

The impact of Duchenne muscular dystrophy on caregiver employment: a survey in Europe, Japan, and the United States

Objective



To understand the impact of patients with Duchenne muscular dystrophy (DMD) on caregiver’s employment.

Conclusions



The majority of DMD caregivers are not employed full- or part-time. Those that are employed had to take time off and experienced impairments while working because of caring for the patient.



Patients required multiple caregivers, including professional care. The results highlight the burden on DMD caregivers’ ability to work and potential economic impact of not working, and professional care.

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Acknowledgements:

We would like to thank the caregivers for taking part in this study and providing the information included.

Disclosures:

- JS is on the advisory board for Sarepta, speaker training for NS Pharma, was PI for Fibrogen pamrevlumab phase 2 trial and currently PI for PTC ataluren phase 3. JS is also a speaker bureau for Biogen (SMA), consultant for UCB and Janssen/Momenta (MG), and PI for Janssen and Roche (MG)
- KI Keiko Ishigaki received honoraria for speakers bureaus from Nippon Shinyaku Co., Ltd and has an advisory role at Pfizer Japan Inc.
- VM, NP, SX, AT and SB are employees of Pfizer
- JCC is an employee and stockholder of Pfizer
- EM, SO and JDC are employees of Adelphi Real World

References:

- Szabo SM, et al. Orphanet J Rare Dis. 2021; 16(237):
 - Landfeldt E, et al. Dev Med Child Neurol. 2018; 60(10):987-996.
 - Schwartz CE, Stark RB, Audhya IF, Gooch KL, J Patent Rep Outcomes. 2021; 5(1): 124
 - Anderson P, et al. Curr Med Res and Opin. 2008; 24(11):3063-3072
 - Babineaux SM, et al. BMJ Open. 2016; 6(8):e010352
 - Higgins V, et al. Diabetes Metab Syndr Obes. 2016; 9:371-380
 - Reilly MC, Zbrozek AS, Dukes E: The validity and reproducibility of a work productivity and activity impairment measure. PharmacoEconomics 1993; 4(5):353-365.
- Presented at ISPOR EU • Copenhagen, Denmark • 12/11–15/11, 2023 • Virtual

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Introduction

- Duchenne muscular dystrophy (DMD) is a genetic disorder associated with progressive muscle degeneration due to a lack of dystrophin¹.
- Most caregivers are a parent of the affected child.
- People with DMD often require extensive care, resulting in substantial economic and humanistic burden and impacting caregiver employment²⁻³.
- However, the degree to which DMD impacts caregiver employment is not well-defined.
- Here we describe the effect DMD has on caregiver employment across three geographical regions – Europe, Japan, and the United States (US).

Methods

- Data were drawn from the Adelphi Real World DMD Disease Specific Programme (DSP™), a cross-sectional survey of DMD caregivers conducted in Europe (France, Germany, Spain), Japan, and the US from October 2022 to May 2023.
- The data collection methodology used to create the DSP database has been previously published and validated⁴⁻⁶.
- Caregivers were asked to complete a survey, reporting data on patient demographics, employment status, and domains from the work productivity and activity impairment (WPAI) questionnaire⁷.

Methods (cont.)

- The WPAI measured impairment across four domains: absenteeism, presenteeism, overall work impairment and non-work activities.
- Analyses were descriptive.

Limitations

- In our study, the US cohort consists of a small sample size of caregivers and patients which may also not be truly representative of the overall population.
- Recall bias, a common limitation of surveys, might also have affected responses of caregivers.

Results

- In this study, 77 DMD caregivers were enrolled. Mean (standard deviation; SD) age of 48.5 (9.1) years, 80.5% were women, 93.5% were a parent of the patient, and 43.4% were employed (**Table 1**), of which 21.1% were in full-time employment.
- Patients had a mean (SD) age of 18.0 (6.3) years; 69.7% of cared-for patients were non-ambulatory (**Table 2**).
- Caregivers spent a mean (SD) of 43.4 (32.2) hours a week providing care (**Table 1**).
- Overall, 35.3% of caregivers who are employed made changes to their work due to caring (**Figure 1**). Of the caregivers who were still employed, a mean (SD) 2.3 (1.7) days were taken off work in the last three months to care for a patient.

Table 1. Caregiver demographics				
Characteristics	All caregivers (n=77)	Europe (n=48)	Japan (n=26)	US (n=3)
Age, mean (SD)	48.5 (9.1)	48.7 (9.4)	47.9 (9.2)	50.0 (7.0)
Female, n (%)	62 (80.5)	37 (77.1)	24 (92.3)	1 (33.3)
Parent of patient, n (%)	72 (93.5)	44 (91.7)	26 (100.0)	2 (66.7)
	n=76	n=47	n=26	n=3
Employed, n (%)	33 (43.4)	27 (57.4)	4 (15.4)	2 (66.7)
	n=72	n=44	n=25	n=3
Hours of DMD care per week, mean (SD)	43.4 (32.2)	41.6 (27.0)	47.7 (40.6)	33.3 (32.2)

Table 2. Patient demographics				
Characteristics	All patients (n=77)	Europe (n=48)	Japan (n=26)	US (n=3)
Age, mean (SD)	18.0 (6.3)	17.2 (6.6)	19.1 (6.1)	21.7 (2.5)
	n=58	n=31	n=24	n=3
Years since DMD diagnosis, mean (SD)	12.8 (6.8)	9.8 (6.2)	15.9 (6.1)	17.9 (4.6)
	n=76	n=47	n=26	n=3
Non-ambulatory, n (%)	53 (69.7)	28 (59.6)	23 (88.5)	2 (66.7)

SD, standard deviation; DMD, Duchenne muscular dystrophy; USA, United States

- Working hours were reduced by 23.5% of caregivers to care for the DMD patient (**Figure 2**).
- Most patients (97.4%) had additional caregivers, including the patient’s second parent (76.0%) and/or a professional caregiver (37.3%, **Figure 3**).
- Mean overall work impairment was 44.1% for caregivers of DMD patients, according to WPAI (**Figure 4**).
- Caregivers missed a mean (SD) of 6.5% (21.3) of contracted work over the previous seven days (absenteeism; **Figure 4**). Mean (SD) impairment while working (presenteeism) was 38.8% (21.7; **Figure 4**).
- Mean (SD) activity impairment was 54.1% (26.5; **Figure 4**).

Figure 4. WPAI scores for DMD caregivers across geographical regions

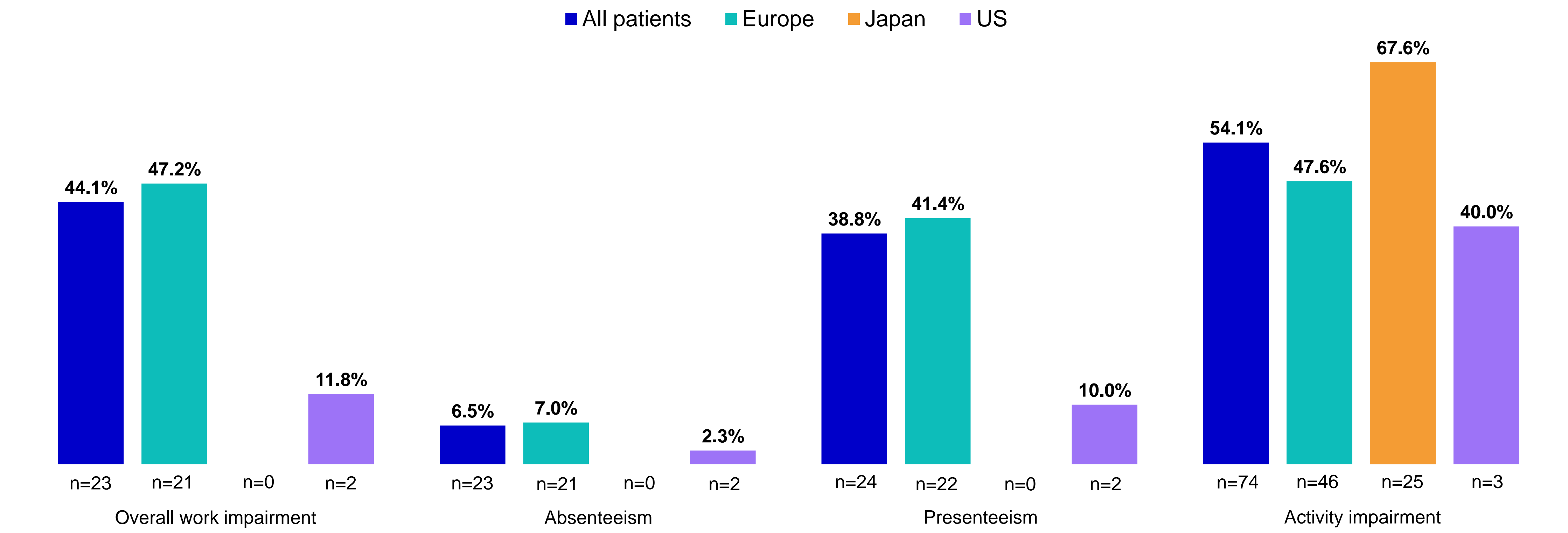


Figure 1. Proportion of caregivers with a change to their employment due to caring for the patient with DMD

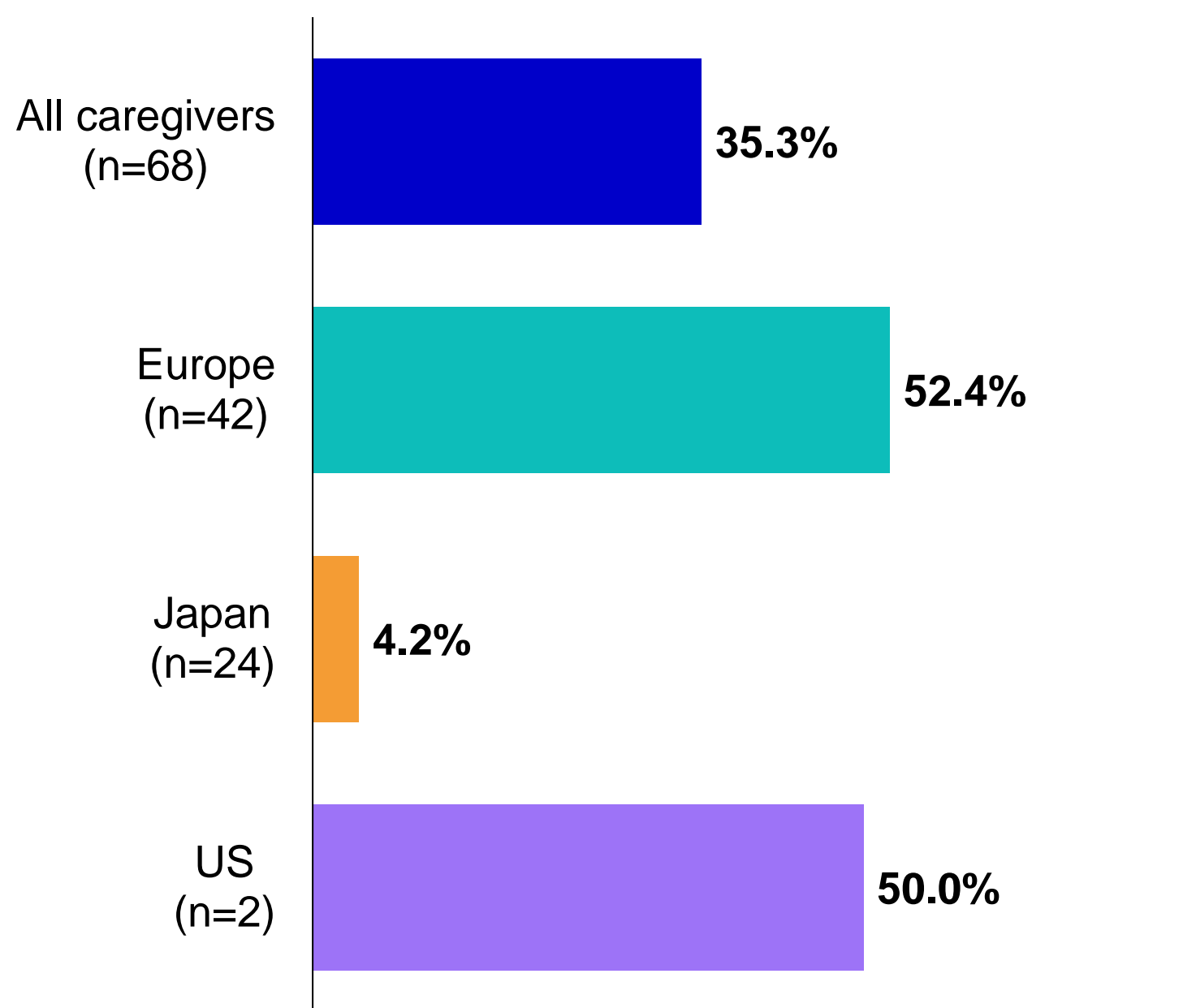


Figure 2. Proportion of caregivers reducing working hours

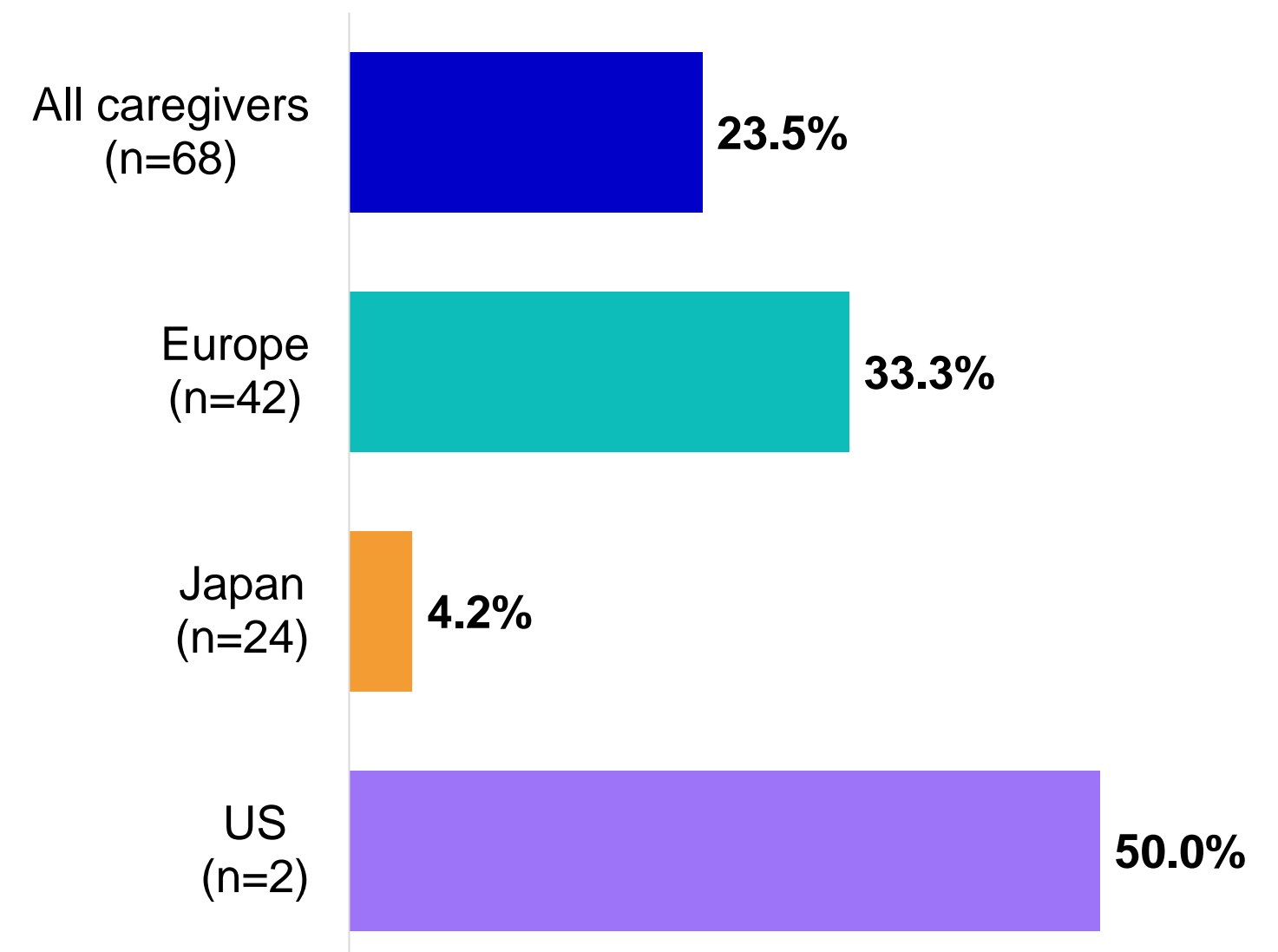
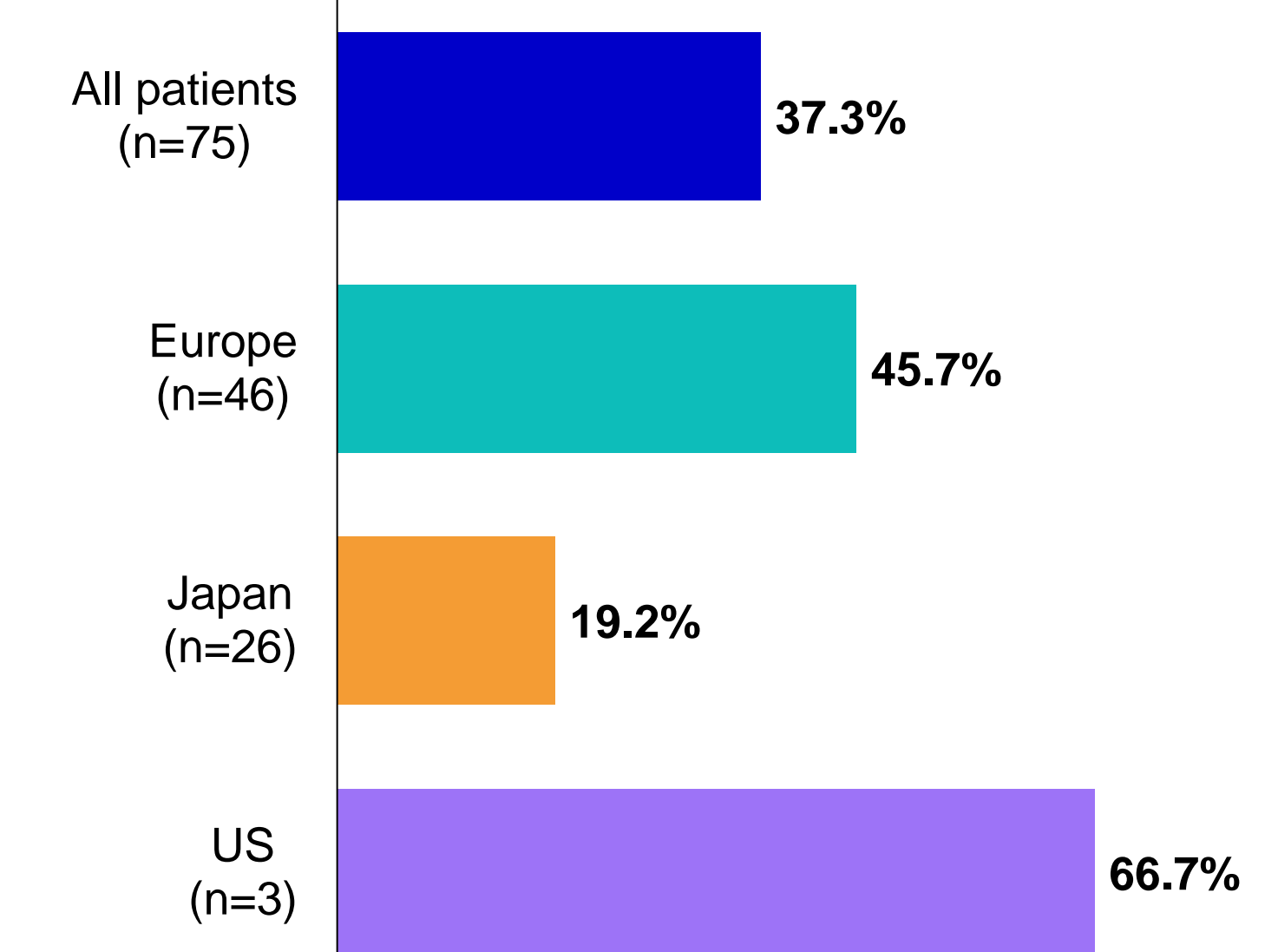


Figure 3. Proportion of patients with professional (paid) caregivers



DMD, Duchenne muscular dystrophy; USA, United States