The Impact of Spinal Muscular Atrophy Type 1 on Caregivers in Taiwan: Results of a Global Survey

RWD164

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Introduction

- SMA is a progressive, autosomal recessive neurodegenerative disorder and the most common genetic cause of infant mortality[1]
- SMA type 1, accounting for >50% of cases, is the most severe form of SMA, with death often occurring prior to 2 years of age without treatment.[1,2] Compared with SMA type 1, SMA types 2, 3, and 4 occur at a lower frequency (20%, 30%, and <5% of cases, respectively) and have less severe clinical manifestations, with patient life expectancies ranging from approximately 25 years of age up to a normal lifespan.[1]
- Within the first 6 months of life, patients with SMA type 1 develop symptoms including limb weakness, respiratory insufficiency, and poor feeding.[1] Historically, patients with SMA type 1 did not achieve the ability to sit independently.[1]
- A few studies, mainly conducted in the United States and Europe, demonstrated that caregivers of patients with SMA types 1 and 2 experienced greater impact on caregiver burden, with time-consuming daily care tasks, financial problems, and work adjustments owing to care tasks[3]
- Overall, data are limited on the impact of the time and costs associated with care for patients with SMA type 1, particularly in Taiwan, where the estimated prevalence of SMA (5.8 per 100,000 births) is greater than the global estimated prevalence (1 per 100,000 births)[4,5]

Objective

- We sought to describe the sociodemographics, HCRU, and time and out-of-pocket costs for caregivers of patients with SMA type 1 in Taiwan

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 the economic impact of caring for patients with SMA types 1–3 (**Figure 1**). Here, we report the results for caregivers of patients with SMA type 1 in Taiwan.

Figure 1. Caregiver survey study design

Study Design

Cross-sectional, non-interventional online survey of voluntary non-HCP caregivers in Taiwan. The survey was disseminated to caregivers through patient advocacy groups and principal investigators

Survey start May 2022 → **Survey end** December 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 1 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

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

Results

Caregiver demographics and patient characteristics

- Sixteen caregivers managing 16 patients with SMA type 1 responded to the survey
- Caregivers were a mean age of 40.6 years and were either the patient's mother (50%) or father (50%; **Table 1**)

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

Characteristic	Caregivers (N=16)
Sex, n (%)	
Female	8 (50.0)
Male	8 (50.0)
Relationship to the patient, n (%)	
Mother	8 (50.0)
Father	8 (50.0)
Age, years	
Mean (SD)	40.6 (4.3)
Median (range)	41.0 (33.0–51.0)
Highest education level completed, n (%)	
Elementary or junior high school	2 (12.5)
Did not graduate high school	1 (6.3)
Vocational or general senior high school	5 (31.3)
Bachelor's degree	4 (25.0)
Master's degree or higher	4 (25.0)
Marital status, n (%)	
Married or in a domestic partnership	16 (100)
Geographic entity, n (%)	
Rural (countryside or village)	5 (31.3)
Urban (town or city)	11 (68.8)
Number of children in the household younger than 18 years of age[a]	
Mean (SD)	1.4 (0.7)
Median (range)	1.0 (1.0–3.0)
Current employment status, n (%)[b]	
Employed full time	7 (38.9)
Employed part time	2 (11.1)
Unemployed	2 (11.1)
Full-time caregiver of a person with SMA	3 (16.7)
Homemaker	4 (22.2)
Current gross income, n (%)[c]	
<NT $400,000/year	5 (31.3)
NT $400,000–749,999/year	3 (18.8)
NT $750,000–999,999/year	5 (31.3)
NT $1,000,000–1,500,000/year	1 (6.3)
>NT $1,500,000/year	1 (6.3)

NT, New Taiwan dollars; SD, standard deviation; SMA, spinal muscular atrophy.
[a]Total number of children in the household, including patient with SMA type 1. [b]Caregivers may select more than one option. [c]One (6.3%) caregiver selected "I prefer not to answer this question."

- The mean age of patients with SMA type 1 was 4.5 years, and the mean age at SMA diagnosis was 0.3 years (**Table 2**)
  - Patients achieved their highest level of motor function at a mean age of 1.5 years
  - Most patients required the use of respiratory, mobility, or nutritional equipment within the past 6 months (each 87.5%; n=14/16)

Table 2. Characteristics of patients with SMA type 1

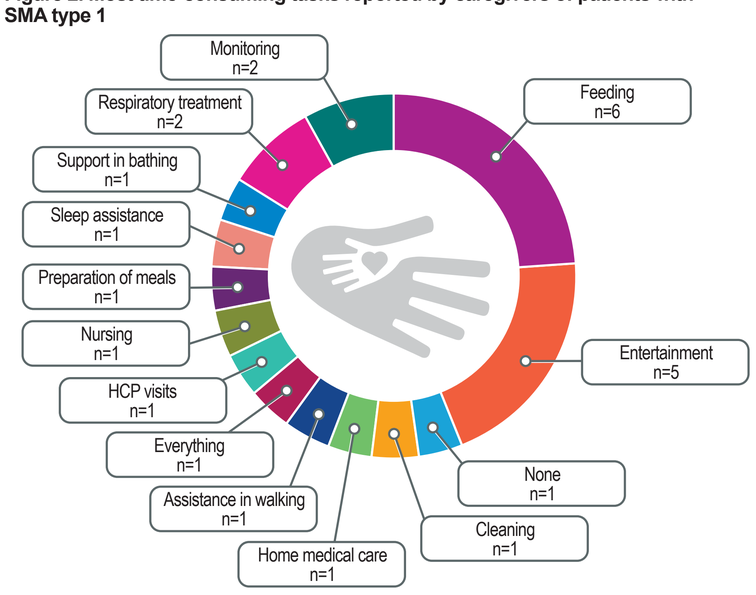
Characteristic	Patients[a] (N=16)
Age, years	
Mean (SD)	4.5 (2.5)
Median (range)	5.0 (0.3–8.0)
Age at SMA diagnosis, years[b]	
Mean (SD)	0.3 (0.3)
Median (range)	0.2 (0–0.9)
Time from first signs/symptoms to diagnosis of SMA, months[b]	
Mean (SD)	1.4 (2.2)
Median (range)	0.5 (0–6)
Highest level of motor function achieved, n (%)[b]	
Head control	3 (27.3)
Sitting independently for >10 seconds	3 (27.3)
Standing with support	0
Standing without support	0
Walking with support	3 (27.3)
Walking without support	0
None of the above	2 (18.2)
Age patient maintained highest level of motor function achieved, years[c]	
Mean (SD)	1.5 (1.8)
Median (range)	0.8 (0.1–5.0)
Current level of motor function, n (%)[c]	
Head control	3 (33.3)
Rolling	0
Sitting independently for >10 seconds	2 (22.2)
Standing with support	0
Standing without support	0
Walking with support	3 (33.3)
Walking without support	1 (11.1)
None of the above	0
Respiratory equipment used within the past 6 months, n (%)[d]	
Cough assist machine	14 (32.6)
Suction machine	7 (16.3)
BiPAP machine	7 (16.3)
Pulse oximeter	6 (14.0)
Nebulizer	4 (9.3)
Noninvasive ventilation	3 (7.0)
None	2 (4.7)
Mobility equipment used in the past 6 months, n (%)[d]	
Ankle-foot orthoses	9 (30.0)
Wheelchair	7 (23.3)
Adaptive	3 (27.3)
Manual	5 (45.5)
Power	3 (27.3)
Special seating or sitting retainer	6 (20.0)
Foot braces	3 (10.0)
Corset or spinal jacket	2 (6.7)
Hand braces	1 (3.3)
None	2 (6.7)
Nutrition equipment used in the past 6 months, n (%)[d]	
NG tube	1 (5.9)
G-tube	14 (82.4)
None	2 (11.8)

BiPAP, bilevel positive airway pressure; G-tube, gastric feeding tube; NG, naso-gastric; SD, standard deviation; SMA, spinal muscular atrophy.
[a]Responses provided by 16 caregivers for a total of 16 patients with SMA type 1. [b]Eleven of 16 caregivers provided responses. [c]Nine of 16 caregivers provided responses. [d]Caregivers may select more than one option.

Level of care provided

- Eleven of 16 (68.8%) caregivers were either the patient's primary (50%; n=8/16) or coprimary (18.8%; n=3/16) caregiver. The remaining five of 16 (31.3%) caregivers were the patient's secondary caregiver.
- The mean (SD; median [range]) patient care time reported by caregivers (n=16) was 79.4 (60.9; 72.0 [2–168]) hours/week
- Caregivers reported that entertainment and feeding were the most time-consuming activities in caring for their patients with SMA type 1 (**Figure 2**)

Figure 2. Most time-consuming tasks reported by caregivers of patients with SMA type 1

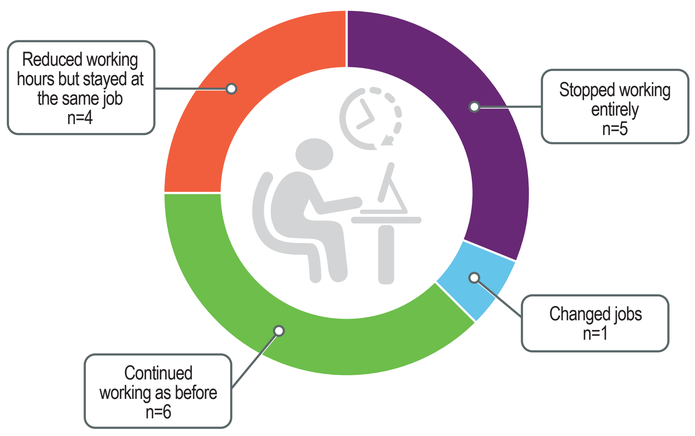


HCP, health care provider; SMA, spinal muscular atrophy.

Employment and income

- Of the 16 caregiver respondents, five stopped working, four reduced their working hours, and one changed jobs to provide care for their patients with SMA type 1 (**Figure 3**)
- Caregivers (n=5 respondents) reduced their working hours by a mean (SD; median [range]) of 62 (63.3; 30 [12–168]) hours/week

Figure 3. Employment changes for caregivers of patients with SMA type 1



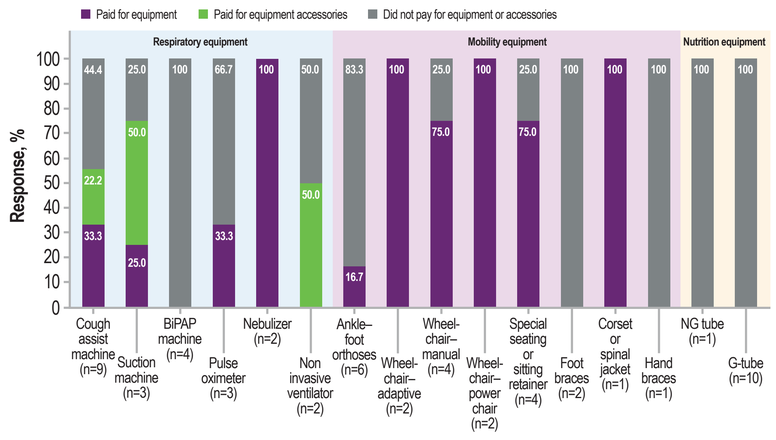
SMA, spinal muscular atrophy.

- Within the past 6 months, eight of 11 (72.7%) caregivers took days off from work for caregiving. The mean (SD; median [range]) number of days these eight caregivers took off from work in the past 6 months was 5.4 (2.9; 5.0 [3.0–12.0]) days.
- Ten of 16 (62.5%) caregivers reported an impact on income owing to caregiving, with an estimated mean (SD; median [range]) income reduction of NT $47,000 (57,164.50; 25,000 [10,000–200,000]) per month for 16 caregiver respondents

HCRU

- Most caregivers (68.8%; n=11/16) consulted at least one HCP during the past 6 months for their patient with SMA type 1. The most common HCPs consulted (n>5 respondents) were neurologists (n=8), physiotherapists (n=6), and specialist neuromuscular nurses (n=6).
- For patients with SMA type 1 who had surgeries, surgery types included gastrostomies (n=7), gastrostomies with Nissan fundoplication (n=4), and spinal tap injections (n=1)
- Five of 11 patients (45.5%) had ≥1 overnight hospitalizations, excluding SMA-related surgeries, within the past 6 months
  - The primary reasons for these hospitalizations were receiving SMA treatment (n=3), chest infection/breathing difficulties (n=1), and infection with epidemic disease (n=1)
  - The mean (SD; median [range]) duration of these hospitalizations for these five patients was 4.5 (2.1; 4.5 [2–7]) days
- Caregivers reported paying out-of-pocket expenses for most respiratory and mobility, but not nutritional, equipment needed for their patients with SMA type 1 (**Figure 4**)

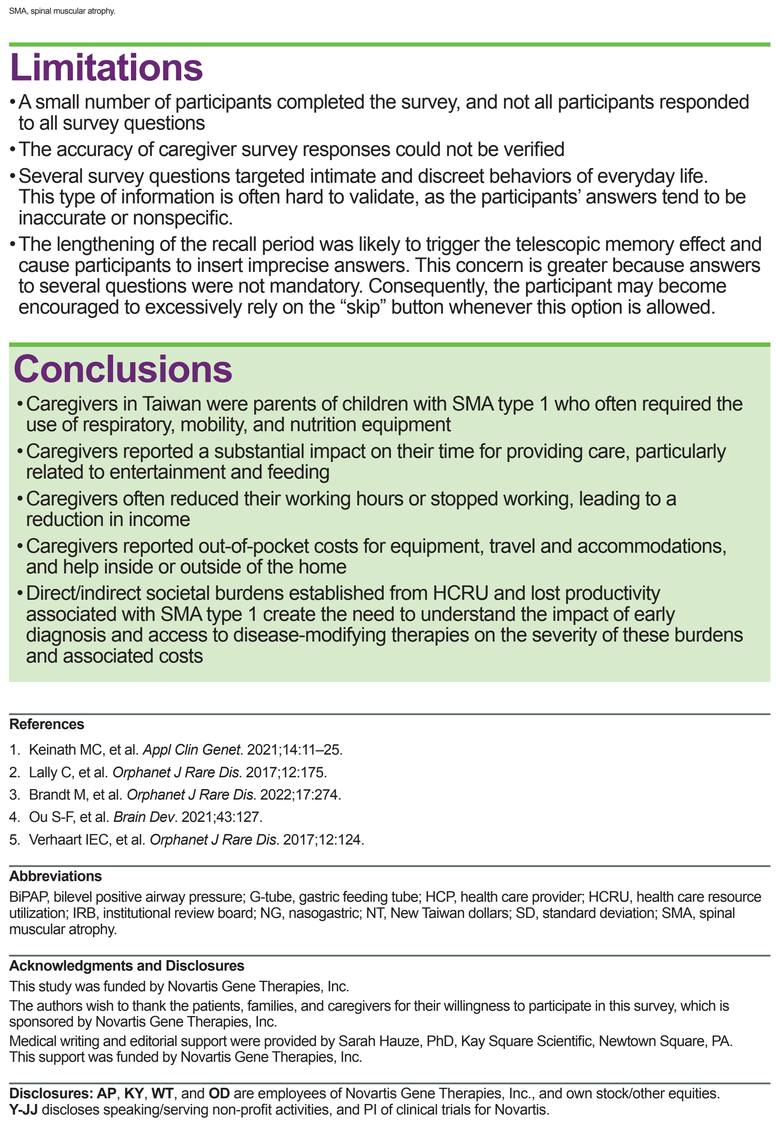
Figure 4. Out-of-pocket expenses caregivers reported for respiratory, mobility, and nutrition equipment needs for their patients with SMA type 1



BiPAP, bilevel positive airway pressure; G-tube, gastric feeding tube; NG, nasogastric; SMA, spinal muscular atrophy.

- Caregivers also reported paying out-of-pocket expenses for travel and accommodations, other mobility equipment, and help inside or outside of the home to care for their patients with SMA type 1 (**Figure 5**)

Figure 5. Additional out-of-pocket expenses reported by caregivers of patients with SMA type 1



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, as 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 were not mandatory. Consequently, the participant may become encouraged to excessively rely on the "skip" button whenever this option is allowed.

Conclusions

- Caregivers in Taiwan were parents of children with SMA type 1 who often required the use of respiratory, mobility, and nutrition equipment
- Caregivers reported a substantial impact on their time for providing care, particularly related to entertainment and feeding
- Caregivers often 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
- Direct/indirect societal burdens established from HCRU and lost productivity associated with SMA type 1 create the need to understand the impact of early diagnosis and access to disease-modifying therapies on the severity of these burdens and associated costs

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Abbreviations

BiPAP, bilevel positive airway pressure; G-tube, gastric feeding tube; HCP, health care provider; HCRU, health care resource utilization; IRB, institutional review board; NG, nasogastric; NT, New Taiwan dollars; SD, standard deviation; SMA, spinal muscular atrophy.

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