The Impact of Spinal Muscular Atrophy Type 2 on Caregivers in Argentina: Results of a Global Survey

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Introduction

- SMA is a progressive, autosomal recessive neurodegenerative disorder characterized by progressive muscle weakness and atrophy of variable severity depending on disease type[1]
- SMA type 2 accounts for >20% of new SMA cases and is an intermediate form of SMA with symptoms including muscle weakness and respiratory insufficiency that first appear between 6 and 18 months of age[1,2]
  - Most (>60%) patients with SMA type 2 have a life expectancy of approximately 25 years of age[1]
  - Compared with SMA type 2, SMA type 1 (occurring in >50% of cases) is more severe, with patient life expectancy of <2 years of age, and SMA types 3 and 4 (occurring in 30% and <5% of cases, respectively) are less severe, with patients having normal life expectancies[1]
- A few studies, mostly conducted in the United States and Europe, demonstrated that caregivers of patients with SMA type 1 or 2 experience greater impact on caregiver burden, with time-consuming daily care tasks and financial problems and work adjustments owing to care tasks[3]
- Evidence on the impact of disease management on caregivers of patients with SMA type 2 is limited, particularly in Argentina, where SMA type 2 is the predominant form of the disease[4]

Objective

- We sought to describe the sociodemographics, HCRU, and time and out-of-pocket costs for caregivers of patients with SMA type 2 in Argentina

Methods

- An online survey of caregivers in Argentina, Brazil, Japan, Taiwan, the United Kingdom, and the United States was conducted to collect data on time spent and economic impact of caring for a patient with SMA types 1–3 (**Figure 1**). Here, we report the results for caregivers in Argentina of patients with SMA type 2.

Figure 1. Caregiver survey study design

Study Design

Cross-sectional, non-interventional online survey of voluntary non-HCP caregivers in Argentina. The survey was disseminated to caregivers through the FAME patient advocacy group

Survey start February 2022

Survey end May 2022

Data Collected in Survey

- Caregiver sociodemographics
- Characteristics of patients with SMA cared for by caregivers
- Time spent as a caregiver
- Impact of caregiving on employment and income
- HCRU
- Direct medical care: HCP visits, surgeries, or hospitalizations
- Indirect medical care: out-of-pocket expenses

Participants

Adult, unpaid, primary or secondary caregivers of a child or adult with SMA type 2 at the time of survey completion

- HCP caregivers
- Paid caregivers (e.g., home health aides or registered nurses)
- Caregivers of patients residing in stationary facilities (e.g., nursing homes or hospice centers)

Ethnics

- All participants provided informed, electronic consent
- Study documents and protocols have been approved by an international IRB and local ethics committees

FAME, Families of SMA, Argentina; HCP, health care provider; HCRU, health care resource utilization; IRB, institutional review board; SMA, spinal muscular atrophy.

Results

Caregiver demographics and patient characteristics

- 49 caregivers managing 50 total patients with SMA type 2 responded to the survey
- Caregivers were a mean age of 43.6 years, and in a majority of cases (60%) the caregiver was the patient's mother (**Table 1**)

Table 1. Sociodemographics for caregivers of patients with SMA type 1

Characteristic	Caregivers (N=49)
Sex, n (%)	
Female	34 (68.0)
Male	16 (32.0)
Relationship to the patient, n (%)	
Mother	30 (60.0)
Father	13 (26.0)
Another family member	2 (4.0)
Not a family member	5 (10.0)
Age, years	
Mean (SD)	43.6 (10.6)
Median (range)	43.0 (21.0–76.0)
Highest education level completed, n (%)	
Elementary or junior high school	2 (4.0)
Did not graduate high school	1 (2.0)
High school graduate	12 (24.0)
Associate's degree	16 (32.0)
Bachelor's degree	9 (18.0)
Master's degree or higher	10 (20.0)
Marital status, n (%)	
Single	4 (8.0)
Divorced or separated	6 (12.0)
Widowed	1 (2.0)
Married or in a domestic partnership	32 (64.0)
Living with a partner (not married or in a domestic partnership)	7 (14.0)
Geographic entity, n (%)	
Rural (countryside or village)	4 (8.0)
Urban (town or city)	46 (92.0)
Number of children in the household younger than 18 years of age[a]	
Mean (SD)	1.4 (1.0)
Median (range)	1.0 (0–3.0)
Current employment status, n (%)[b]	
Employed full time	11 (19.0)
Employed part time	9 (15.5)
Self-employed/business owner	19 (32.8)
Student	2 (3.4)
Retired	3 (5.2)
Full-time caregiver of a person with SMA	7 (12.1)
Not working due to own disability	2 (3.4)
Homemaker	5 (8.6)
Current gross income, n (%)[c]	
<ARS 240,000/year	7 (14.0)
240,000–360,000 ARS/year	0
360,000–620,000 ARS/year	4 (8.0)
620,000–1,560,000 ARS/year	12 (24.0)
>1,560,000 ARS/year	9 (18.0)

ARS, Argentine pesos; SD, standard deviation; SMA, spinal muscular atrophy.
[a]Total number of children in the household younger than 18 years of age, including the patient with SMA type 2. [b]Caregivers may select more than one option. [c]Seventeen caregivers (34.0%) selected "I prefer not to answer this question," and one (2.0%) selected "I don't know."

- The patients with SMA type 2 under the care of these caregivers were a mean age of 15.2 years and were diagnosed with SMA at a mean age of 3.2 years (**Table 2**)
  - Patients achieved their highest level of motor function at a mean age of 7.3 years
  - Most caregivers reported that their patients required the use of respiratory (75.5%; n=37/49) or mobility (95.9%; n=47/49) equipment within the past 6 months

Table 2. Characteristics of patients with SMA type 2

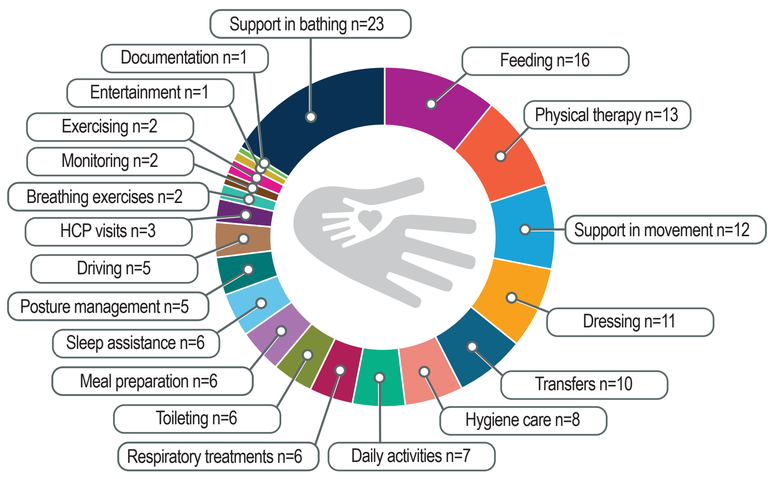
Characteristic	Patients (N=50)[a]
Age, years	
Mean (SD)	15.2 (8.9)
Median (range)	13.3 (1.4–41.3)
Age at SMA diagnosis, years	
Mean (SD)	3.2 (6.1)
Median (range)	1.5 (0.9–29.0)
Time from first signs/symptoms to diagnosis of SMA, months	
Mean (SD)	29.5 (79.4)
Median (range)	6.0 (0–344.0)
Highest level of motor function achieved, n (%)	
Head control	11 (22.0)
Rolling	2 (4.0)
Sitting independently for >10 seconds	8 (16.0)
Standing without support	6 (12.0)
Standing with support	6 (12.0)
Walking with support	12 (24.0)
Walking without support	4 (8.0)
None of the above	1 (2.0)
Age patient maintained highest level of motor function achieved, years[b]	
Mean (SD)	7.3 (8.0)
Median (range)	6.0 (0.1–40.2)
Current level of motor function, n (%)[b]	
Head control	18 (36.7)
Rolling	0
Standing without support	0
Standing with support	8 (16.3)
Sitting independently for >10 seconds	16 (32.7)
Walking with support	3 (6.1)
Walking without support	4 (8.2)
None of the above	0
Respiratory equipment used within the past 6 months, n (%)[c]	
Cough assist machine	28 (34.1)
Pulse oximeter	17 (20.7)
BiPAP machine	9 (11.0)
Nebulizer	7 (8.5)
Noninvasive ventilation	3 (3.7)
Invasive ventilation via tracheostomy	2 (2.4)
Suction machine	2 (2.4)
Night BiPAP machine	1 (1.2)
Other: Ambu	1 (1.2)
None	12 (14.6)
Mobility equipment used in the past 6 months, n (%)[b]	
Ankle-foot orthoses	27 (23.5)
Wheelchair	46 (40.0)
Foot braces	12 (10.4)
Corset or spinal jacket	6 (5.2)
Special bed	5 (4.3)
Special seating or sitting retainer	5 (4.3)
Hand braces	4 (3.5)
Foam collar and bathing chair	1 (0.9)
Knee stabilizer/standing frame	1 (0.9)
Long bilateral splints	1 (0.9)
Motorized standing frame/walker	1 (0.9)
Scooter	1 (0.9)
Walking sticks	1 (0.9)
Standing tricycle	1 (0.9)
Vibration machine	1 (0.9)
None	2 (1.7)
Nutrition support used in the past 6 months, n (%)	
Calorie supplements	1 (2.0)
None	49 (98.0)

BiPAP, bilevel positive airway pressure; SD, standard deviation; SMA, spinal muscular atrophy.
[a]Responses provided by 49 caregivers for a total of 50 patients with SMA type 2. [b]Forty-eight of 49 caregivers provided responses. [c]Caregivers may select more than one option.

Level of care provided

- All respondents were either the patient's primary (44.9%; n=22/49) or coprimary (57.1%; n=28/49) caregiver
- The mean (SD; median [range]) patient care time reported by caregivers (n=49) was 79.7 (58.1; 63.0 [5–168]) hours/week
- Support in bathing, feeding, physical therapy, and support in movement were reported by caregivers as the most time-consuming activities in caring for their patients with SMA type 2 (**Figure 2**)

Figure 2. Most time-consuming tasks reported by caregivers of patient with SMA type 2

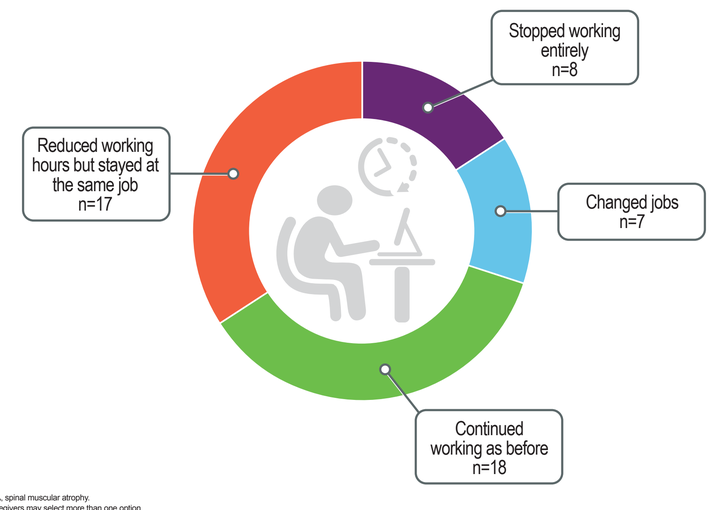


HCP, health care provider; SMA, spinal muscular atrophy.

Employment and income

- Of the 49 caregiver respondents, eight stopped working to provide care, 17 reduced their working hours, and seven changed jobs to provide care for their patients with SMA type 2 (**Figure 3**). Caregivers (n=23 respondents) reduced their working hours by a mean (SD; median [range]) of 13.8 (12.2; 10.0 [1.0–40.0]) hours/week.

Figure 3. Employment changes for caregivers of patients with SMA type 2[a]



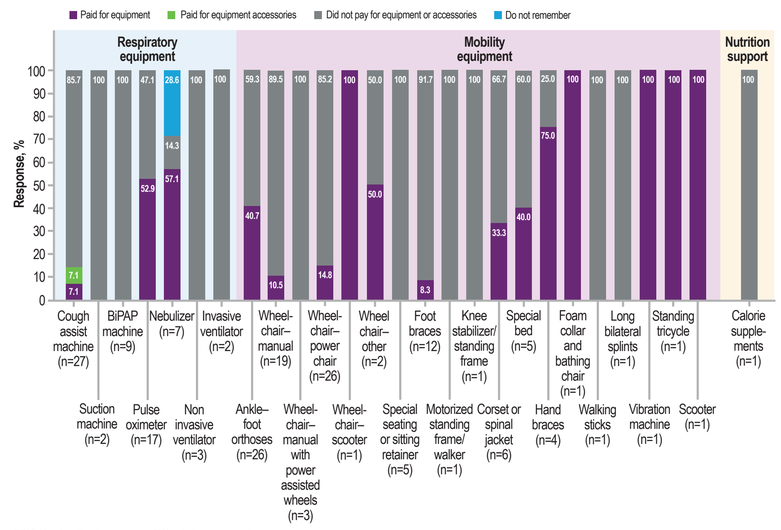
SMA, spinal muscular atrophy.
[a]Caregivers may select more than one option.

- Within the past 6 months, 25 of 41 (59.5%) caregiver respondents took days off from work to provide care. The mean (SD; median [range]) number of days caregivers (n=24 respondents) took off from work was 22.0 (37.4; 10.0 [2.0–180.0]) days.
- 29 of 49 (59.2%) caregivers reported an impact on income due to caregiving, with an estimated mean (SD; median [range]) income reduction of 42.2% (26.2%; 40.0% [0–100%]) per month among 28 caregiver respondents

HCRU

- All 49 caregivers consulted at least one HCP during the past 6 months for their patients with SMA type 2. The most common (n>35 respondents) HCPs consulted were neurologists (n=44), physiotherapists (n=43), and pediatricians (n=39).
- For patients with SMA type 2 who had surgeries, the most common (n≥4 respondents) surgeries included spinal fusions (n=14), spinal growth rod surgeries with periodic surgical mechanical expansion (n=6), and tracheostomies (n=4)
- 11 caregiver respondents reported ≥1 overnight hospitalizations, excluding SMA-related surgeries, for their patients with SMA type 2 within the past 6 months
  - The primary reasons for these hospitalizations were receiving SMA treatment (n=5), chest infection/difficulties breathing (n=4), and pregnancy, bronchospasm, gall bladder surgery, or pneumothorax (each n=1)
  - The mean (SD; median [range]) duration of these hospitalizations reported by these 11 caregiver respondents was 11.6 (16.4; 5.0 [1.0–56.0]) days
- Caregivers reported paying out-of-pocket expenses for some respiratory and mobility equipment needed for their patients with SMA type 2 (**Figure 4**)

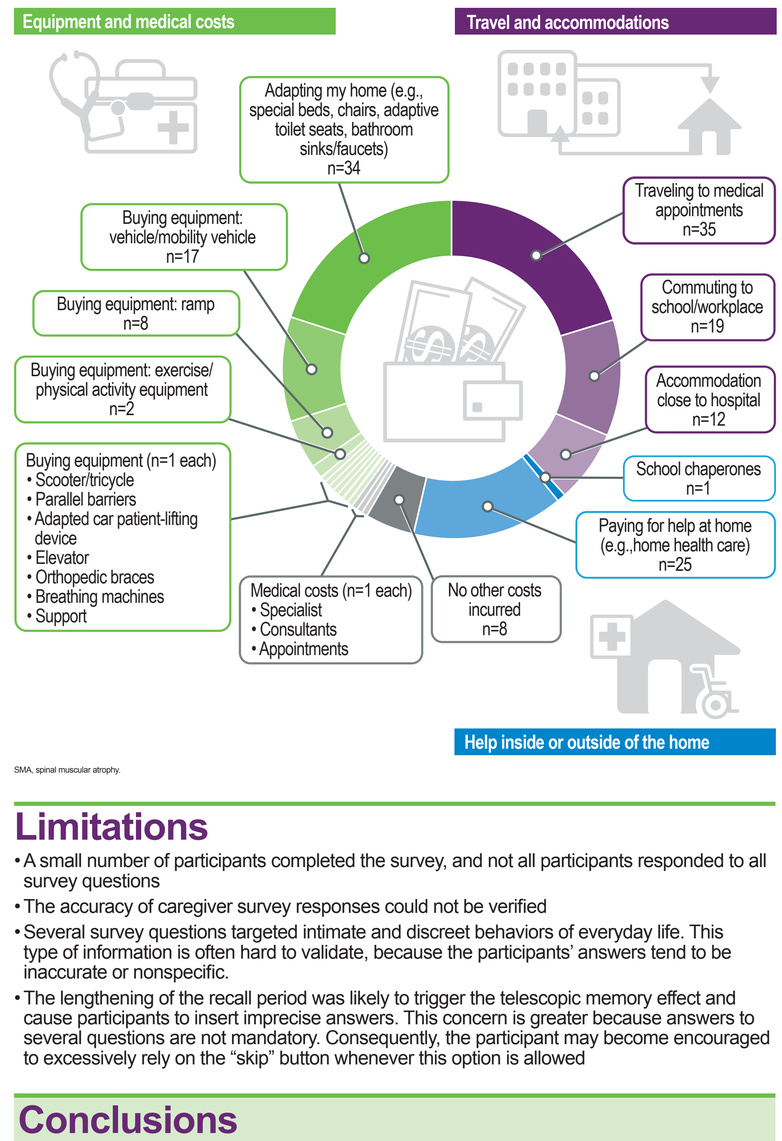
Figure 4. Out-of-pocket expenses caregivers reported for respiratory, mobility, and nutrition support needed for their patients with SMA type 2



BiPAP, bilevel positive airway pressure; SMA, spinal muscular atrophy.

- Caregivers also reported paying out-of-pocket expenses for equipment and other medical costs, travel and accommodations, and help inside or outside of the home to care for their patients with SMA type 2 (**Figure 5**). The mean (SD; median [range]) expenditures for the most common (n>20 respondents) out-of-pocket expenses were:
  - Traveling to medical appointments (n=34): ARS 55,964.60 (140,711.80; 10,000 [0–780,000])
  - Home adaptations (n=33): ARS 356,198.50 (801,495.20; 40,000 [0–4,000,000])
  - Paying for help at home (n=25): ARS 140,626 (131,466.30; 120,000 [0–500,000])

Figure 5. Additional out-of-pocket expenses reported by caregivers of patients with SMA type 2



SMA, spinal muscular atrophy.

Limitations

- A small number of participants completed the survey, and not all participants responded to all survey questions
- The accuracy of caregiver survey responses could not be verified
- Several survey questions targeted intimate and discreet behaviors of everyday life. This type of information is often hard to validate, because the participants' answers tend to be inaccurate or nonspecific.
- The lengthening of the recall period was likely to trigger the telescopic memory effect and cause participants to insert imprecise answers. This concern is greater because answers to several questions are not mandatory. Consequently, the participant may become encouraged to excessively rely on the "skip" button whenever this option is allowed

Conclusions

- Caregivers in Argentina were often family members of teens with SMA type 2, who often required respiratory or mobility equipment
- Caregivers reported a substantial impact on their time for providing care, particularly related to support in bathing, feeding, physical therapy, and movement
- Often caregivers reduced their working hours or stopped working, leading to a reduction in income
- Caregivers reported out-of-pocket costs for equipment, travel and accommodations, and help inside or outside of the home
- Early identification and treatment of SMA type 2 may reduce associated costs and resulting impact on caregivers and patients

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Abbreviations

ARS, Argentine peso; BiPAP, bilevel positive airway pressure; FAME, Families of SMA, Argentina; HCP, health care provider; HCRU, health care resource utilization; IRB, institutional review board; SD, standard deviation; SMA, spinal muscular atrophy.

Acknowledgments and Disclosures

This study was funded by Novartis Gene Therapies, Inc.

The authors would like to thank the patient advocacy group Families of SMA Argentina (Familias AME; FAME) for their support of this analysis. The authors also wish to thank the patients, families, and caregivers for their willingness to participate in this survey, which is sponsored by Novartis Gene Therapies, Inc.

Medical writing and editorial support were provided by Sarah Hauze, PhD, Kay Square Scientific, Newtown Square, PA. This support was funded by Novartis Gene Therapies, Inc.

Disclosures: AP, **WT**, **MEE**, and **OD** are employees of Novartis Gene Therapies, Inc. and own stock/other equities.

Presented at The Professional Society for Health Economics and Outcomes Research (ISPOR) Europe 2023, November 12–15, 2023, Copenhagen, Denmark.