Treatment-Related and Non-Treatment-Related Out-of-Pocket Costs by Amyotrophic Lateral Sclerosis Disease Stage: A Cross-Sectional Patient Survey and Retrospective Chart Review

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Please use this QR code to download a PDF of the poster and the supplemental tables.

Summary

What we did

- Retrospective medical chart review and patient/caregiver survey of patients with ALS to determine treatment-related and non-treatment-related out-of-pocket costs in relation to three ALS staging systems

What we found

- King's, MiToS and FT9 staging systems each provide unique insights into treatment- and non-treatment-related out-of-pocket costs associated with ALS
- Treatment-related out-of-pocket costs were generally higher in later disease stages than in earlier disease stages, but the staging system used influenced the magnitude of the difference observed

Why it matters

- To our knowledge, this is the first study to assess out-of-pocket costs by ALS disease stage
- More information is needed on the socioeconomic burden of ALS during different stages of disease progression

Objective

- To assess treatment-related and non-treatment-related out-of-pocket costs for people with amyotrophic lateral sclerosis (ALS) by disease stage, using data from physician review of medical chart records and a patient/caregiver survey

Introduction

- ALS is a progressive neurodegenerative disease that results in upper and lower motor neuron degeneration, leading to paralysis and respiratory failure[1,2]
- Treatment options for ALS are limited[1] and ALS is associated with a significant economic burden for patients[3]
- The King's clinical staging system and the Milano-Torino (MiToS) functional staging system are complementary tools used to monitor ALS disease progression[4]
- The more recently developed Fine'til 9 (FT9) staging system is based on the revised ALS functional rating scale (ALSFRS-R),[5] which is commonly used in clinical trials[6]

Methods

Study design

- Data were collected via the Adelphi ALS Disease Specific Programme™, a point-in-time survey of physicians and their consulting patients with ALS in France, Germany, Italy, Spain (EU4), the UK and US between July 2020 and March 2021
- Physician inclusion criteria were: Neurology as a primary speciality of the physician, active involvement in the management of patients with ALS, including management of at least one patient with a diagnosis of ALS
- Patient enrolment occurred on a prospective basis following physician enrolment; patients were ≥18 years old with a confirmed diagnosis of ALS

Physician-completed patient record forms

- Data on patient demographics and ALS staging were collated using retrospective medical chart reviews by neurologists
  - Disease status for each patient was classified by King's, MiToS and FT9 staging systems

Patient/caregiver survey

- Patients for whom the physician completed a patient record form were invited to complete a survey themselves; non-professional caregivers were invited to complete the survey on behalf of patients who were willing but unable to complete the survey
- Treatment-related out-of-pocket costs included monthly costs (2021 euros) for prescription medicines; non-prescription medicines; professional caregivers; supportive therapies, services, counselling; and other treatment-related costs
- Non-treatment-related out-of-pocket costs included annual costs (2021 euros) for transport to appointments/hospital visits; transport modifications, home modifications; supportive devices; testing; hospital visits; and care home/assisted living
  - Scan poster QR code for supplemental tables

Results

Patient baseline demographics and characteristics

- Data were reported for 172 patients globally; 70% of respondents were from EU4 (**Table 1**)
  - All cost data were derived from surveys completed by the patient only (n=90), caregiver only (n=37) or patient and their caregiver (n=45); in cases where a patient and their caregiver both completed the survey, only the patient data were used

Table 1 Baseline patient characteristics and disposition

	Patient population (N=172)
Geographical location, n (%)	
EU4	121 (70)
UK	8 (5)
US	43 (25)
Age, years, mean (SD)	60.8 (11.5)
Sex, female, n (%)	68 (40)
BMI, kg/m^2, mean (SD)	23.7 (3.0)
Employment status, n (%)*	
Working full-time	13 (8)
Working part-time	21 (12)
On long-term sick leave	32 (19)
Retired	81 (48)
Unemployed	9 (5)
Homemaker	13 (8)
Student	1 (1)
Living circumstances, n (%)†	
Lives alone in own home	15 (9)
Lives with partner/spouse/immediate family	141 (82)
Lives with other family/friends	8 (5)
Lives in hospice	1 (1)
Lives in nursing home	5 (3)
Lives in assisted living residence/residential home	1 (1)
ALSFRS-R score, mean (SD)	32.7 (12.4)
Number of comorbidities, mean (SD)	1.5 (1.4)
Charlson Comorbidity Index, mean (SD)	0.28 (0.81)

*Data for employment status represent 170 patients. †Data for living circumstances represent 171 patients.

ALS staging of study population

- More patients were reported as having more severe disease according to the King's and FT9 staging systems than the MiToS staging system (**Table 2**)

Table 2 Clinical characteristics*

	Patient population (N=172)
King's staging system, n (%)	
Stage 1 (involvement of one region)†	29 (17)
Stage 2 (involvement of two regions)†	27 (16)
Stage 3 (involvement of three regions)†	61 (36)
Stage 4a‡ (need for gastric tube)	2 (1)
Stage 4b‡ (need for non-invasive ventilation)	50 (30)
MiToS staging system, n (%)	
Stage 0 (zero functional domains lost)§	122 (71)
Stage 1 (one functional domain lost)§	18 (10)
Stage 2 (two functional domains lost)§	10 (6)
Stage 3 (three functional domains lost)§	8 (5)
Stage 4 (four functional domains lost)§	14 (8)
FT9 staging system, n (%)	
Stage 0 (zero ALSFRS-R subscores ≤9)‖	25 (15)
Stage 1 (one ALSFRS-R subscore ≤9)‖	36 (21)
Stage 2 (two ALSFRS-R subscores ≤9)‖	23 (13)
Stage 3 (three ALSFRS-R subscores ≤9)‖	42 (24)
Stage 4 (four ALSFRS-R subscores ≤9)‖	46 (27)

*Data represent the disease status of patients according to King's (n=169), MiToS (n=172) and FT9 (n=172) ALS staging systems; ALS staging system definitions are as described in Thakore NJ, et al. 2020.[7] †Regions are bulbar, arm or leg. ‡Stage 4a and Stage 4b are overlapping stages; Stage 4b is used when gastric tube and non-invasive ventilation are required. §Domains are movement, swallowing, communicating and breathing. ‖Subscores relate to bulbar, fine motor, gross motor and respiratory function.

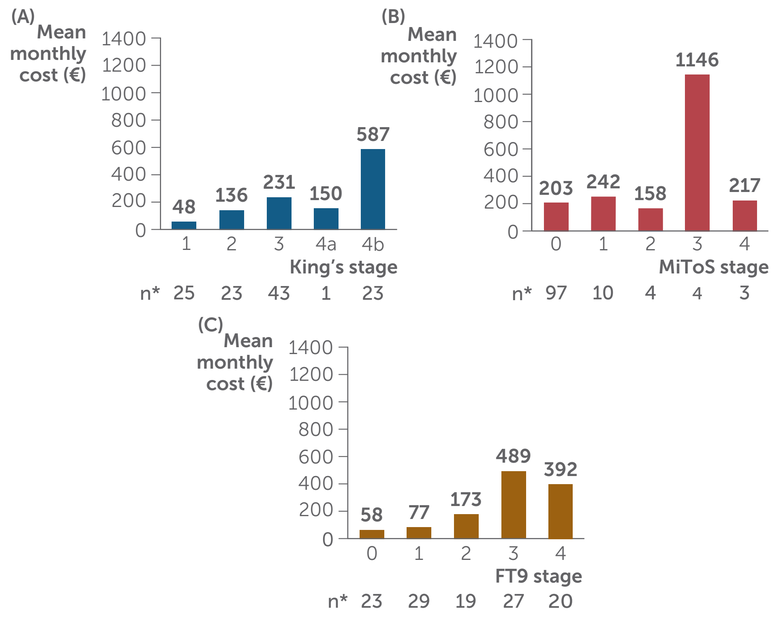
Treatment-related out-of-pocket costs

- For all staging systems, mean monthly treatment-related out-of-pocket costs were generally higher for patients at more severe disease stages than for patients at less severe disease stages (**Figure 1**)
- The categories with the highest treatment-related out-of-pocket costs reflect a pattern toward more severe disease status
  - Costs for professional caregivers were highest for MiToS Stage 3: €500, followed by FT9 Stage 3: €288 and King's Stage 4b: €283 (**Supplemental Table 1**)
  - Prescription medicines were highest for MiToS Stage 3: €336, followed by King's Stage 4b: €146 and FT9 Stage 4: €118 (**Supplemental Table 1**)

Conclusions

- These results suggest that assessment of the socioeconomic burden of ALS should take into consideration inter- and intra-variability of ALS staging systems in classifying disease status
- Despite low patient numbers in some stages of the ALS staging systems, some patterns in out-of-pocket costs were observed
  - Treatment-related out-of-pocket costs generally increased as ALS severity progressed, but the staging system influenced the magnitude of the difference
  - Increases in non-treatment-related out-of-pocket costs fluctuated both within and across ALS staging systems

Figure 1 Treatment-related out-of-pocket costs for patients staged by (A) King's, (B) MiToS and (C) FT9 staging systems



*n numbers represent prescription medicines category only; n numbers and out-of-pocket costs for all treatment-related categories are presented in Supplemental Table 1.

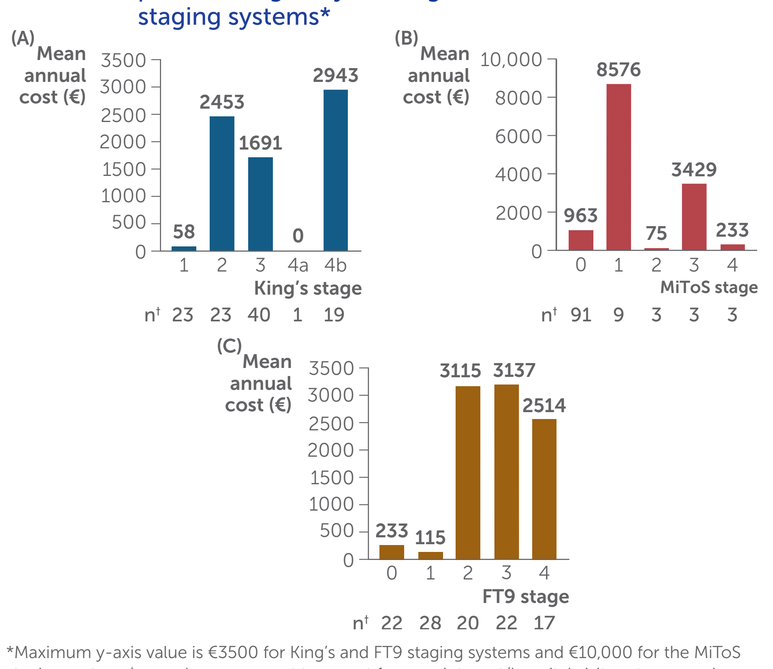
Non-treatment-related out-of-pocket costs

- Mean annual non-treatment-related out-of-pocket costs varied as disease severity increased in each staging system (**Figure 2**)
- The categories with the highest mean annual non-treatment-related out-of-pocket costs were transport modifications and home modifications:
  - Costs for transport modifications were highest for MiToS Stage 1: €3485, followed by FT9 Stage 3: €1306 and King's Stage 4b: €1000 (**Supplemental Table 2**)
  - Costs for home modifications were highest for MiToS Stage 1: €4845, followed by FT9 Stage 2: €2153 and King's Stage 2: €1734 (**Supplemental Table 2**)

Study limitations

- Data may have been impacted by responder bias with respect to both the clinician and patient/caregiver study participation
- Small sample size for some stages of the ALS staging systems introduces a level of uncertainty for the results
- Non-treatment-related out-of-pocket costs associated with care home/assisted living may not reflect the real-world setting owing to low patient numbers in this study

Figure 2 Non-treatment-related out-of-pocket costs for patients staged by (A) King's, (B) MiToS and (C) FT9 staging systems*



*Maximum y-axis value is €3500 for King's and FT9 staging systems and €10,000 for the MiToS staging system. †n numbers represent transport for appointment/hospital visits category only; n numbers and out-of-pocket costs for all non-treatment-related categories are presented in Supplemental Table 2.

Abbreviations: ALS, amyotrophic lateral sclerosis; ALSFRS-R, revised ALS functional rating scale; BMI, body mass index; FT9, Fine'til 9; MiToS, Milano-Torino staging; SD, standard deviation.

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