

Home-Based Versus In-Clinic Functional Assessments: Preferences of Caregivers and Patients with Duchenne

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Background

- People with Duchenne muscular dystrophy (DMD) adopt compensatory movement patterns to maintain independence as their muscles get weaker. These compensatory movements are hallmarks of weakening muscles and may represent earlier, more sensitive opportunities to detect disease progression.
- The Duchenne Video Assessment (DVA) quantifies these compensations, providing a standardized way to assess quality of movement as an indication of disease severity.
- Caregivers video record patients with DMD performing specific movement tasks at home using a secure mobile application. The movement tasks included in the DVA are daily tasks that have an impact on patient independence, such as walking and eating.
- Trained physical therapists rate the videos using scorecards with prespecified compensatory movement criteria.

Objective

To investigate caregiver and patient preferences around completing functional assessments at home using the DVA or in clinic using other measures.

Methods

Study Design: Longitudinal study of male participants with DMD aged ≥4 with Brooke score <6

Study Subpopulations:
 Ages 4-6 Young Ambulatory
 Age 7+ Early Ambulatory
 Age 7+ Late Ambulatory
 Age 7+ Early Non-Ambulatory
 Age 7+ Late Non-Ambulatory

DEFINITIONS

Early ambulatory: ambulatory and able to rise from the floor within 30 seconds without assistance or use of furniture, as reported by the caregiver

Late ambulatory: ambulatory and unable to rise from the floor within 30 seconds without assistance or use of furniture, as reported by the caregiver

Early non-ambulatory*: non-ambulatory and Brooke score <4 (can still raise hand to mouth), as reported by the caregiver

Late non-ambulatory*: Brooke score 5 (cannot raise hand to mouth but can use hands to hold a pen, pick up pennies, or drive a powerchair), as reported by the caregiver

*Non-ambulatory is defined as needing a wheelchair to move approximately 10 meters (e.g. from the living room to the bathroom), as reported by the caregiver

Data Collection:

- Caregivers collected video data of participants performing DVA movement tasks at 2 or 3 timepoints during the study depending on subgroup.
- Caregivers completed a questionnaire at the end of the study to rate the emotional difficulty for both the caregiver and the patient of completing the DVA at home and other assessments in the clinic. They used a 5-point Likert scale to rate emotional difficulty (very difficult to very easy). In addition, caregivers indicated both caregiver and patient preferences around completing DVA at home or traveling to a clinic for evaluation.

Data Analyses:

- Emotional difficulty with assessments was compared using the McNemar exact test using a binary variable: very difficult / difficult versus neutral / easy / very easy.
- Qualitative responses were analyzed for themes.

Results

Table 1. Characteristics of participants with DMD

Characteristic	Participants N=48 n (%)
Age at baseline	
4-9	15 (31)
10-14	20 (42)
15-19	9 (19)
20-24	3 (6)
25-29	1 (2)
Functional subgroup	
Young Ambulatory	11 (23)
Early Ambulatory	9 (19)
Late Ambulatory	8 (16)
Early Non-Ambulatory	10 (21)
Late Non-Ambulatory	10 (21)

Table 3. Emotional difficulty of functional assessments for caregivers and patients, as reported by the caregiver

Assessments	Caregivers n (%)	p-value ¹	Patients n (%)	p-value ¹
Duchenne Video Assessment	N=48	Ref.	N=48	Ref.
Very difficult	3 (6)		3 (6)	
Difficult	12 (25)		11 (23)	
Neutral	14 (29)		15 (31)	
Easy	13 (27)		15 (31)	
Very Easy	6 (13)		4 (8)	
North Star Ambulatory Assessment	N=32	0.007	N=33	0.344
Very difficult	7 (22)		5 (15)	
Difficult	12 (38)		8 (24)	
Neutral	6 (19)		10 (31)	
Easy	4 (12)		7 (21)	
Very Easy	3 (9)		3 (9)	
Performance of Upper Limb test	N=39	0.424	N=41	1.00
Very difficult	4 (10)		3 (7)	
Difficult	10 (26)		8 (20)	
Neutral	10 (26)		12 (29)	
Easy	10 (26)		12 (29)	
Very Easy	5 (12)		6 (15)	
4-Stair Climb	N=29	0.003	N=30	0.004
Very difficult	6 (21)		7 (23)	
Difficult	13 (45)		9 (30)	
Neutral	5 (17)		8 (27)	
Easy	2 (7)		3 (10)	
Very Easy	3 (10)		3 (10)	
6-Minute Walk test	N=26	0.125	N=28	0.219
Very difficult	7 (27)		8 (28)	
Difficult	6 (23)		3 (11)	
Neutral	8 (31)		10 (36)	
Easy	3 (11)		4 (14)	
Very Easy	2 (8)		3 (11)	

¹McNemar exact test comparing each functional assessment to the Duchenne Video Assessment

Table 2. Preferences around home-based video assessments versus travel to clinical sites, as reported by caregivers

Prefer	Caregivers N=48 n (%)	Patients N=48 n (%)
Home-based video assessments	26 (54)	38 (79)
Travel to clinical site	14 (29)	6 (13)
No preference	8 (17)	4 (8)

Caregiver Representative Quotes

Prefers Home-Based Video Assessment

"The stress of [patient] being compliant the one day we are in clinic is very hard on me and him." (Caregiver of 5-year-old patient)

"It becomes tiring for him to travel and he misses school for it, so we would prefer to do home-based video assessments if possible." (Caregiver of 12-year-old patient)

"While it is emotionally difficult to do the assessment ourselves, it is more difficult to travel with a non-ambulant child. The airplanes are not accessible, and they get anxiety away from home." (Caregiver of 12-year-old patient)

"Travel is really difficult for these guys. It's painful and hard on the parents logistically. Home is way more comfortable and they are in their regular environment. I think you get a better view of the reality." (Caregiver of 17-year-old patient)

Prefers Travel to Clinical Site

"Nice to separate clinic from home, recognizing my boys are still ambulatory." (Caregiver of 4-year-old patient)

"I would prefer to travel to a clinical site just so I know the assessment is being done completely correct. My child would rather do the home base because he would be in the comfort of his home." (Caregiver of 5-year-old patient)

"I would prefer an expert in doing assessments perform the task, but he does like the natural setting." (Caregiver of 10-year-old patient)

"As a parent, I like to have him seen with another set of eyes periodically, especially if those eyes belong to a qualified professional." (Caregiver of 17-year-old patient)

Key Findings

- Most patients (79%) and more than half (54%) of caregivers preferred home-based video assessments to traveling to a clinical site.
- More caregivers reported experiencing emotional difficulty with the North Star Ambulatory Assessment (NSAA) than the DVA (60% vs. 31%; p=0.007).
- More caregivers reported experiencing emotional difficulty with the 4-stair climb test than the DVA (66% vs. 31%; p=0.003).
- More caregivers reported that patients experience emotional difficulty with the 4-stair climb test than the DVA (53% vs. 29%; p=0.004).
- The caregiver emotional difficulty reported for the Performance of Upper Limb (PUL) and 6-minute walk tests were not statistically significantly different from the DVA. The patient emotional difficulty reported for the NSAA, PUL, and 6-minute walk tests were not statistically significantly different from the DVA.

Conclusion

Most patients and more than half of caregivers prefer home-based video assessments over travel to a clinical site for evaluation. While conducting the DVA at home is emotionally difficult for some patients and caregivers, more reported emotional difficulty with assessments they currently travel to clinic to complete.