's Yale-brown Obsessive Compulsive Scale	−0.002	0.004
Stress Index for Parents of Adolescents	0.14	0.13
BASC Externalizing Problems T Score	0.01	0.001
BASC Internalizing Problems T Score	0.26	0.38 **
Total Caregiver Barriers	−0.45 **	−0.44 **
Total Prescribed Medications (excluding PRN)	−0.06	−0.07
Child Variables		
Child Age in Years	−0.01	−0.02
Children's Florida Obsessive Compulsive Inventory	0.18	0.21
BASC: Emotional Symptoms Index T Score	0.15	0.22
BASC: Internalizing Problems T Score	0.30 *	0.32 *
Total Child Barriers	−0.60 ***	−0.65 ***
Total Prescribed Medications (excluding PRN)	0.05	0.04

Note:

* $p < 0.05$,

** $p < 0.01$,

*** $p < 0.001$. Data were missing for some of these variables and thus N's ranged from

† 36–39 and

‡ 38–40.

Table 5.

Regression Analyses Predicting Adherence

Predicting Percent days ≥ 4 hours NIV use		
	β	***Adjusted R^2***
Caregiver Model		**0.12**
• Child Age in Years	−0.15	
• Total Prescribed Medications (excluding PRN)	0.08	
• Total Caregiver Barriers	−0.43 *	
• CYBOCS	−0.05	
• BASC Externalizing T Score	−0.10	
• BASC Internalizing T Score	0.28	
• Stress Index for Parents of Adolescents	0.11	
Child Model		**0.52**
• Child Age in Years	−0.06	
• Total Prescribed Medications (excluding PRN)	0.16	
• Total Child Barriers	−0.65 ***	
• CFOCI	0.11	
• BASC Emotional Symptom T Score	−0.59	
• BASC Internalizing T Score	0.88 **	
Predicting Percent Days of NIV Use		
	β	***Adjusted R^2***
Caregiver Model		**0.25**
• Child Age in Years	−0.14	
• Total Prescribed Medications (excluding PRN)	0.06	
• Total Caregiver Barriers	−0.38 *	
• CYBOCS	−0.05	
• BASC Externalizing T Score	−0.28	
• BASC Internalizing T Score	0.53 *	
• Stress Index for Parents of Adolescents	0.10	
Child Model		**0.55**
• Child Age in Years	−0.04	
• Total Prescribed Medications (excluding PRN)	0.14	
• Total Child Barriers	−0.71 ***	
• CFOCI	0.13	
• BASC Emotional Symptom T Score	−0.25	
• BASC Internalizing T Score	0.58 *	

Note:

* $p < 0.05$,

** $p < 0.01$,

*** $p < 0.001$