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Impact of Amyotrophic Lateral Sclerosis Disease Progression on Healthcare Resource Use and Health-Related Quality of Life Using MiToS Staging and ALSFRS-R: Analysis of a Real-World Cross-Sectional Survey

Paulos Gebrehiwet[1], Johan Brekke[1], Stacy Rudnicki[1], Jennifer Mellor[2], Jack Wright[2], Lucy Earl[2], Nathan Ball[2], Halima Iqbal[2], Owen Thomas[2]

[1]Cytokinetics, Incorporated, South San Francisco, CA, USA; [2]Adelphi Real World, Bollington, UK

INTRODUCTION

- Amyotrophic lateral sclerosis (ALS) is a progressive neurodegenerative disease with mean survival of 36 months from symptom onset.[1,2]
- ALS is a rapidly progressive disease associated with substantial medical costs and a decline in health-related quality of life (HRQoL).[3,4]

Objective

- The current study evaluated healthcare resource use (HCRU) and HRQoL in people living with ALS according to:
  - Milano-Torino staging (MiToS).
  - ALS progression rate.

METHODS

Data source

- Data for this analysis were drawn from the Adelphi ALS Disease Specific Programme™ (DSP).
- This multinational, cross-sectional survey was conducted in the US and Europe (France, Germany, Italy, Spain, UK), with surveys collected between July 2020 and March 2021.
- The DSP methodology details have been previously published.[5]
  - Briefly, neurologists completed a one-timepoint questionnaire on demographics, characteristics, and HCRU for patients with ALS under their care, and patients (or caregivers) completed the EQ-5D-5L.

Analyses

- The cohort was analyzed according to MiToS staging, a tool measuring ALS progression from stages 0–5 (with stages 0–4 reflecting 0–4 functional domains lost; stage 5 is death).
  - Mean and 95% CI for HCRU (ALS-related hospitalization and total durable medical equipment [DME] use) and HRQoL (EQ-5D-5L, US value set) were evaluated for each stage.
  - Hospitalization duration, healthcare provider consultations, and professional caregiver support were also reported.
  - DME included mobility, communication, therapeutic, and respiratory aids.
- The analysis was repeated using tertiles of scores on the ALS functional rating scale-revised (ALSFRS-R) as a measure of disease progression rate.
  - People with ALS were classified as slow, intermediate, and fast progressors based on their calculated disease progression rate (48 minus ALSFRS-R total score divided by symptom duration in months).

Statistical methods

- Comparisons of patient characteristics and outcomes across groups were conducted using analysis of variance (ANOVA), chi-squared, Fisher's exact, or *t*-tests (*P*<0.05 considered significant).

RESULTS

- Participant characteristics for the overall group are shown in **Table 1**, and characteristics according to disease progression rate are presented in **Supplementary Table S1***.

Table 1. Participant characteristics

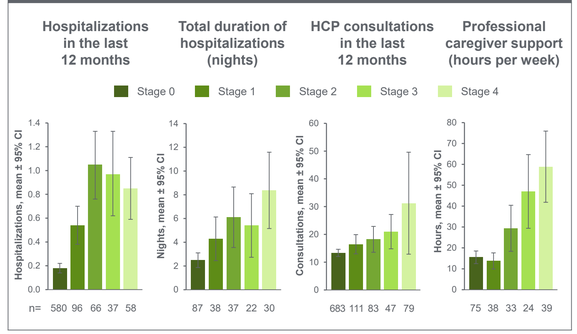
Characteristic	Overall population (N=1003)
Age, mean (SD), y	61.4 (11.0)
Male, n (%)	629 (62.7)
White, n (%)	893 (89.1)
BMI, mean (SD), kg/m^2	24.3 (4.1)
ALSFRS-R total score, mean (SD)	33.2 (12.0)
Time since diagnosis, mean (SD), months	19.0 (22.0)
Time since 1st symptom, mean (SD), months	27.4 (27.4)
ALS site of onset: bulbar, n (%)	190 (19.1)
On riluzole alone, n (%)	638 (63.6)
On edaravone alone, n (%)[a]	104 (10.4)
On riluzole plus edaravone, n (%)[a]	69 (6.9)

[a] Edaravone is not approved in Europe, where the majority of people in this survey were located.
ALSFRS-R, ALS functional rating scale-revised; BMI, body mass index

HCRU and HRQoL

- Overall, people in higher MiToS stages (≥1) and those with faster disease progression rates had increased HCRU and decreased HRQoL (**Figures 1–4**, **Supplementary Tables S2–S7***).

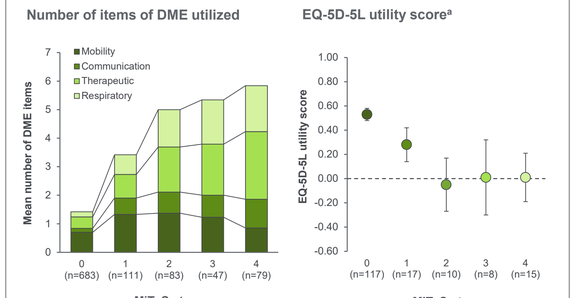
Figure 1. HCRU by MiToS stage in the overall population



HCP, healthcare professional; HCRU, healthcare resource use; MiToS, Milano-Torino staging

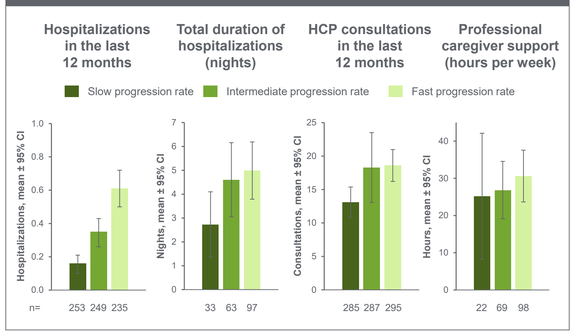
- Results for the subgroups from the US and Europe were broadly consistent with the overall population, reflecting increased HCRU and decreased HRQoL at higher MiToS stages and with faster progression (**Supplementary Tables S2–S7**, **Supplementary Figures S1** and **S2***).
  - When comparing the regions, the US subgroup had fewer ALS-related hospitalizations, higher total DME utilization, and higher HRQoL than those in Europe (**Supplementary Figures 1** and **2***).

Figure 2. DME utilization and HRQoL by MiToS stage in the overall population



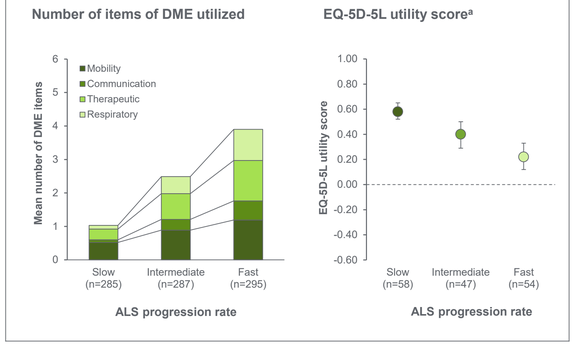
[a] Utility results were from US-based value set for the EQ-5D-5L.
DME, durable medical equipment; HRQoL, health-related quality of life; MiToS, Milano-Torino staging

Figure 3. HCRU by ALS progression rate in the overall population



ALS, amyotrophic lateral sclerosis; HCP, healthcare professional; HCRU, healthcare resource use

Figure 4. DME utilization and HRQoL by ALS progression rate in the overall population



[a] Utility results were from US-based value set for the EQ-5D-5L. ALS, amyotrophic lateral sclerosis; DME, durable medical equipment; HRQoL, health-related quality of life; MiToS, Milano-Torino staging

Limitations

- Data from the survey are cross-sectional, with limited information about individual patient journeys or disease history.
- Participation is affected by the willingness to complete the survey and may not reflect a random sample of neurologists or people living with ALS.

CONCLUSIONS

- With faster rates of disease progression and with advancement into later MiToS stages, HCRU increased and HRQoL deteriorated in people living with ALS.
- Effective treatments that lower HCRU and delay HRQoL deterioration are needed to reduce the economic and humanistic burden for people living with ALS as well as healthcare systems.

References

1. Chiò A, et al. *J Neurol Neurosurg Psychiatry* 2015;86:38-44. **2.** Steinbach R, et al. *J Clin Med* 2020;9:2873. **3.** Meng L, et al. *Amyotroph Lateral Scler Frontotemporal Degener* 2018;19:134-42. **4.** Wei Q-Q, et al. *Health Qual Life Outcomes* 2021;19:1-8. **5.** Anderson P, et al. *Curr Med Res Opin* 2008;24:3063-72.

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*Congress attendees can access supplementary information via the virtual event platform