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SCIENTIFIC INVESTIGATIONS

Determinants of usage and nonadherence to noninvasive ventilation in children and adults with Duchenne muscular dystrophy

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Study Objectives: Duchenne muscular dystrophy (DMD) is a neuromuscular disorder that leads to chronic respiratory insufficiency and failure. Use of home noninvasive ventilation (NIV) has been linked to improved outcomes including reduced mortality. Despite the importance of NIV, factors promoting optimal NIV usage and determinants of nonadherence have not been rigorously examined. Moreover, given that respiratory issues in DMD span between childhood and adulthood, examination across a broad age group is needed. The objectives of this study were to (1) evaluate NIV usage across a broad spectrum of patients with DMD, including both children and adults, and (2) identify biological and socioeconomic determinants of NIV usage and NIV nonadherence.

Methods: We performed a retrospective review of all patients with DMD from February 2016 to February 2020 who underwent evaluation at associated pediatric and adult neuromuscular disease clinics. NIV use was determined objectively from device downloads. A priori, we defined nonadherence as < 4 hours use per night, quantified as the percentage of nights below this threshold across a 30-day period within 6 months of a clinic visit. We also assessed the average hours of NIV usage over this time period. Predictors examined included demographics, social determinants, and pulmonary function.

Results: 33 patients with DMD were identified, 29 (87%) of whom were using NIV (13 age < 21 years). Mean age was 22.9 ± 6.6 years (range 13–39 years), body mass index was 23.4 ± 10.4 kg/m^2, and seated forced vital capacity was 23% ± 18% predicted. Mean nightly NIV usage was 7.4 ± 3.8 hours and mean percentage of nonadherent nights was 13% ± 30%. In univariable analysis, age did not predict use. Those with lower forced vital capacity had higher NIV usage hours (P = .01) and a trend toward less nonadherence (P = .06). Higher estimated household income demonstrated a trend toward increased usage hours and less nonadherence (both P = .08). Multivariable analysis found increased usage hours were predicted best by higher income, higher inspiratory positive airway pressure, and higher bicarbonate. Nonadherence was higher in those with lower income or higher forced vital capacity.

Conclusions: In this cohort of adult and pediatric patients with DMD, most individuals were using NIV. While usage hours were higher with lower lung function, substantial variability remains unexplained by examined factors. Nonadherence was observed in some individuals, including those with advanced disease. Further investigations should focus on evaluating patient-oriented outcomes to define optimal NIV usage across the spectrum of disease and determine strategies to counteract issues with nonadherence.

Keywords: adherence, children, Duchenne muscular dystrophy, noninvasive ventilation, bilevel positive airway pressure, usage

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BRIEF SUMMARY

Current Knowledge/Study Rationale: Duchenne muscular dystrophy is a neuromuscular disorder causing chronic respiratory insufficiency necessitating noninvasive ventilation (NIV). Despite the importance of NIV, factors promoting optimal NIV usage and determinants of nonadherence have not been rigorously examined.

Study Impact: In this cohort of adult and pediatric patients with Duchenne muscular dystrophy, most individuals were using NIV. While usage hours were higher with lower lung function, substantial variability remains unexplained by examined factors. Nonadherence was observed in some individuals, including those with advanced disease. Further investigations should focus on evaluating patient-oriented outcomes to define optimal NIV usage across the spectrum of disease and determine strategies to counteract issues with nonadherence.

INTRODUCTION

Duchenne muscular dystrophy (DMD) is a progressive neuromuscular disorder affecting approximately 1 in 3,600 male newborns worldwide.[1] The disorder is caused by a mutation in the DMD gene resulting in defective or absent dystrophin protein that is inherited in an X-linked recessive fashion or sporadic.[2] Clinically, affected males demonstrate progressive muscular weakness, contractures, skeletal deformity, and cardiomyopathy. Respiratory muscle weakness leads to hypoventilation and chronic respiratory failure and is a major cause of mortality, with few individuals surviving beyond the third decade of life.[3,4] Presently, noninvasive ventilation (NIV) is the preferred mode of respiratory support for patients with respiratory insufficiency related to respiratory muscle weakness.[5,6] In DMD, NIV device use has been shown to substantially prolong survival and improve respiratory outcomes.[7,8] As one would expect, the success of NIV therapy is contingent upon adequate device use.[9] In

other breathing disorders, the benefits of positive airway pressure therapies are dose dependent, with greater improvements derived from consistent nightly use.[10] In patients with obstructive sleep apnea (OSA) syndrome, a number of factors have been associated with increased positive airway pressure use, such as age, sex, and socioeconomic status.[11] Despite the known benefit of NIV therapy in DMD, there are limited studies examining determinants of NIV use across the spectrum of disease.

Given the substantial variability in respiratory insufficiency across individuals with DMD, as well as the progressive nature of the disease, optimal NIV use is difficult to define. "Adherence" has been used as a dichotomous method to classify use, with patients deemed adherent if using the NIV device ≥ 4 hours/night.[12,13] While benefits might not be expected if use is lower than this threshold (ie, "nonadherence"), even consistent use just above this threshold is unlikely to represent optimal usage for all individuals, particularly as respiratory insufficiency progresses. Studies examining the impact of NIV use on patient-oriented outcomes across the spectrum of disease are not presently available to individualize an adherence threshold.

With this background, the aim of our study is to (1) evaluate patterns of NIV use across a broad spectrum of DMD patients, including both children and adults, and (2) identify biological and/or socioeconomic determinants of NIV therapy use. We hypothesize that declining lung function, increasing age, and socioeconomic factors will lead to greater use of NIV devices in children and adults with DMD.

METHODS

Study population

This retrospective cross-sectional study included all patients ≥ 1 year of age with a genetically confirmed diagnosis of DMD prescribed NIV who underwent evaluation at the Rady Children's Hospital San Diego Pediatric Neuromuscular Clinic or the University of California, San Diego Adult Neuromuscular Clinic between February 1, 2016 and February 3, 2020. NIV therapy was initiated in patients based on physician assessment of lung function, symptoms, blood gasses, and/or sleep testing if needed.[14] In all patients, NIV therapy had been initiated at least 30 days prior to data collection and patients were advised to use their NIV device during all sleeping hours by a respiratory therapist at every clinic encounter. We examined all patients with DMD, including those with a tracheostomy using continuous invasive ventilation, but these individuals were excluded from the final analysis. As our focus was on patients utilizing NIV, we also excluded patients examined in the associated clinics that were not utilizing NIV at the time of the study (n = 6).

Study design

This study was approved by the Institutional Review Board at the University of California, San Diego. Subject demographics, concurrent diagnoses, growth parameters, and clinical features were collected from electronic medical chart review using the most recent neuromuscular clinic visit as the cross-sectional time point for data collection. Pulmonary function tests (PFT) were performed using C-NHANES III reference values and American Thoracic Society criteria for acceptability and repeatability.[15] Pulmonary function test parameters were evaluated at the time of the neuromuscular clinic visit or the most recent (< 6 months) neuromuscular clinic visit and included percent predicted and absolute value for seated forced vital capacity (FVC) and forced expiratory volume in 1 second.

Objective NIV data were abstracted from the time period 30 days and 90 days prior to the most recent neuromuscular clinic visit. To obtain objective assessment, we utilized manufacturer device-specific software to obtain remote monitoring reports (ResMed AirView, Philips EncoreAnywhere, and Philips Respironics Care Orchestrator). The 30-day time period is a standard time for evaluation of sleep-disordered breathing. The 90-day time period was additionally selected as it aligned with clinic follow-up and allowed for inclusivity of all patients examined. Report data examined included NIV device type, settings, the number and percentage of nights used, the number and percentage of nights with < 4 hours use, the number and percentage of nights with ≥ 4 hours use, and the average hours used per night across all nights. A priori, we defined nonadherence as NIV for < 4 hours per night and quantified as a continuous variable by determining the percentage of nights below this threshold across a 30-day period within 6 months of the neuromuscular clinic visit. The rationale is that if use was < 4 hours per night, NIV was unlikely to be effective respiratory support in this patient population. NIV usage hours were defined by average hours used across all nights.

Potential determinants of nonadherence and usage hours were examined and included biological, socioeconomic, and medical-utilization variables. Biological variables examined included: age, percent predicted seated FVC, body mass index (kg/m^2), age at NIV initiation, left ventricular ejection fraction, serum bicarbonate, median unintentional leak, median observed end-expiratory positive airway pressure, median observed inspiratory positive airway pressure, previously diagnosed OSA, and history of steroid treatment. Socioeconomic variables examined included estimated median household income, English vs not English preferred primary language, Hispanic vs non-Hispanic ethnicity, number of household parents, and type of insurance. Median household income was inferred from demographic postal code using the 2018 United States Census Bureau report for household income.[16,17]

Acute medical utilization as a potential predictor of NIV nonadherence and usage hours was examined. These included number of lower respiratory tract infections, antimicrobial prescriptions, emergency room visits, and inpatient hospitalizations all occurring within the previous 12 months from the clinic evaluation. We also examined the relationship between 30-day and 90-day NIV therapy use.

Statistical analysis

Descriptive statistics were used to summarize baseline demographic and clinical characteristics. Dichotomous predictors of use were examined using the independent samples *t* test. Continuous predictors of use were examined using simple linear regression. To determine independent predictors of NIV use, all univariable predictors with $P \leq .10$ were examined in

backward-selection multivariable linear regression. A P value of $< .05$ was deemed statistically significant for all analyses. All statistical analyses were performed using IBM SPSS Statistics software versions 26 and 27.

RESULTS

Thirty-three individuals with clinically and genetically confirmed diagnosis of DMD were identified. Four (12%) were tracheostomy dependent on continuous invasive mechanical ventilation and excluded from the final analysis as the focus of our study was adherence issues surrounding NIV. Of the 29 included individuals on NIV, 13 (45%) represented pediatric patients with age < 21 years that were receiving care at a children's hospital. The mean age was 22.9 ± 6.6 years and mean body mass index was 23.4 ± 10.4 kg/m^2. The mean age at diagnosis was 4.6 ± 3.1 years, age at initiation of NIV device was 15.8 ± 5.5 years, and seated forced vital capacity (FVC) was 23% ± 18% predicted (**Table 1**).

Twenty (69%) individuals were Hispanic and 22 (76%) reported that English was their preferred primary language. The average household income was 62,393 ± 23,776 US dollars, which fell below the county mean household income of 79,646 US dollars.[16,17] Government-funded or public insurance was utilized by 21 (72%), and 18 (62%) had only 1 parent in the home. Eleven (38%) reported 2 parents in the household, and of those, 1 (9%) patient reported a maternal aunt as an additional caregiver. Medical utilization in the 12 months prior to the most recent clinic visit was evaluated and demonstrated 9 (30%) required 1 or more hospitalizations for respiratory related issues. Additionally, 14 (48%) were prescribed antimicrobials for respiratory related illness within the past 12 months prior to their clinic visit (**Table 2**).

In regards to NIV mode, 24 (83%) utilized bilevel positive airway pressure in the spontaneous timed mode, with the remaining 5 (17%) utilizing volume assured pressure support. Median observed inspiratory positive airway pressure (IPAP) and expiratory positive airway pressure was 15.1 ± 4.5 and 6.1 ± 2.5 cm H_2O, respectively. Compliance reports provided objective data on mean hourly NIV usage and nonadherence with 30-day reports correlating to 90-day reports (rs = 0.72; P = .00006 and rs = 0.93; P = .00001). On average, our population used their NIV device 83% ± 32% of nights averaged over the 30-day period prior to documented clinic visit. The mean percentage of nights used ≥ 4 hours was 79% ± 35% with mean device usage of 7.4 ± 3.8 hours on all days used (**Table 3**).

Table 1—Population data and pulmonary function.

	Mean ± SD (min–max)	n
Population Data		
Age (y)	22.9 ± 6.6 (7–38)	29
Age at diagnosis (y)	4.6 ± 3.1 (0.1–11.9)	21
Age NIV initiated (y)	15.8 ± 5.5 (6–26)	23
BMI (kg/m^2)	23.4 ± 10.4 (9.9–51.6)	29
LV ejection fraction (%)	47.9 ± 10.9 (30–65)	23
Median household income (USD)	62,393 ± 23,776 (30,554–103,176)	29
Pulmonary Function		
FEV1 (%)	24 ± 19 (6–74)	26
FVC (%)	23 ± 18 (5–68)	26
Serum bicarbonate (mEq/L)	27 ± 3 (24–35)	19

BMI = body mass index, FEV1 = forced expiratory volume in 1 second, FVC = forced vital capacity, LV = left ventricular, NIV = noninvasive ventilation, SD = standard deviation, USD = US dollars.

Table 2—Socioeconomic and medical data.

Demographic	n	%
Pediatric noninvasive ventilation	13	44.8
Adult noninvasive ventilation	16	55.2
Socioeconomic Factors		
Hispanic	20	69.0
Non-Hispanic	9	31.0
English language preferred	22	75.9
Non-English language preferred	7	24.1
1 parent in the home	18	62.1
2 parents in the home	11	37.9
Public/government-funded insurance	21	72.4
Private insurance	18	27.6
Hospital Utilization		
Respiratory tract illness*	6	20.7
Antimicrobial*	14	48.3
Emergency room visit*	7	24.1
Hospitalization*	9	31.0

*Event occurred within 12 months preceding the clinic visit.

Biological factors

There was no association between nonadherence (ie, percentage of nights with device usage < 4 hours) and age (β = 0.02, 95% CI −2.05 to 2.08; P = .99) nor was there an association between device usage hours and age (β = 0.01, 95% CI, −0.22 to 0.23; P = .91) (**Figure 1A** and **Figure 1B**). While there was no statistically significant association between nonadherence and FVC (β = 0.70, 95% CI, −0.0.3 to 1.43; P = .06), there were significantly higher usage hours in those with lower FVC (β = −1.1, 95% CI, −0.19 to −0.03; P = .01) (**Figure 1C** and **Figure 1D**). While there was no significant association between nonadherence and observed median IPAP (β = −3.69, 95% CI, −8.00 to 0.60; P = .09), there was a statistically significantly association between usage hours and IPAP (β = 0.58, 95% CI, 0.15 to 1.02; P = .01). There was no significant relationship between nonadherence or device usage hours and other examined biological factors, including body mass index (P = .19 and P = .22, respectively), age at NIV initiation (P = .54 and P = .43), left ventricular ejection fraction (P = .14 and P = .57), serum bicarbonate (P = .12 and P = .08), median unintentional leak (P = .97 and P = .29), observed median expiratory positive airway pressure (P = .88 and P = .32), prior

Table 3—Noninvasive ventilation device parameters and usage.

	30-Day Report	90-Day Report
	Mean ± SD	Mean ± SD
Ventilation Parameters		
Bilevel ST*, n (%)	24 (82.8)	24 (82.8)
VAPS, n (%)	5 (17.2)	5 (17.2)
Median EPAP (cm H_2O)	6.1 ± 2.5	6.3 ± 1.9
Median IPAP (cm H_2O)	15.1 ± 4.5	15.4 ± 3.6
Median respiratory rate (bpm)	12.6 ± 1.7	12.6 ± 1.7
Unintentional leak (L/min)	14.5 ± 15.9	10.4 ± 11.9
Usage Data†		
Nights with any use (%)	82.8 ± 32.1	81.2 ± 31.7
Nights used > 4 h (%)	79.4 ± 34.6	77.6 ± 32.4
Nights used < 4 h (%)	20.6 ± 34.6	22.4 ± 32.4
Usage on total nights (h)	7.4 ± 3.8	6.2 ± 3.3
Usage on nights used (h)	8.0 ± 3.5	7.8 ± 2.5

*Bilevel positive airway pressure was delivered in spontaneous-timed mode. †Usage data are described as mean value averaged over the 30- or 90-day time period. EPAP = end-expiratory positive airway pressure, IPAP = inspiratory positive airway pressure, ST = spontaneous-timed, VAPS = volume-assured pressure support.

OSA diagnosis ($P = .32$ and 0.44), or history of steroid treatment ($P = .87$ and 0.28).

Socioeconomic factors

There was no statistically significant association between nonadherence or device usage hours and English vs not English as preferred primary language ($P = .24$ and $P = .10$) (**Figure 2A** and **Figure 2B**), although potential trends were noted. Similarly, there was no significant association between nonadherence or usage hours and median household income, although a trend was noted ($P = .08$ and $P = .08$) (**Figure 2C** and **Figure 2D**). There was no significant relationship between nonadherence or device usage hours and other examined socioeconomic factors, including Hispanic vs not Hispanic ($P = .89$ and $P = .41$), number of household parents ($P = .91$ and $P = .46$), and private vs government-funded insurance ($P = .53$ and $P = .74$).

Health care utilization factors

There was no significant difference in nonadherence or NIV usage between patients that had vs had not experienced 1 or more respiratory tract infections within the past year ($P = .84$ and $P = .50$). Similarly, there was no difference in nonadherence or NIV usage in patients that were prescribed antimicrobials ($P = .74$ and $P = .50$), utilized the emergency room for respiratory-related issue ($P = .99$ and $P = .32$), or were hospitalized for respiratory-related illness within the 12 months preceding their clinic visit ($P = .53$ and $P = .51$).

Multivariable analysis

Predictors included in the multivariable model and model statistics are summarized in the supplemental material (**Table S1** and **Table S2**). Univariable predictors ($P \leq .10$) of nonadherence to NIV included FVC, observed median IPAP, and median household income. Independent predictors of higher nonadherence were lower median household income and higher FVC. The overall model had a modest correlation ($r = .500$, $P = .02$).

Univariable predictors of device usage hours included FVC, observed median IPAP, serum bicarbonate, preferred primary language, and median household income. Independent predictors of higher usage hours were higher household income, higher median IPAP, and higher serum bicarbonate. The overall model had modest correlation ($r = .659$; $P < .01$).

DISCUSSION

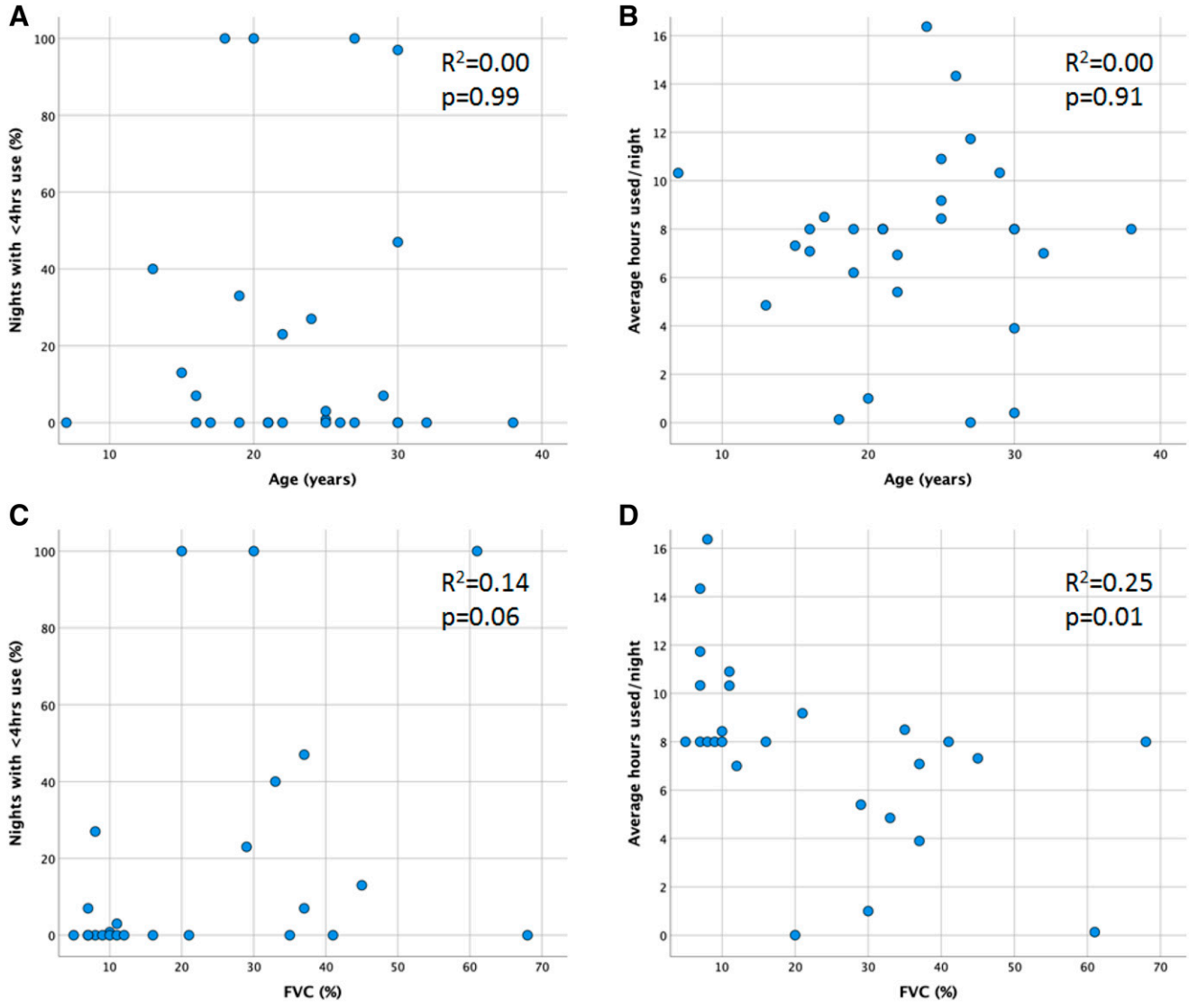
The major findings of this study were as follows: (1) among children and adults with DMD, NIV usage hours were high on average but also variable across individuals with a minority of patients classified as nonadherent according to traditional thresholds, (2) patients in households with lower income have lower NIV usage hours and more nonadherence, (3) declining percent FVC function (and other markers of disease severity such as higher IPAP and higher bicarbonate) modestly corresponded with improved use patterns, and (4) substantial variability in NIV use was not explained by the examined variables.

NIV use in DMD

Despite recent advances in the care of children and adults with DMD, respiratory issues remain a leading cause of mortality.[18] The progressive weakness of respiratory muscles contributes to sleep-disordered breathing, restrictive lung disease, and chronic respiratory insufficiency that necessitate respiratory support. NIV improves quality of life and survival in these individuals, yet there are little data specifically focused on use patterns.[19] A recent study by Pascoe et al[20] examined NIV adherence and device usage in children with DMD. Adherence, defined as percentage of nights used > 4 hours, was 56% on average, but with substantial variability. Similarly, the same study reported average duration of use on days worn as 5.6 ± 4.2 hours. By comparison, our study demonstrated that overall NIV device usage was robust with individuals using their device on the majority of nights. Seventy-nine percent of patients utilized NIV for at least 4 or more hours, with an average NIV usage of 7.4 ± 3.8 hours. In addition, our study is novel in including both pediatric and adult patients, which is crucial since patients with DMD typically initiate NIV in late adolescence and usually survive into adulthood.

The question remains as to what constitutes adequate or optimal NIV usage in the population with DMD. In terms of patients with OSA, adherence has been defined as device usage for more than 4 hours/night for more than 70% of nights.[21,22] This definition of adherence likely has limited applicability outside adults with OSA.[23] In DMD, progression of disease leads to an increased need for ventilatory support, both in terms of pressure settings and in terms of hours used. However, to our knowledge, there are no patient-oriented outcomes studies to guide specific recommendations. Furthermore, the extent to which daytime lung function

Figure 1—Influence of biological variables with NIV usage and nonadherence.



There was no association between nonadherence and age (P = .99) **(A)** or usage hours and age (P = .91) **(B).** While there was no significant association between nonadherence and FVC **(C)** (P = .06), there were significantly higher usage hours in those with lower FVC **(D)** (P = .01). FVC = forced vital capacity, NIV = noninvasive ventilation.

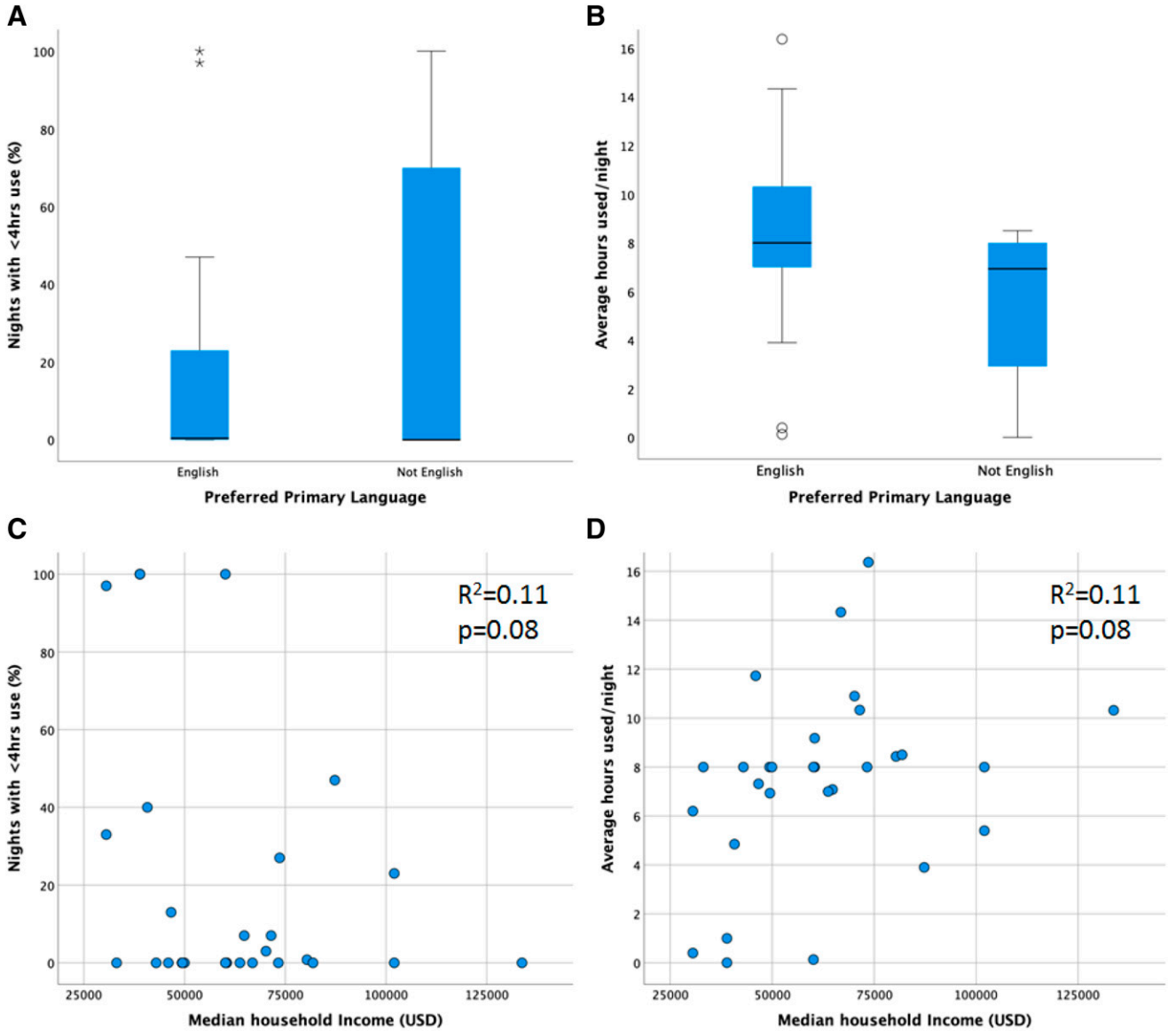
testing predicts ventilatory support needs in terms of hours of use are unclear. Despite the difficulties in defining adequate adherence in this population, there is certainly reason to suspect that NIV device usage less than 4 hours (ie, nonadherence) might indicate ineffective treatment and thus would be a reasonable metric to examine.[24] Future studies must include prospective longitudinal data with outcomes assessment to better define optimal NIV usage at an individual level.

Factors promoting NIV device use

Facilitators and barriers to positive airway pressure device use have been primarily investigated in patients with OSA using CPAP. In adults with OSA, CPAP adherence ranges from 40 to 85%, whereas children are more varied at 24–87%, with average daily usage of 5.2 ± 3.4 hours.[25–27] A recent systematic review found that young age, female sex, and developmental delay were associated with CPAP usage.[28] Children with OSA on positive airway pressure have demonstrated improved usage with factors such as caregiver support, authoritative parenting style, and stable family structure.[21,29,30] Additional studies have demonstrated that race, maternal education, and medical comorbidities may influence usage in OSA.[31–33] Whether such factors might apply to NIV in DMD is unclear. To the authors' knowledge, only 1 previous study has examined factors influencing NIV usage in the population with DMD. The aforementioned study was limited to children and reported that internalizing behaviors such as anxiety and depressive symptoms significantly predict NIV usage but did not investigate socioeconomic determinants.[20]

In our study, we did see an important relationship between lower lung function and better NIV use. Severity of disease has

Figure 2—Influence of socioeconomic factors on NIV usage and nonadherence.



There was no association between nonadherence and English vs not English as preferred primary language **(A)** ($P = .24$) or between device usage hours and English vs not English as preferred primary language despite some numerically apparent differences **(B)** ($P = .10$). Similarly, there was no statistically significant association between nonadherence ($P = .08$) **(C)** or usage hours ($P = .08$) **(D)** and median household income despite a visual trend toward worse use in those with lower income. **Subjects with outlying points. NIV = noninvasive ventilation, USD = US dollars.

been previously shown to influence positive airway pressure usage in the general population; however, pulmonary function in DMD has not been extensively examined as a determinant of NIV usage.[34,35] For most patients with DMD, nocturnal NIV may be considered when FVC is less than 50% predicted.[36] In our cohort, pulmonary function was skewed toward severe restriction, which may have contributed to the robust NIV device use. Interestingly patient age did not influence NIV usage, although it has been a predictor of adherence to CPAP in children with OSA.[13,37,38] One potential contributor toward this observed difference may be that patients with DMD in our study on average were initiated on NIV at a greater average age than patients with OSA in recent studies.[28] Nonetheless, substantial variability in NIV use was unexplained by lung function, including identification of those with a pattern of nonadherence. Thus, clinicians should not assume that a patient will up-titrate their use as their disease progresses. Indeed, clinical experience shows that symptoms that might prompt an increase in use (eg, dyspnea, orthopnea) vary widely across individuals.

Higher median neighborhood income has previously been found to predict increased positive airway pressure use.[39] Our study population on average fell below the median household income for the county but showed a significant trend for better NIV use with increasing median household income. Interestingly

despite the impact of household income, other economic factors such as type of insurance did not influence NIV usage. In children with OSA on CPAP therapy, insurance type has similarly not affected rates of NIV usage or adherence.[40] Additional studies will help to clarify the challenges that economic factors might place on NIV adherence in the DMD population.

Our study found potentially increased issues with nonadherence in patients who did not identify English as their preferred primary language. This suggests that primary spoken language is integral to promoting use of a medical device in DMD. Patient language preference has been previously reported to influence medication adherence in both adults and children.[41,42] English language is primarily utilized at our neuromuscular clinic visit with media translation service available for all other languages. However, we are uncertain regarding the availability and effectiveness of language translation services for vendors associated with durable medical equipment maintenance and repairs.

Utilization of health care for acute respiratory issues did not seem to influence NIV use. Lower respiratory tract infection, antimicrobial prescription, emergency room utilization, and hospitalization in the 12 months preceding the clinic visit did not significantly impact nonadherence or use hours. The lack of association might reflect that some patients get acutely ill despite efforts toward remaining healthy, while in others, acute illness is an indicator of nonadherence to recommendations. Nonetheless, an acute episode such as hospitalization is likely to represent a sentinel and high-risk event, and thus could be a key target for addressing any issues with suboptimal NIV use.

Limitations

There were a number of important limitations to this study, largely related to the retrospective nature of investigation. First, there may have been survival bias given that NIV influences survival in DMD. For example, those with very poor lung function might not have been alive if not using NIV, which would falsely strengthen the predictive utility of lung function toward NIV use. Conversely, we may have underestimated the impact of other factors toward poor NIV use. Second, we used a clinically available dataset. For median household income, we inferred from ZIP code rather than actual individual household income. We did not perform analysis on individual cost of living or additional sources of income. There is also a risk of selection bias such that patients of low household income may not have presented to the clinic due to resource limitations or mortality. Regarding language, we were not able assess English proficiency, patient/parent education level, or preferred learning modality. Lastly, as an observational study, we acknowledge that these data are associative in nature, and longitudinal and interventional studies will be needed to draw casual conclusions.

CONCLUSIONS

Using a cohort of adult and pediatric patients with DMD on nightly NIV, usage was relatively high and few patients were nonadherent. NIV device use was higher in those with more severe pulmonary involvement and with higher median household income. Language barriers might also be important. The progressive and variable nature of DMD contributes to the challenge of defining optimal NIV device use. Future multicenter, prospective studies are needed evaluating longitudinal outcomes to determine factors influencing use and to define optimal NIV use criteria across the spectrum of disease.

ABBREVIATIONS

CPAP, continuous positive airway pressure
DMD, Duchenne muscular dystrophy
FVC, forced vital capacity
IPAP, inspiratory positive airway pressure
NIV, noninvasive ventilation
OSA, obstructive sleep apnea

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DISCLOSURE STATEMENT

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