REAL-DMD: Caregiver Baseline Characteristics From an Electronic Survey of Long-Term Real-World Experiences of Patients With Duchenne Muscular Dystrophy (DMD)

Ivana F. Audhya[1], Shivangi Patel[1]*, Min Yang[2], Edward Tuttle[3], Bruno Martins[2], Fan Yang[4], Jessie Lan[2], Katherine L. Gooch[1]

[1]Sarepta Therapeutics, Inc., Cambridge, MA, USA; [2]Analysis Group, Inc., Boston, MA, USA; [3]Analysis Group, Inc., Menlo Park, CA, USA; [4]Analysis Group, Inc., London, UK

*Employee of Sarepta Therapeutics, Inc., at the time of analysis

Background

- Duchenne muscular dystrophy (DMD) is a rare, X-linked, monogenic, neuromuscular disease characterized by progressive loss of skeletal muscle, pulmonary function, and cardiac function, with evidence of muscle damage and myopathy observable from birth[1-3]
- Over time, lower extremity functioning becomes increasingly strenuous, leading to loss of independent ambulation; serious orthopedic, respiratory, and cardiac complications; and ultimately, premature mortality[4-5]
- In addition to the impact of DMD on patients, caregiving activities pose significant challenges to caregivers' physical and psychological well-being, including affected sleep quality, family function, ability to work, and productivity, as well as increased levels of depression, pain, and stress.[6] Moreover, caring for older patients has been associated with higher burden, suggesting that the impact of DMD on caregivers increases with disease progression[7]
- REAL-DMD is a real-world, observational, prospective, longitudinal cohort study in the US that surveys caregivers of DMD patients, focusing on patients' function and their caregiving experiences[8]
- Previously presented data from the REAL-DMD study on ambulatory patient experience and function showed moderate to severe impairments in mobility and upper extremity function, which were more prevalent in children ≥13 years old[8]

Objective

To generate real-world data on the experiences of caregivers of ambulatory DMD patients by describing the baseline characteristics of REAL-DMD participants

Methods

Study population

- Caregivers were recruited through 2 patient advocacy groups (Parent Project Muscular Dystrophy and The Akari Foundation)

Inclusion criteria

- Aged ≥18 years at the time of the study enrollment
- Resident in the US and able to read English or Spanish
- Primary caregiver (not for hire) for at least 6 months of a patient:
  - With a caregiver-reported diagnosis of DMD
  - Ambulatory at the time of the baseline survey
- Consent to participate

Exclusion criteria

- Professional paid caregiver
- The patient is currently enrolled in a DMD clinical trial or treated with PF-06939926 (fordadistrogene movaparvovec) or SGT-001

Data collection

- Data were initially collected through a web-based survey and will be collected every 6 months for a minimum of 5 years
- Caregivers were given the option to provide the experience of up to 2 ambulatory patients with DMD

Outcomes

Caregivers reported on their socio-demographics, the characteristics of the ambulatory patients they care for, and their caregiving experience, including:

- Average number of hours/day providing care for the ambulatory DMD patient over the past 7 days
- Financial support/assistance provided for the care of the ambulatory DMD patient
- Changes in employment status and productivity due to caregiving
- Health-related quality of life (HRQoL):
  - Patient Reported Outcomes Measurement Information System (PROMIS®) Scale v1.2 Global Health – Adult[9]:
    - Contains 10 items assessing physical, mental, and social health
    - Scores are assigned to 2 domains: Physical Health (physical health, physical function, pain, and fatigue items) and Mental Health (quality of life, mental health, satisfaction with discretionary social activities, and emotional problem items)
    - Raw summed scores are translated into a T-score for each respondent. A higher T-score represents better health

Statistical analysis

- PROMIS Global Health was scored using item-level calibrations via the HealthMeasures Scoring Service
- Continuous variables were summarized as mean, SD, and median, whereas categorical variables were summarized as frequency count and percentage

Results

Caregivers' characteristics

- Overall, 123 caregivers completed the baseline survey and provided the experience of 137 ambulatory patients
- The mean age (SD) of caregivers was 40.6 (7.3) years; most were female (91.9%) and white (86.2%) (**Table 1**)
- Most caregivers cared for 1 child with DMD (1 child: 82.1%; 2+ children: 17.9%) (**Table 1**)
- 57.7% of caregivers reported having anxiety or depression, with 35.8% undergoing treatment at the time of baseline (**Table 1**)

Table 1 *Baseline Caregivers' Demographics*

	Caregivers (N=123)
Age at enrollment, years	
Mean ± SD [median]	40.6 ± 7.3 [40.6]
Sex at birth, n (%)	
Female	113 (91.9)
Male	10 (8.1)
Race,[a] n (%)	
White	106 (86.2)
Asian	9 (7.3)
Other	13 (10.6)
Ethnicity,[a] n (%)	
Not Hispanic/Latino	102 (82.9)
Hispanic/Latino or Spanish origin	17 (13.8)
Prefer not to say	4 (3.3)
Geographic region, n (%)	
Southwest	45 (36.6)
Midwest	39 (31.7)
Northeast	20 (16.3)
West	19 (15.4)
Number of children with DMD, n (%)	
1	101 (82.1)
2	20 (16.3)
3	2 (1.6)
Marital status, n (%)	
Married or in a domestic partnership	97 (78.9)
Single, never married	11 (8.9)
Divorced	10 (8.1)
Separated	5 (4.1)
Comorbidities,[b] n (%)	
Anxiety and/or depression	71 (57.7)
Anxiety	62 (50.4)
Depression	49 (39.8)
Seasonal allergies	39 (31.7)
Overweight/obesity	37 (30.1)
High cholesterol	19 (15.4)
Hypertension	19 (15.4)
Insomnia/sleep disorder	17 (13.8)
Asthma	13 (10.6)
Cardiovascular diseases	11 (8.9)
Comorbidities under treatment,[c,d] n (%)	
Anxiety and/or depression	44 (35.8)
Anxiety	37 (30.1)
Depression	31 (25.2)
Seasonal allergies	18 (14.6)
Hypertension	15 (12.2)
Overweight/obesity	9 (7.3)
Cardiovascular diseases	8 (6.5)
High cholesterol	8 (6.5)
Insomnia/sleep disorder	8 (6.5)

[a]Categories not mutually exclusive. [b]Only comorbidities reported by at least 9% of respondents are shown. [c]Only those reported by at least 5% of respondents are shown. [d]Treatment is relevant to the condition listed.
DMD=Duchenne muscular dystrophy.

- Mean age of care recipients was 9.6 (4.7) years and time since DMD diagnosis was 5.7 (4.7) years (**Table 2**)
- All care recipients were either fully ambulatory or used a wheelchair/scooter part time (**Table 2**)
- A majority (87.6%) of the ambulatory patients had their experience provided by their biological mothers (**Table 2**)

Table 2 *Baseline Demographics of Care Recipients*

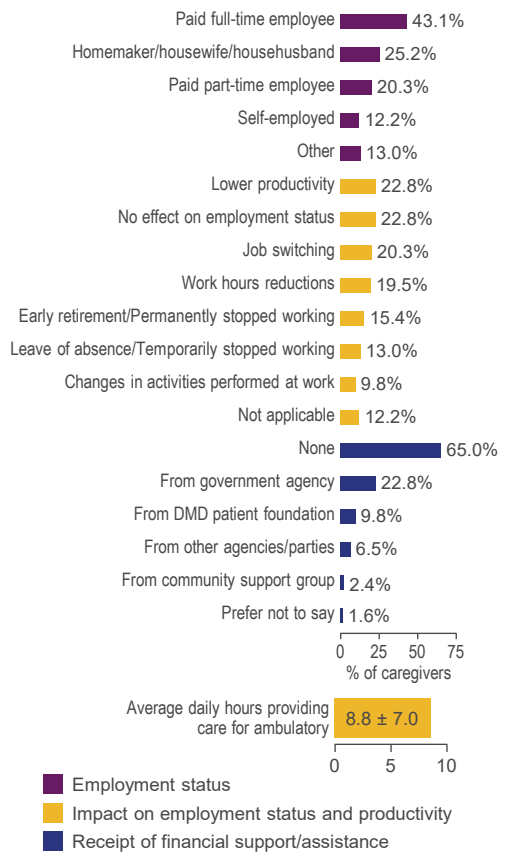
	Patients (N=137)
Age at enrollment, years	
Mean ± SD [median]	9.6 ± 4.7 [9.0]
Age groups, n (%)	
≤7 years	50 (36.5)
8–12 years	50 (36.5)
≥13 years	37 (27.0)
Age at DMD diagnosis, years	
Mean ± SD [median]	3.9 ± 2.4 [3.8]
Time since DMD diagnosis, years	
Mean ± SD [median]	5.7 ± 4.7 [4.7]
Ambulatory status	
Walks all day	90 (65.7)
Uses wheelchair or scooter part time	47 (34.3)
Continuously uses a wheelchair outside of home	0
Caregiver relationship,[a] n (%)	
Biological mother	120 (87.6)
Biological father	8 (5.8)
Other	9 (6.6)

[a]Response options in "Other" include stepfather (n=2), grandparent (n=2), stepmother (n=1), sibling (n=1), nonfamily legal guardian (n=1), and nonspecified nonfamily member (n=2).
DMD=Duchenne muscular dystrophy.

Caregiver experience

- Many caregivers were employed full (43.1%) or part time (20.3%) (**Figure 1**)
- 65.0% of caregivers reported experiencing impact on their ability to work due to caregiving responsibilities
- In particular, 22.8% of caregivers reported lower productivity, 20.3% reported switching jobs, and 19.5% reported reducing hours. 28.5% temporarily stopped working, went on leave, or permanently left the workforce since taking on a provider role (**Figure 1**)
- Approximately one third of caregivers (35%) received financial support or assistance for caring for the patient (**Figure 1**)
- Caregivers reported spending 8.8 (7.0) hours daily caring for the ambulatory patients (**Figure 1**)

Figure 1 *Impact of Caregiving on Caregivers of Ambulatory Patients With DMD*[a]



[a]Categories in employment status, impact on employment status and productivity, and financial support/assistance were not mutually exclusive.
DMD=Duchenne muscular dystrophy.

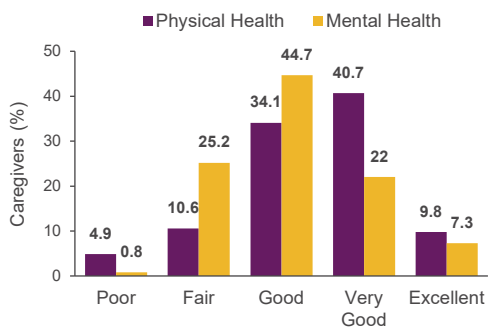
- The PROMIS Global Health – Physical T-score was 49.1 (7.9), indicating a study population with physical health comparable to the US general population (T-score of 50 [SD: 10]) (**Table 3**)
- The PROMIS Global Health – Mental T-score was 44.7 (8.3), which was one-half SDs lower than the US general population (T-score of 50 [SD: 10]) (**Table 3**)
- Less than one third of caregivers (29.3%) had very good/excellent mental health (**Figure 2**)

Table 3 *PROMIS Global Health Scores*

	Caregivers (N=123)
Physical Health	
Mean raw score	
Mean ± SD [median]	3.4 ± 0.5 [3.3]
T-score	
Mean ± SD [median]	49.1 ± 7.9 [50.4]
Mental Health	
Mean raw score	
Mean ± SD [median]	3.1 ± 0.8 [3.0]
T-score	
Mean ± SD [median]	44.7 ± 8.3 [43.3]

PROMIS=Patient-Reported Outcomes Measurement Information System.

Figure 2 *PROMIS Global Health Scores Distribution*[a]



[a]Categories used are based on the following PROMIS T-scores: **Physical Health:** T-score <35: Poor; 35 ≤ T-score < 42: Fair; 42 ≤ T-score < 50: Good; 50 < T-score ≤ 58: Very Good; 58 ≤ T-score: Excellent. **Mental Health:** T-score <29: Poor; 29 ≤ T-score < 40: Fair; 40 ≤ T-score < 48: Good; 48 < T-score ≤ 56: Very Good; 56 ≤ T-score: Excellent.
PROMIS=Patient-Reported Outcomes Measurement Information System.

Key Finding

The physical health of caregivers of patients with DMD was comparable to that of the general US population, while mental health was slightly lower

Conclusions

REAL-DMD baseline data provide insights into the real-world characteristics and experiences of caregivers of ambulatory DMD patients

Findings related to work productivity showed that almost two thirds of caregivers reported impacts on their ability to work due to caregiving responsibilities.
In addition, one third of caregivers reported receiving financial assistance for their ambulatory DMD patients.
Together, these results indicate the potential for a large economic burden among DMD caregivers

Caregivers' PROMIS mental health scores were one-half standard deviations lower than the general US population, suggesting that these caregivers may be experiencing higher levels of stress, anxiety, or depression. This observation highlights the potential emotional and psychological impact associated with caregiving for children with chronic and progressive conditions such as DMD

Longitudinal follow-up data from this study will depict the caregiver experience over time to improve the current understanding of the impact of caregiving for DMD patients

Disclosures & Funding

Disclosures: **IFA** and **KLG** are employees of Sarepta Therapeutics, Inc., and may own stock/options in the company. **SP** was an employee of Sarepta Therapeutics, Inc., at the time of analysis and may have owned stock/options in the company. **MY, ET, BM, FY**, and **JL** are employees of Analysis Group, Inc., which received funding from Sarepta Therapeutics, Inc., to support this research.

Funding: This work was supported by Sarepta Therapeutics, Inc.

References

1. Bushby K, et al. *Lancet Neurol.* 2010;9(1):77-93.
2. Evans MI, et al. *Fetal Diagn Ther.* 1995;10(2):71-5.
3. Min Y-L, et al. *Annu Rev Med.* 2019;70:239-55.
4. Duan D, et al. *Nat Rev Dis Primers.* 2021;7(1):13.
5. Mercuri E, et al. *Lancet.* 2019;394(10213):2025-38.
6. Landfeldt E, et al. *Dev Med Child Neurol.* 2018;60(10):987-96.
7. Schwartz CE, et al. *J Patient Rep Outcomes.* 2021;5(1):124.
8. Patel S, et al. *Value Health.* 2024;27(6): S304.
9. Hays RD, et al. *Qual Life Res.* 2009;18(7):873-80.

SCAN THE QR CODE

The QR code is intended to provide scientific information for individual reference, and the information should not be altered or reproduced in any way.

Presented at ISPOR Europe
17–20 November 2024
Barcelona, Spain