Characterizing The Experience of Caregiving For Those With Duchenne Muscular Dystrophy: Results From a Cross-Sectional Survey

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INTRODUCTION

- Duchenne muscular dystrophy (DMD) is a rare, pediatric neuromuscular disease characterized by diminishing functional ability and loss of independent ambulation
- As DMD progresses, affected individuals rely more heavily on their caregivers[1-2]
- While caregiver impact in DMD has been previously described in international studies, [1,3-5] contemporary US-based analyses are lacking

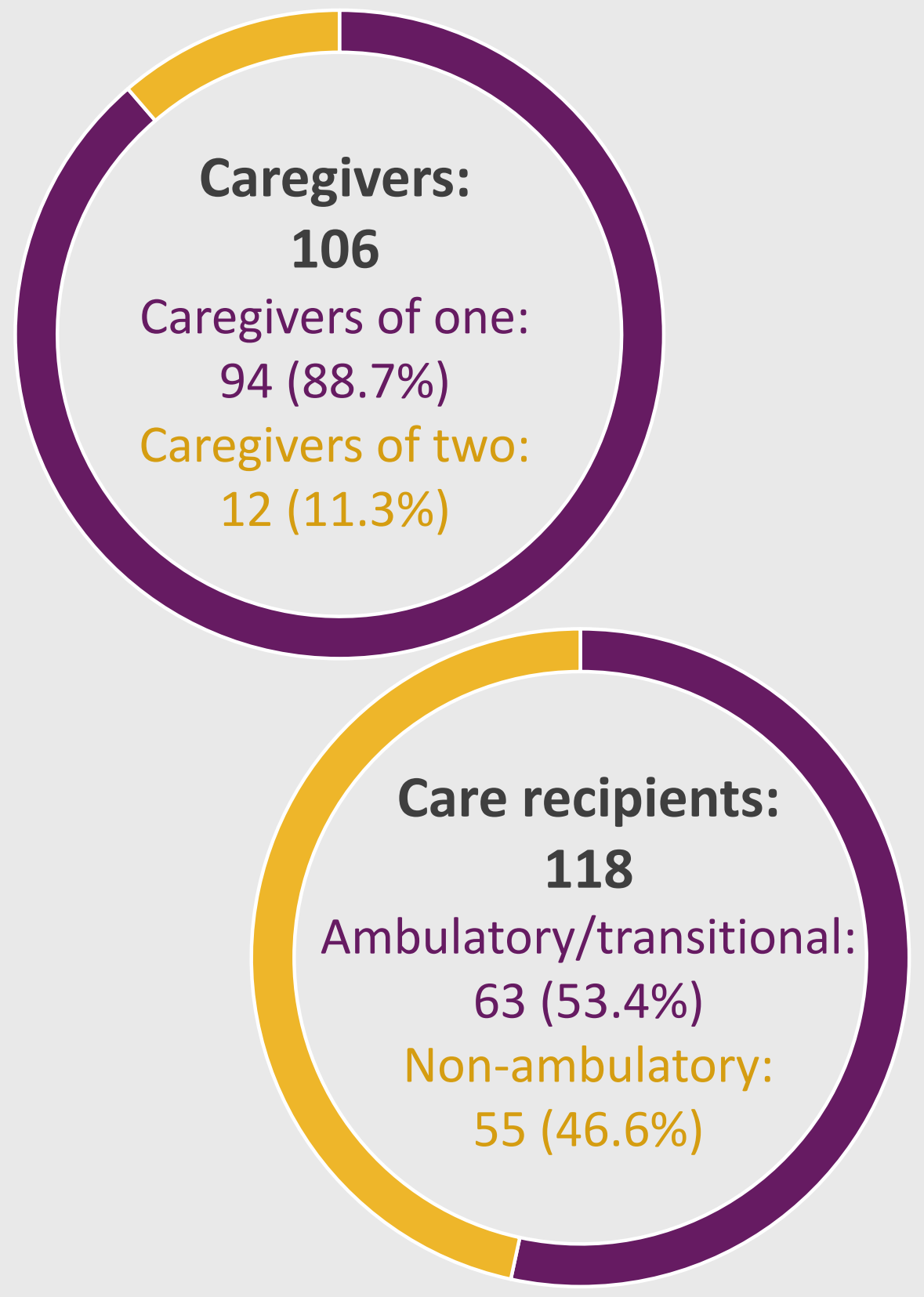
OBJECTIVES

- As part of a larger survey, the aim of this analysis was to:
  - Characterize the experiences of caregivers who care for individuals with DMD
  - Explore the differences in caregiver experiences between caregivers of one and two person(s) with DMD; and between caregivers of those who are non-ambulatory vs. ambulatory

METHODS

- Caregivers of individuals with DMD from a prior study living in the US were recruited to complete a web-based survey
  - In the prior study, caregivers were recruited through Parent Project Muscular Dystrophy, a US-based DMD patient advocacy group[6]
- Ethics approval was solicited and informed consent obtained prior to participation
- The survey was programmed online (Qualtrics Core XM software) and included both closed- and open-ended questions to capture caregivers' experience, including:
  - Caregiver burden, which was measured on a scale of 0 (providing care is not challenging) to 10 (providing care is very challenging)
  - Time spent caregiving i.e., time helping care recipient(s) with activities of daily living (ADLs) and healthcare needs
  - Caregivers' use of additional informal (unpaid) and formal (paid) care
  - The impact of accessibility barriers on family daily plans, (i.e., frequency of care recipients leaving their home, experiencing accessibility barriers outside of home, and needing to change daily family plans) using Likert scales – which ranged from daily/always to never
- Responses were summarized descriptively and stratified by care recipient ambulatory status and number of care recipients per caregiver

RESULTS

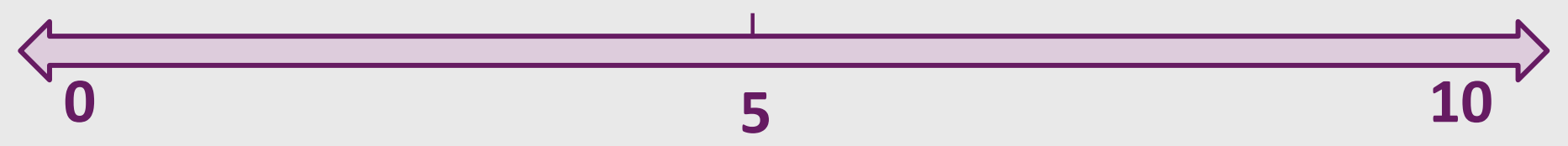


Sample characteristics

- One hundred and six caregivers responded to the survey, representing 118 care recipients
- Sixty-seven percent of caregivers were the primary caregivers, and 32.1% provided an equal amount of care as another person
- Mean caregiver age was 46 years; 82.1% were mothers of the care recipients
- Mean age of care recipients was 14.5 years; 27.1% were non-ambulatory and on ventilation

Caregiver burden

- Median caregiver burden in the past week was 5/10

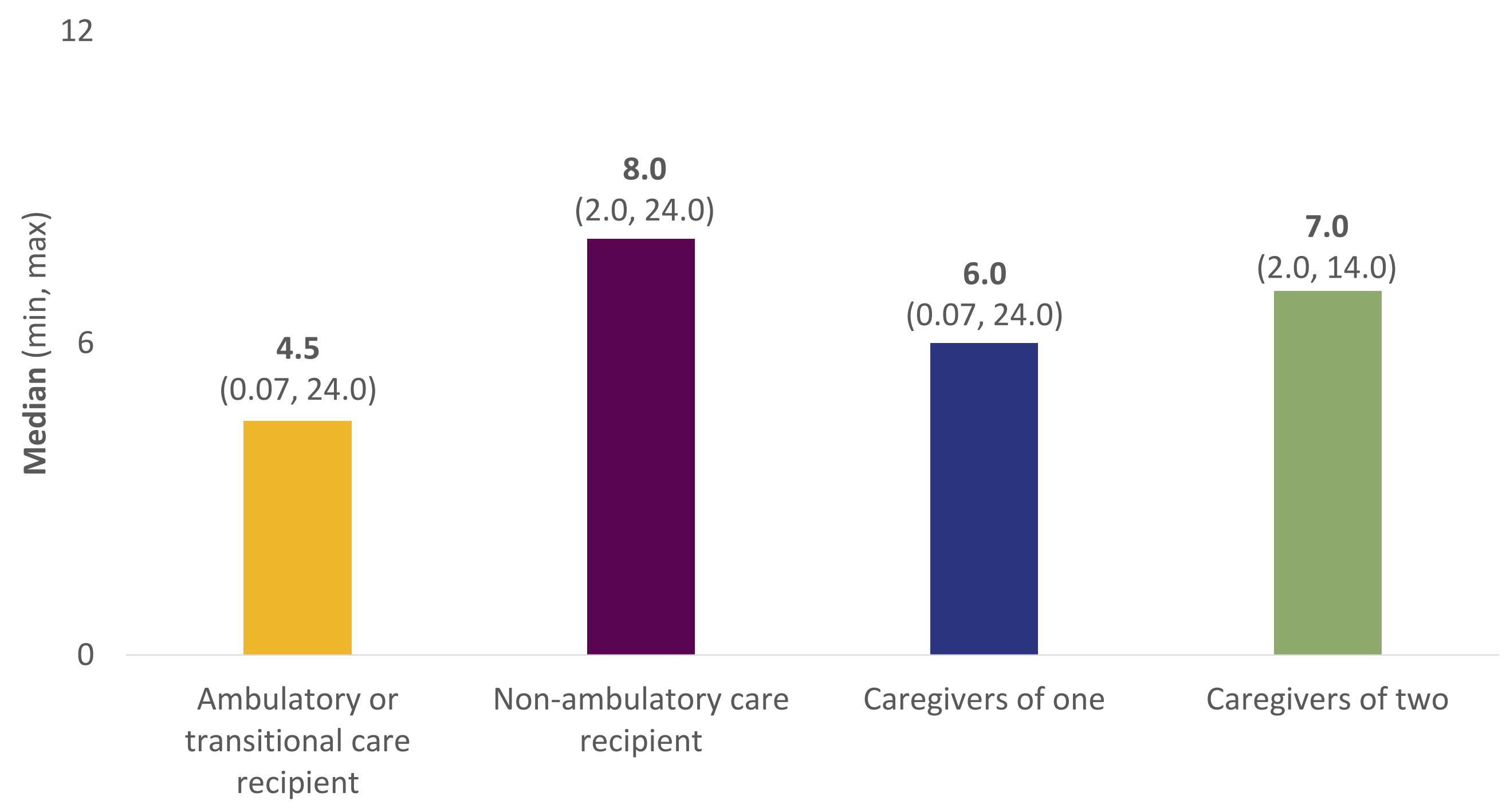


Caregivers of one care recipient	Caregiver of two care recipients
5	5.5
Caregivers of ambulatory/ transitional care recipients	Caregivers of non-ambulatory care recipients
4	6

Time spent caring for care recipient(s)

- Caregivers spend a median of 6 hours per day caring for a loved one with DMD
- Caregivers of one and two care recipients spend a median of 6- and 7-hours a day caregiving, respectively, (Figure 1)
- Caregivers of ambulatory/transitional and non-ambulatory care recipients spend a median of 4.5- and 8-hours a day caregiving, respectively (Figure 1)
- Most caregivers help care recipients with ADLs (79.2%), home exercises and orthotic care (89.6%)
- Caregivers who help care recipients with ADLs:
  - Spend a median (range) of 2.9 (0.5, 24.0) hours on ADLs per day;
  - Help with a median (range) of 6 (0.0, 100.0) transfers per day
- Caregivers who help care recipients with home exercises and orthotic care spend a median (range) of 2 (0.1, 24.0) hours per day on these activities
- In addition, caregivers spend a median (range) of 1.5 (0.0, 20.0) hours attending and 0.5 (0.0, 20.0) hours preparing for healthcare appointments per week

Figure 1 *Hours spent caregiving per day by care recipient ambulatory status and number of care recipients per caregiver*



*Nine caregivers of one care recipient reported they spend 24 hours a day caregiving

Use of informal and formal care

- Seventy-five percent of caregivers get help from additional informal caregivers
- Additional informal caregivers spend a median of 2.0 hours per day caregiving; hours spent caregiving were higher among caregivers of two, or non-ambulatory, care recipients (Table 1)

Table 1 *Use of additional informal care by care-recipient ambulatory status and number of care recipients**

	Number of care recipients		Care recipients' ambulatory status	
	One	Two	Ambulatory/ transitional	Non-ambulatory
N	**94**	**12**	**63**	**55**
Additional informal care, n(%)	72 (76.6)	8 (66.7)	48 (76.2)	40 (72.7)
Hours per day, median (range)	2.0 (0.1, 24.0)	4.4 (1.0, 11.0)	1.9 (0.1, 24)	3 (0.6, 24)

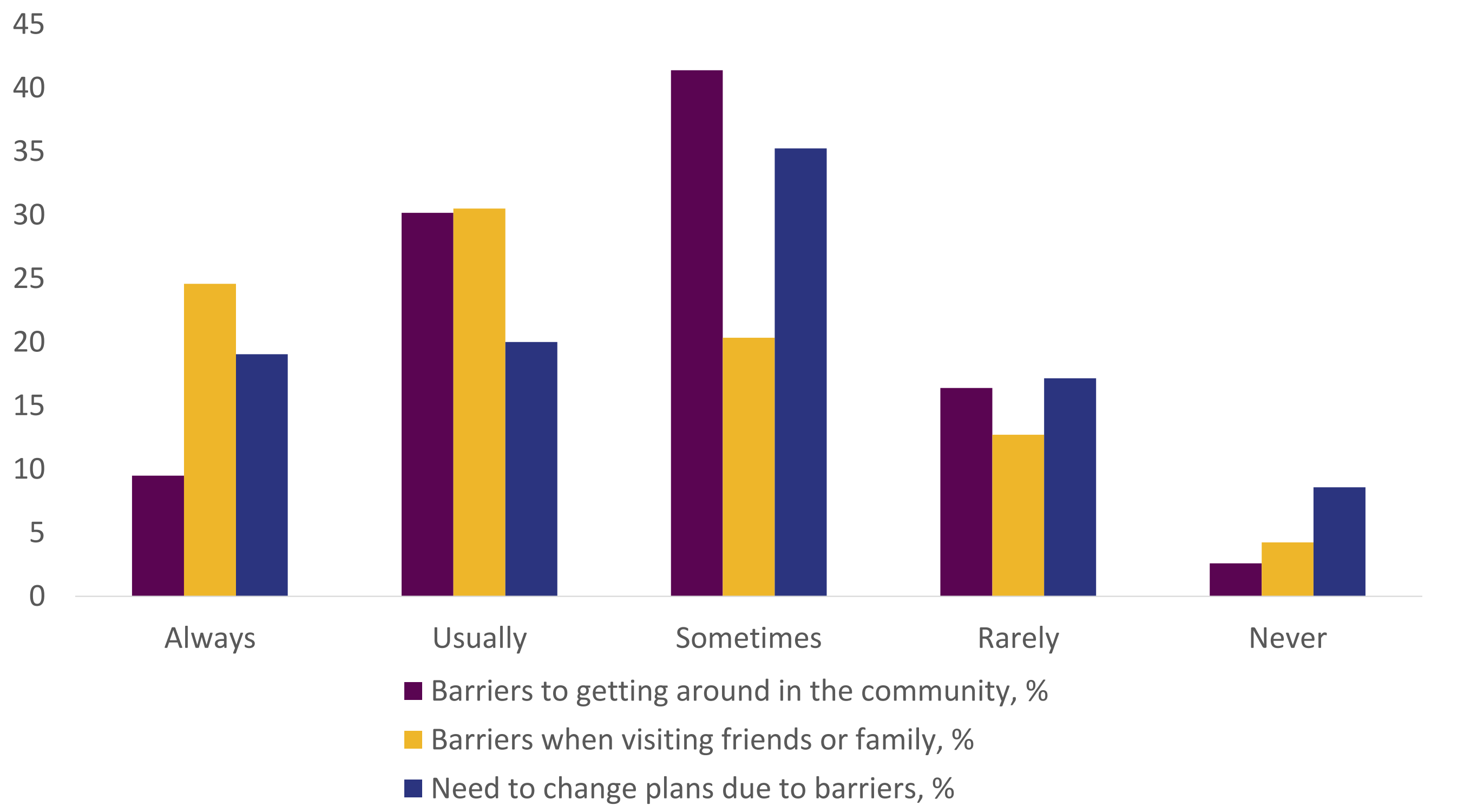
*Informal care from another person not answering the survey e.g., other parent

- Informal care was supplemented by formal caregiving for 26.4% of caregivers
- More caregivers of non-ambulatory care recipients (41.8%, 23/55) utilized formal caregiving compared to caregivers of ambulatory care recipients (12.7%, 8/63)
- Of the caregivers who use formal caregiving:
  - Sixty-four percent (18/28) use formal caregiving 5-7 days/week
  - Eighty-two percent (23/28) reported formal caregiving improves care recipient health and wellbeing; and 75.0% (21/28) of caregivers reported it improves their own health and wellbeing
- Of the caregivers who do not use formal caregiving, 38.2% (29/76) were unable to obtain formal caregiving, most commonly because of affordability (34.5%, 10/29) and not meeting financial criteria (41.4%, 12/29)

Accessibility barriers

- Eleven percent of care recipients only leave their homes several times per month or less; 92.3% of these individuals (12/13) being non-ambulatory
  - Two care recipients, cared for by the same caregiver, never leave their home
- Of the care recipients who leave their homes, 81.0% (94/116) and 76.7% (89/116) experience accessibility barriers (e.g., lack of ramps/railings, uneven steps, narrow doorways) in their community and when visiting friends or family, respectively (Figure 2)
- Additionally, 74.3% of caregivers of care recipients who leave their home (78/105) reported regularly needing to change plans as a result (Figure 2)
  - This is more common in caregivers of non-ambulatory (88.2%; 45/51) vs. ambulatory (63.2%; 36/57) care recipients

Figure 2 *Frequency of experiencing accessibility barriers and changing plans as a result (n=105 caregivers, representing n=116 care recipients)**



*As two care recipients, cared for by the same caregiver, never leave their home

Key Findings and Conclusions

- This study highlights the substantial amount of time, effort, and commitment provided by DMD caregivers; and the increasing caregiving demands associated with DMD progression

Discussion

- Caregivers of non-ambulatory care-recipients reported higher caregiving demands than caregivers of ambulatory or transitional care-recipients
- These findings support that caregiver impact increases with DMD progression[7,1]
- Some caregivers reported high levels of impact (burden scale and time spent caregiving)
  - For this reason, the median burden and time spent caregiving was presented rather than the mean
- People with DMD and their families often face accessibility barriers that impact their ability to move around the community
- Comparisons between caregivers of one and two person(s) with DMD were limited by the small sample (n=12)

Acknowledgments & disclosures

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References

[1]Yamaguchi M, Sonoda E & Suzuki M. Disabil. Rehabil. 2019;41(7):746-752. [2]Schwartz CE, Stark RB, Audhya IF, et al. J PRO. 2021;5(1):1-16. [3]Andreozzi V, Labisa P, Mota M, et al. Health Qual Life Outcomes. 2022;20(1);36. [4]Flores D, Ribate MP, Montolio M, et al. Eur J Health Econ. 2020;21:1015-1023. [5]Pangalila RF, van den Bos GA, Stam HJ, et al. Disabil. Rehabil. 2012;34(12):988-996. [6]Audhya IF, Szabo SM, Bever A, et al. J PRO. 2023;7(1);132. [7]Landfeldt E, Lindgren P, Bell CF, et al. Neurol. 2014;83(6):529-536.

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