PCR62

Household Costs for Home, Vehicle, and Medical Equipment Expenditures to Accommodate Functional Impairments for Individuals With Duchenne Muscular Dystrophy

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Introduction

- Duchenne muscular dystrophy (DMD) is an X-linked neuromuscular disease characterized by progressive muscle damage and degeneration[1,2]
- Clinical manifestations of DMD include progressive muscle weakness, motor milestone delays, loss of ambulation, respiratory impairment, cardiomyopathy, and reduced life expectancy[1–3]
- Earlier studies have reported that families incur extensive costs to accommodate DMD-related functional impairments, including expenses for critical mobility aids and home modifications[4–6]

Objective

This study quantified household costs in the US for home, vehicle, and medical equipment expenditures to accommodate functional impairments for individuals with DMD

Methods

- A noninterventional, retrospective online survey was completed by caregivers of individuals with DMD, who provided information on financial, social, and other aspects of life influenced by DMD
- The survey collected de-identified information on DMD-related home, vehicle, and medical equipment costs incurred over the past 5 years that were not covered by insurance

Study Inclusion Criteria

- Aged ≥18 years and read, understood, and spoke English
- Resided in the US for ≥12 months
- Provided care or support to individual(s) with DMD (≥2 years old) in their household for ≥12 months
- Could provide estimates of the household's costs to support the individual(s) with DMD
- Home modification– or transportation-related expenses within the past 5 years (at least 1 expense was required)
  - Moved to or built new home
  - Modified home entrance (eg, ramp)
  - Modified bathroom, bedroom, or kitchen
  - Modified interior home doorway(s)
  - Installed elevator or lift
  - Purchased and/or modified a handicap-accessible vehicle
- The survey provided data for 2 other separate analyses

Results

Caregivers

- A total of 90 caregivers representing 106 individuals with DMD completed the survey
- 98.1% self-identified as the primary or co-primary caregiver
- 74 caregivers (82.2%) cared for 1 individual and 16 caregivers (17.8%) cared for ≥2 individuals with DMD
- Caregivers were mostly female (94.4%), predominantly White (90.0%), with a mean age of 45.6 years (SD: 8.4)
- Caregivers lived in the South (36.7%), Midwest (31.1%), Northeast (16.7%), or West (15.6%), in a town or suburban area outside of a city (57.8%), rural or countryside area (30.0%), or urban city (12.2%)

Individuals With DMD

- Mean age across the 106 male individuals with DMD was 14.5 years (range: 2–35 years) (**Table 1**)
  - Age 2–5 years (3.8%), 6–12 years (33.0%), 13–17 years (38.7%), or 18+ years (24.5%)
- 59.4% used a wheelchair or scooter full-time

Household Costs Over the Past 5 Years

- Average DMD-related household costs over the past 5 years totaled a mean (SD) of $78,303 ($78,411) for home and vehicle expenses
- The most common DMD-related expense was purchasing or modifying a handicap-accessible vehicle (64%; average cost, mean [SD]: $30,932 [$33,034])
- The most common DMD-related home modification expenses were home entrances (61%; average cost, mean [SD]: $10,236 [$23,697]), bathroom (46%; average cost, mean [SD]: $6,155 [$12,525]), and interior home doorways (37%; average cost, mean [SD]: $991 [$2,122]) (**Table 2**)
- Caregivers reported unaffordability as a reason for avoiding or deferring moving to or building a new home (30%), modifying a bathroom (29%), or purchasing and/or modifying a handicap-accessible vehicle (28%) in the past 5 years
- Average DMD-related medical equipment purchases totaled a mean (SD) of $14,071 ($27,427)

Household Costs Over the Past 12 Months

- Out-of-pocket DMD-related expenses incurred over the past 12 months included health care visits and prescription drugs (54%; average cost, mean of $8,184), supportive therapy (22%; average cost, mean of $929), and in-home professional caregiving (16%; average cost, mean of $4,529) (**Table 3**)
- Out-of-pocket DMD-related health care visits and prescription drug costs were highest for transitional individuals, and in-home professional caregiving costs were greatest for nonambulatory individuals

Table 1 *Demographics of Caregivers and Individuals With DMD*

Demographic Information	Caregivers (N=90)	Individuals With DMD (N=106)
Age in years, mean (SD)	45.6 (8.4)	14.5 (5.3)
Age in years, n (%)		
<18	0 (0)	80 (75.5)
18+	89 (98.9)	26 (24.5)
Missing	1 (1.1)	0 (0)
Sex		
Male, n (%)	5 (5.6)	106 (100.0)
Female, n (%)	85 (94.4)	0 (0)
Race, n (%)[a]		
White	81 (90.0)	92 (86.8)
Hispanic or Latino	7 (7.8)	11 (10.4)
Other[b]	5 (5.6)	15 (14.2)

[a]Survey respondents could select multiple response categories. Therefore, percentages may sum to over 100%. [b]American Indian, Alaska Native, Asian, Black/African American, Other. DMD=Duchenne muscular dystrophy.

Table 2 *Expenses Incurred Over the Past 5 Years to Accommodate Individual(s) With DMD*

Expense Category	Total Households With Purchases/ Modifications (N=90), n (%)	Average Cost Among All Households, Mean (SD)
Home and vehicle purchases/modifications	90 (100)	$78,303 ($78,411)
Purchased and/or modified a handicap-accessible vehicle	58 (64)	$30,932 ($33,034)
Modified home entrances (eg, ramps)	55 (61)	$10,236 ($23,697)
Modified bathroom	41 (46)	$6,155 ($12,525)
Modified interior home doorway(s)	33 (37)	$991 ($2,122)
Moved to or built a new home	23 (26)	$19,679 ($47,344)
Modified bedroom	21 (23)	$1,192 ($4,155)
Installed elevator or lift	17 (19)	$5,289 ($18,270)
Modified kitchen	10 (11)	$1,889 ($6,961)
Other home or vehicle purchases/modifications	20 (22)	$1,940 ($6,062)
Medical equipment purchases[a]	75 (83)	$14,071 ($27,427)

[a]Survey respondents were asked to provide household costs not paid for by health insurance. Respondents were asked to not report the share of expenses paid by private health insurance. DMD=Duchenne muscular dystrophy.

Table 3 *Expenses Incurred Over the Past 12 Months to Accommodate Individual(s) With DMD[a]*

Expense Category[b]	Total Households (N=90), n (%)	Average Cost Among All Households, Mean (SD)
Health care visits and prescription drugs[c]	49 (54)	$8,184 ($37,124)
Ambulatory[d]	7 (47)	$3,401 ($8,299)
Transitional[e]	8 (53)	$35,132 ($87,327)
Nonambulatory[f]	34 (57)	$2,642 ($5,598)
In-home professional caregiving[c]	14 (16)	$4,529 ($20,115)
Ambulatory[d]	1 (7)	$1,941 ($7,517)
Transitional[e]	3 (20)	$1,300 ($3,881)
Nonambulatory[f]	10 (17)	$5,983 ($24,225)
Supportive therapy[c]	20 (22)	$929 ($2,401)
Ambulatory[d]	2 (13)	$312 ($1,078)
Transitional[e]	2 (13)	$133 ($399)
Nonambulatory[f]	16 (30)	$1,283 ($2,828)
Other expenses	72 (80)	$2,593 ($4,144)
Ambulatory[d]	10 (73)	$2,265 ($3,986)
Transitional[e]	14 (93)	$4,165 ($7,470)
Nonambulatory[f]	48 (80)	$2,282 ($2,853)

[a]Survey respondents provided estimated expenses paid during the past 3 months. Results shown are annualized to 12-month expenses based on these data. [b]Average costs were stratified by the greatest ambulatory impairment experienced by a household member with DMD. [c]Survey respondents were asked to provide household costs not paid for by health insurance. Respondents were asked to not report the share of expenses paid by private health insurance. [d]N=15; [e]N=15; [f]N=60. DMD=Duchenne muscular dystrophy.

Key Findings

Households caring for individuals with DMD face a significant financial burden as a result of high out-of-pocket costs, as well as financial barriers in making accommodations due to the progressive nature of DMD

Conclusions

Average household costs over the past 5 years totaled $78,303 for DMD-related home and vehicle expenses (76% out of pocket), and an additional $14,071 for medical equipment purchases (69% out of pocket)

The most frequent expenses were purchasing or modifying a handicap-accessible vehicle and modifying home entrances, bathrooms, and interior doorways

Caregivers reported unaffordability as a reason for avoiding or deferring accommodations for DMD-related functional impairments in the past 5 years

This survey was also used in an analysis of education and employment (PCR118) and sources of financial support to households (podium presentation May 7; 1:45 PM), also presented at ISPOR 2024

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Disclosures: BI, **SP**, and **IA** are employees of Sarepta Therapeutics, Inc., and may own stock/options in the company. **JJ** and **TR** are employees of Medicus Economics, LLC, which received consulting fees from Sarepta Therapeutics, Inc.

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Methods (cont)

Recruitment and Screening

- Survey participants were recruited in collaboration with Rare Patient Voice, an organization with a panel of caregivers for individuals with DMD
- Rare Patient Voice encouraged and invited referrals to join the Rare Patient Voice panel and complete the survey
- Rare Patient Voice contacted panelists and caregiver members living in the US via email with an online link, which directed respondents to an online screening questionnaire
- A Rare Patient Voice screener reviewed the consent and screening sections of the questionnaire. Respondents who passed the screening questions were directed to complete the survey
- Medical records or other personal documents were not accessed or assessed to inform screening or recruitment

Ambulatory Status

- Ambulatory, transitional, or nonambulatory status was determined based upon caregiver-reported scooter or wheelchair usage by individuals with DMD

Statistical Analysis

- Primary analyses used the midpoint of cost ranges to estimate costs for each expense category

Limitations

- Due to the retrospective nature of this study and reliance on self-reported data, there is a potential for recall bias to influence the results
- Findings may not be demographically representative of all US households caring for individuals with DMD and account for costs only within the past 5 years
- The outcomes should be viewed as indicating correlation rather than suggesting a causal relationship

Results (cont)

- The most common DMD mobility-related medical equipment purchases over the past 5 years were powered wheelchair (43%; average cost, mean [SD]: $15,718 [$21,979]), foldable/travel wheelchair (34%; average cost, mean [SD]: $2,084 [$1,804]), and walker/leg braces (33%; average cost, mean [SD]: $1,298 [$1,830]) (**Table 4**)
- The most common DMD respiratory-related medical equipment purchases were cough assist machine (28%; average cost, mean [SD]: $967 [$1,332]) and BiPAP machine (9%; average cost, mean [SD]: $437 [$651]) (**Table 4**)

Table 4 *Medical Equipment Expenses Incurred Over the Past 5 Years to Accommodate Individual(s) With DMD*

Medical Equipment Expense Category	Total Households With Purchases/Modifications, n (%)[a]	Average Cost Among All Households,[b] Mean (SD)
Medical equipment	75 (83)	$16,885 ($30,309)
Powered wheelchair	39 (43)	$15,718 ($21,979)
Foldable/travel wheelchair	31 (34)	$2,084 ($1,804)
Walker/leg braces (eg, ankle foot orthotics)	30 (33)	$1,298 ($1,830)
Cough assist machine	25 (28)	$967 ($1,332)
Manual wheelchair	25 (28)	$3,837 ($8,485)
Safety/hospital bed(s)	22 (24)	$3,513 ($4,431)
Scooter	18 (20)	$3,110 ($4,184)
BiPAP machine	8 (9)	$437 ($651)
Stroller	7 (8)	$708 ($813)
Other breathing assists	4 (4)	$1,937 ($2,563)
Other medical equipment	16 (18)	$4,278 ($7,741)

[a]Percentages reported across the entire population of survey respondents. [b]For each expense category, household costs were derived using the midpoint of the reported cost range. BiPAP=bilevel positive airway pressure; DMD=Duchenne muscular dystrophy.