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TITLE: Benefits of powered standing wheelchair devices for adolescents with Duchenne muscular dystrophy in the first year of use

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All authors contributed to the design and running of the study and preparation of the manuscript. NV collected adolescent and parent reported data, SP measured the joint angles in standing, and PJ and JD conducted the analyses. KB, SP, NV, CS and JD wrote the first draft of the manuscript. All authors have critically reviewed the final version. All authors have approved the final article.

Abstract

Aim: Poorer physical and mental health often accompany loss of walking in Duchenne muscular dystrophy. This study assessed the impacts of Powered Wheelchair Standing Device (PWSD) use on muscle and joint pain, joint angles when standing and mental health in adolescents with Duchenne muscular dystrophy.

Methods: Fourteen adolescents and parents participated in a stepped wedge design study over 12 months. During a baseline and intervention period, adolescents described pain and mental health, and parents reported their child's mental health. Video data were collected to measure hip, knee and ankle joint angles in the preferred standing position.

Results: Compared with baseline and adjusting for covariates, standing wheelchair use was associated with no change in muscle or joint pain or videoed joint angles in standing. Child-reported Strengths and Difficulties total scores decreased (coefficient -3.1, 95%CI -4.6, -1.5); and parent-reported Personal Adjustment and Role Skills Scale total scores increased (coefficient 7.9, 95%CI 3.3, 12.5).

Conclusions: PWSD use was associated with maintenance of musculoskeletal status and advantages to mental health. Long term observations are necessary to improve understanding of how to support wellbeing in adolescents with Duchenne muscular dystrophy.

Keywords

Duchenne muscular dystrophy; musculoskeletal; mental health; powered wheelchair

Introduction

Duchenne muscular dystrophy (DMD) is an X-linked degenerative neuromuscular disorder caused by a mutation on the dystrophin gene[1] that occurs in approximately 1 in 5000 male births[2] and occasionally affects females.[3] Ability to walk is lost during adolescence. While their peers are increasing their independence,[4] individuals with DMD become more reliant on their carers for everyday needs. A Powered Wheelchair Standing Device (PWSD) with powered control to support standing is one example of a support for retaining independence when disease is progressing.[5]

A PWSD could also be useful to mitigate accompanying physical and mental health issues. For example, mild to moderate levels of pain were experienced regularly by the majority of 43 individuals with DMD aged 8 to 18 years, most commonly in the back and lower limbs.[6] Stretch and changing body position were commonly used strategies to manage pain.[6] The development of lower limb contractures with reduced ambulation is also likely,[7, 8] particularly progression of ankle plantarflexion which can preclude the ability to tolerate supported standing.[9] Ongoing therapy programs include active and passive stretching regimens with use of ankle-foot orthoses[10] but these programs do not always prevent contracture.[7, 9] The PWSD provides capacity for more frequent change in body position which could mitigate against increasing deformity, as observed in four individuals with DMD who used a PWSD over a six to 12-month period.[11] Poorer emotional wellbeing has been observed, particularly when walking becomes limited[12, 13] with accompanied lifestyle

restrictions, reduced independence and social isolation. Greater independence in daily activities has been associated with improved mental health in DMD[14] consistent with qualitative data indicating that using a PWSD provides greater independence for self-care activities and social participation and appears to support mental health.[5]

There are limited quantitative data on the relationships between PWSD use in DMD and physical and mental health. The aims of this study were to quantify associations between PWSD use and pain, joint angles during standing and mental health in adolescents with DMD.

MATERIALS AND METHODS

A modified stepped wedge trial was conducted with 14 adolescents (aged nine to 16 years who were provided with a PWSD) and their primary caregiver. Study approval was obtained from the Human Research Ethics Committees at the Child and Adolescent Health Services, Western Australia (2015012EP); the Queensland Children's Hospital, Queensland (HREC/16/QRCH/27); the Women's and Children's Hospital, South Australia (HREC/16/WCHN/103); and the Royal Children's Hospital, Victoria (36372A).

Adolescents with a diagnosis of DMD were recruited after their local therapist recommended that they receive a PWSD (Levo C3; www.levo.net.au). Parents reported their child's height, weight, mobility level, fracture history and current treatments including

medication use. The Egen Klassifikation (EK) scale, a 10-item scale that describes functional abilities during the non-ambulatory stage of the disease (e.g., abilities to stand, turn in bed, cough) was administered to describe functional abilities.[15] After receiving the chair, it was recommended that the adolescent use the standing function of the PWSD for at least 60 minutes each day, a duration that could be accumulated in multiple bouts of at least 10 minutes over the course of the day.

Study participants were distributed across five states of Australia and telephone interviews were used to collect adolescent- and parent-reported data. Questionnaire materials were sent to each participating family for them to view during telephone administration of the questionnaires. Interviews were conducted at approximately fortnightly intervals with the adolescent and parent, until ten assessments had been conducted with each family and were collected over approximately a 20-week period. Baseline data were collected before the PWSD was provided to the child; the duration of this period was variable due to logistical details of chair arrival, fitting and provision to the child. The control period could not be randomised because of this variability of chair availability and this was therefore a modified stepped wedge trial (Figure 1). The baseline period was a median of 9 (range 2 to 42) weeks duration, depending on the timing of chair provision. The intervention period was a median of 16 (range 6 to 20) weeks. After the first six families had participated, additional measures of child-reported pain and parent-reported child mental health were introduced to the study protocol.

Adolescent reported variables

The adolescent participant was asked to rate the intensity of muscle and joint pain using the Wong-Baker Faces Pain Rating Scale, a horizontal scale of 6 faces scored from 0 to 10 ranging from a smiling face meaning “no pain” to a crying face meaning “hurts worst” face.[16] The Strengths and Difficulties Questionnaire (SDQ), an emotional and behavioural screening questionnaire, was also administered.[17] The SDQ contains 20 items each scored on a three-point scale. The total possible score is 40 and scores below 15 are within the normal range. Domain scores describing emotional symptoms, conduct problems, hyperactivity/inattention and peer relationships can also be calculated.[17]

Parent reported variable

The Personal Adjustment and Role Skills Scale (PARS III) is a parent-reported measure of their child’s psychological and social functioning which has been specifically developed for children with chronic physical illness. It comprises 28 items rated on a four-point scale with higher scores indicating better wellbeing.[18] The PARS III was validated for DMD in 287 five to 18-year old individuals and a score lower than 72 was identified as suggesting risk of adjustment problems.[19] This questionnaire was administered to the parent of the adolescent participant at fortnightly intervals.

Video assessment

Digital videography was requested soon after receiving the PWSD and again after approximately six months. A filming protocol was provided for parents or therapists to film the adolescent undertaking the transition from sitting to the preferred standing position and then returning to sitting in the PWSD, recording from the front and then from the side. Measures were taken wearing shoes. Video recordings of all sagittal plane views of sit to stand were analysed using the Kinovea software program (www.kinovea.org v0.8.15), a technique shown to have similar reliability to goniometry in children with cerebral palsy.[20] Hip knee and ankle joint centres were visually approximated during multiple viewings of the video. Lines were then drawn for the sole of the shoe, ankle to knee joint, knee to hip joint and hip joint to approximately mid-shoulder joint to construct angles. Hip, knee and ankle angles in the preferred standing position were then measured. Angles recorded were the ankle angle (plantar grade would be 90 degrees with dorsiflexion less than 90 degrees), knee angle (180 degrees would indicate knee straight) and hip angle (180 degrees would indicate hip straight).

Analysis

Descriptive statistics were employed to outline the characteristics of the study participants and mean and standard deviation (SD) values were reported for each of the outcomes at baseline. Multivariate linear mixed models were used to assess the effect of the PWSD on adolescent reported pain and mental health, adjusting for time since recruitment, the age of the adolescent at recruitment and ability to walk at recruitment. Rates of change in hip,

knee and ankle angles in the preferred standing position as calculated from 2D videographs were estimated using fixed effects linear regression model, automatically adjusting for age when receiving the chair. Data analyses were undertaken using Stata version 14.2 (StataCorp, College Station, TX).

RESULTS

Participants

At recruitment, the mean (SD) age of the 14 adolescents was 11.9 (2.3) years, EK score was 5.3 (2.7) and BMI was 24.7 (2.6). At recruitment, 9 adolescents were in the late-ambulatory and 5 were in the early non-ambulant stages. All individuals underwent regular physiotherapy assessments locally. Some also used night splints (n=11), hydrotherapy (n=7), home-based stretching regimens (n=4), a formal standing and exercise session three times weekly (n=1) or a post-fracture rehabilitation program (n=1). No child wore ankle foot orthoses during the day at recruitment. Three adolescents had been diagnosed with a neurodevelopmental disorder (attention deficit and/or hyperactivity disorder, autism spectrum disorder, intellectual disability) and each of these individuals were able to participate in conversations using full sentences. Other descriptive characteristics are presented in Table 1.

Adolescent and parent reported variables

Data describing muscle and joint pain were collected from eight adolescents (79 data points) and all individuals experienced some muscle and joint pain at baseline (Table 1). Average muscle pain over the duration of data collection was not independently associated with time since recruitment (months), ability to walk at baseline (yes/no) or receiving the PWSD (yes/no). Adjusting for covariates, there was a very small reduction in muscle pain with increasing age at recruitment in years (coefficient -0.4, 95% CI -0.7, -0.2) (Table 2). Average joint pain was not independently associated with time since recruitment, age at baseline or receiving the PWSD. Being able to walk at baseline was associated with a small and nonsignificant reduction in joint pain over the study period (coefficient -1.7, 95% CI -2.4, 0.9) after adjusting for the covariates (Table 2).

The mean (SD) SDQ total score at baseline was 13.5 (4.3). There were no significant associations between the duration of time since recruitment, age at baseline or the ability to walk at baseline and total SDQ or subscales scores. However, receiving the chair was independently associated with a lower total (coefficient -3.1, 95%CI -4.6, -1.5), hyperactivity subscale (coefficient -1.1, 95%CI -2.0, -0.1) and emotion subscale (coefficient -1.0, 95%CI -1.9, -0.3) scores reflecting better mental health; but not in the peer or conduct subscales (Table 3).

The mean (SD) PARS III total score was 82.5 (16.2). Adjusting for the effects of time since recruitment, age at baseline and ability to walk at baseline, receiving the PWSD was

independently associated with almost a nine-point increase in total score (coefficient 7.9, 95%CI 3.3, 12.5), and improved anxiety/depression (coefficient 4.3, 95%CI 1.3, 7.2), peer relations (coefficient 2.4, 95%CI 1.0, 3.9) and dependency (coefficient 1.7, 95%CI 0.7, 2.6) scores, reflecting better mental health; but not in the hostility, withdrawal or productivity subscales (Table 4).

Video assessment

Two sit-to-stand videos were available for eleven adolescents. The first video was filmed after a mean (SD) of 2.6 (2.5) months; the interval between videos was 8.0 (5.0) months. Standing hip angle showed a small increase towards extension between measurements adjusting for the age of the adolescent (coefficient 2.72, 95% CI -7.08, 12.52), equating to an average increase of three degrees in one year. There was a slight decrease in standing knee extension angles (coefficient -3.83, 95% CI -11.15, 3.48) equating to the boys preferring to stand with knees being four degrees more flexed on average in one year and little change in standing ankle plantarflexion (coefficient 0.53, 95% CI -4.20, 5.25) (Table 5).

DISCUSSION

This study examined various markers of musculoskeletal health in adolescents with DMD over approximately 20 weeks of PWSD use. All adolescents experienced musculoskeletal pain on a regular basis, consistent with literature in adolescents with DMD[6] but PWSD use did not appear to be associated with any aggravation or reduction in muscle and joint pain.

Joint angles estimated from video data allowed hip, knee and ankle angles in the preferred standing position to be assessed. The participants appeared to avoid progression of joint deformity when using the PWSD over the 8-month observation period, although we acknowledge that the video data did not directly assess the presence or absence of joint contracture which would have required direct assessment of full passive joint range. In standing, hips were more extended over time, consistent with slightly increased hip flexor length observed in a previous case series of PWSD users with DMD,[11] although these findings were not significant and may have reflected increased anterior pelvic tilt rather than true hip extension. This contrasts with increased hip contracture observed in one series of 34 adolescents during their first year of wheelchair use.[8] We did however observe knee flexion to be slightly more pronounced with time. Ankle angles were maintained, also consistent with previous observations following PWSD use.[11] A small raising of the heels in standing while in shoes may, however, not have been detected. Contractures at the hips, knees and ankles become more prevalent with greater reliance on wheelchairs in DMD[7-9] and can result in difficulty with wearing shoes. Maintenance of standing ankle angle is a positive finding in this study and evaluation over time is warranted to determine whether this persists over a longer period.

Mental health scores were mostly below the clinical threshold as determined using mental health screening tools.[17, 19] At a time when mental health often declines,[12, 13] adolescent-reported scores improved when using the PWSD and reflected more positive

mood, reduced hyperactivity and better peer relations. Scores on the parent-reported PARS III dependence subscale also decreased and provided some triangulation for the findings. Adolescents with DMD become more aware of their disease and typically experience increased anxiety and depression during adolescence as individuals attempt to meet the demands of their illness.[13, 21] Adjusting for relevant covariates, retained capacity to stand in the PWSD was associated with better mental health scores compared to baseline and was possibly explained by retained independence in daily life.[5] This includes opportunities to stand at school, an important setting for the establishment and maintenance of positive social relationships and friendships, and consequently, this may serve to promote mental health. Our findings are consistent with the notion of the disability paradox, that environmental supports can encourage a more positive mood and support social interactions despite severe disability.[22] Data also suggest the potential value in developing a program to accompany receiving a PWSD, that would suggest opportunities to practice skills that are protective of mental health and further enhance their sense of autonomy, competence and belonging at this vulnerable time.

We used a stepped wedge design trial[23] with variable baseline periods because a randomised clinical trial would not be feasible or ethical to evaluate the PWSD. Adolescent mental health data were reported by both the adolescents and their parents providing an opportunity to view this aspect of health from two perspectives. Our sample represented variation in the presentation of DMD including the presence of a neurodevelopmental

disorder supporting the generalisability of findings. However, we do acknowledge limitations. For example, the sample size was small; DMD is a rare disease, and recruitment was limited to individuals at a very specific point in their disease course. Mixed regression models were used in analysis to reduce between subject confounding and take full advantage of the large number of data points provided by our necessarily small number of participants. We videoed the preferred standing position; and it is possible that hip, knee and ankle angles may have been influenced by muscle length and/or choice. However, it might be expected that, had flexion contractures had worsened during the study, that the boys would not have been able to maintain their baseline standing posture. Whilst our method of analysing video data using Kinovea has similar reliability for measuring ankle angles as goniometry in cerebral palsy,[20] this should be evaluated also in DMD. As such we have some evidence to suggest that PWSD use protected against the development or worsening of contractures. It would be of value to compare data with a similar sample who do not receive a PWSD and to continue observations over a longer period of time to observe whether joint angles and mental health status are maintained.

PWSD use in our sample appeared to be associated with advantages over the short term to both physical and mental health, setting the scene for further longitudinal evaluation of PWSD use. These data contribute to the evidence base to inform clinical and therapy discussions as to how to support the child and family's personal goals when the disease is progressing.

What is already known on this topic

- There is risk of increased joint contracture following loss of ambulation in Duchenne muscular dystrophy.
- Loss of ambulation is associated with greater dependency on caregivers when their peers without Duchenne muscular dystrophy are gaining independence.
- Powered Wheelchair Standing Devices (PWSD) have electronic control within the powered wheelchair structure and allow for independence in choosing when to stand.

What this paper adds

- Functional joint angles for standing were preserved over the first 12 months of PWSD use.
- Opportunity to continue standing was associated with better mental health.
- Independence in supported standing when ambulation is deteriorating assisted both physical and mental health.

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Figure legend

Figure 1: Schematic diagram illustrating the variable baseline and intervention periods in this modified stepped wedge design study.

Table 1: Characteristics of the adolescents with Duchenne muscular dystrophy at baseline (n=14)

		n
Baseline description	Mobility level when received the PWSD	
	Walking around the house without assistance	5
	Walking around the house with help	2
	Walking a few steps with help	2
	Unable to walk	5
	Presence of pain over the previous two weeks	11
	Previous fracture	
	Never	6
	Once	4
	More than once	4
	Medications	
	Corticosteroids [†]	14
	Vitamin D supplement	11
	Calcium supplement	9
Outcome variables at baseline	Bisphosphonates	4
	Constipation-related	2
	Mean (SD)	
	Wong-Baker Faces pain rating scores (/10)	
	Muscle	3.5 (3.0)
	Joint	2.4 (2.6)
	Strengths and Difficulties questionnaire scores	
	Total (/40)	13.5 (4.3)
	Hyperactivity (/10)	5.6 (4.5)
	Emotion (/10)	3.4 (1.6)
	Peer (/10)	2.1 (1.9)
	Conduct (/10)	3.1 (1.6)
	PARS III questionnaire scores	
	Total (/112)	82.5 (16.2)
	Hostility (/24)	15.2 (7.1)
	Anxiety/depression (/24)	19.1 (3.4)
	Withdrawal (/16)	13.6 (3.2)
	Peer relations (/16)	11.2 (3.2)
	Dependency (/16)	10.8 (2.1)
	Productivity (/16)	8.4 (4.3)
	Preferred standing joint angle (degrees) at baseline	
	Hip extension (degrees)	165.0 (12.3)
	Knee extension (degrees)	157.0 (9.1)
	Ankle plantarflexion (degrees)	90.0 (4.2)

[†] 7 prescribed deflazacort, 7 prescribed prednisolone

Table 2: Multivariate model of associations between muscle and joint pain (n=8, 79 data points), and time, age and ability to walk at baseline, and receiving the Powered Wheelchair Standing Device (PWSD)

Dependent variables	Independent variables	Coefficient (95% CI [‡])	P value
Muscle pain	Has PWSD vs does not yet have PWSD	-0.47 (-2.07, 1.12)	0.563
	Time since recruitment (m)	0.01 [†] (-0.29, 0.26)	0.930
	Age at baseline (y)	-0.45 [†] (-0.73, -0.18)	0.001
	Able vs unable to walk at baseline	-0.57 (-2.08, 0.93)	0.455
Joint pain	Has PWSD vs does not yet have PWSD	-0.76 (-2.45, 0.93)	0.378
	Time since recruitment (m)	0.001 [†] (-0.29, 0.29)	0.9993
	Age at baseline (y)	-0.27 [†] (-0.62, 0.08)	0.137
	Able vs unable to walk at baseline	-1.67 (-3.63, -0.30)	0.096

[†] Coefficient is the change in the dependent variable per unit change of the continuous independent variables

[‡] CI – confidence interval

Table 3: Multivariate assessment of relationships between the Strengths and Difficulties total and subscale scores and age at recruitment, ability to walk at baseline, time since recruitment and receiving the Powered Wheelchair Standing Device (PWSD) (n=14, 137 data points).

	Total score		Hyperactivity		Emotion		Conduct		Peer	
	Coefficient (95% CI [‡])	P value	Coefficient (95% CI)	P value	Coefficient (95% CI)	P value	Coefficient (95% CI)	P value	Coefficient (95% CI)	P value
Has PWSD vs does not yet have PWSD	-3.05 (-4.60, -1.51)	<0.001	-1.04 (-2.02, -0.06)	0.037	-1.11 (-1.91, -0.31)	0.007	-0.56 (-1.20, 0.07)	0.084	-0.58 (-1.28, 0.13)	0.108
Time since recruitment (m)	0.062 [†] (-0.22, 0.34)	0.659	-0.04 [†] (-0.21, 0.14)	0.673	0.12 [†] (-0.02, 0.27)	0.089	-0.02 [†] (-0.13, 0.10)	0.759	-0.01 [†] (-0.12, 0.12)	0.988
Age at baseline (y)	-0.043 [†] (-0.82, 1.91)	0.922	-0.18 [†] (-0.63, 0.27)	0.439	-0.07 [†] (-0.36, 0.27)	0.646	0.13 [†] (-0.09, 0.34)	0.244	0.12 [†] (-0.22, 0.46)	0.480
Able vs unable to walk at baseline	-2.71 (-6.97, 1.54)	0.211	-1.06 (-3.28, 1.15)	0.346	-0.26 (-1.69, 1.17)	0.722	-0.60 (-1.63, 0.44)	0.260	-0.68 (-2.34, 0.98)	0.420

[†] Coefficient is the change in the dependent variable per unit change of the continuous independent variables

[‡] CI – confidence interval

Table 4: Multivariate assessment of relationships between PARS III total and subscale scores and age at recruitment, ability to walk at baseline, time since recruitment and receiving the Powered Wheelchair Standing Device (PWSD) (n=8, 80 data points).

	Total score		Hostility		Anxiety/depression		Withdrawal		Peer relations		Dependency		Productivity	
	Coefficient (95% CI [†])	P value	Coefficient (95% CI)	P value	Coefficient (95% CI)	P value	Coefficient (95% CI)	P value	Coefficient (95% CI)	P value	Coefficient (95% CI)	P value	Coefficient (95% CI)	P value
Has PWSD vs does not have PWSD	7.93 (3.33, 12.54)	0.001	1.39 (-1.45, 4.24)	0.330	4.26 (1.30, 7.22)	0.005	0.17 (-0.82, 1.16)	0.738	2.41 (0.96, 3.86)	<0.001	1.65 (0.74, 2.56)	<0.001	0.98 (-0.81, 2.76)	0.284
Time since recruitment (m)	-1.38 [†] (-2.19, - 0.56)	0.001	-0.16 [†] (-0.65, 0.34)	0.533	-0.69 [†] (-1.19, - 0.18)	0.008	-0.01 [†] (-0.16, 0.18)	0.913	-0.53 [†] (-0.79, - 0.28)	<0.001	-0.26 [†] (-0.42, - 0.09)	0.002	-0.09 [†] (-0.40, 0.98)	0.581
Age at baseline (y)	0.73 [†] (-2.76, 4.22)	0.682	-0.18 [†] (-0.79, 0.43)	0.570	0.29 [†] (-0.18, 0.77)	0.226	-0.02 [†] (-0.57, 0.53)	0.648	-0.02 [†] (-0.93, 0.89)	0.965	0.13 [†] (-0.38, 0.64)	0.610	0.29 [†] (-0.41, 0.98)	0.418

Able vs unable to walk at baseline	-2.53 (-17.65, 22.71)	0.806	-1.39 (-4.82, 2.04)	0.427	1.87 (-0.72, 4.46)	0.158	-0.03 (-3.21, 3.16)	0.983	0.09 (-5.16, 5.34)	0.974	-1.08 (-4.03, 1.86)	0.470	0.80 (-3.18, 4.79)	0.692
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[†] Coefficient is the change in the dependent variable per unit change of the continuous independent variables; [‡] CI – confidence interval

Table 5: Rate of change in joint angles in one year in the preferred standing position adjusting for age (n=11).

	Rate of change (degrees/year)	95% CI [‡]	p-value
Hip extension	2.72 [†]	(-7.08,12.52)	0.550
Knee flexion	-3.83 [†]	(-11.15,3.48)	0.270
Ankle plantarflexion	0.53 [†]	(-4.20,5.25)	0.809

[†] Coefficient is the change in the dependent variable per unit change of the continuous independent variables

[‡] CI – confidence interval

TITLE: Benefits of powered standing wheelchair devices for adolescents with Duchenne muscular dystrophy in the first year of use

TYPE OF ARTICLE: Original article

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