

Characterizing Real-World Disease Burdens in Cardiac Amyloidosis: A Descriptive Study on Social, Economic, and Access-Related Impacts on Patients in Germany and Spain



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INTRODUCTION

- Transthyretin amyloid cardiomyopathy (ATTR-CM) is a progressive disease characterized by accumulation of misfolded transthyretin amyloid in the myocardial extracellular space.^{1,2}
- Patients with ATTR-CM report debilitating symptoms and impaired health-related quality of life (HRQoL), particularly those with more advanced disease.^{3,4} Early diagnosis and treatment of ATTR-CM are important to improve patient outcomes and quality of life (QoL).^{5,6}
- We sought to describe the disease burden and HRQoL of real-world patients with ATTR-CM in Germany and Spain.

METHODS

Study objectives

- To describe HRQoL, symptom burden, and self-reported financial, informational, psychological, and access burdens in patients with ATTR-CM in Germany and Spain.

Study design

- This was a descriptive, secondary analysis of survey data from the Adelphi Real World ATTR I Disease Specific Programme (DSP)TM, a cross-sectional survey of cardiologists and their consulting patients with ATTR-CM, which included elements of retrospective data collection.⁷
- German and Spanish cardiologists treating ≥1 patient with ATTR-CM were asked to fill out a Patient Record Form (PRF) for 1–10 consecutively consulted patients aged ≥18 years with a confirmed diagnosis of ATTR-CM. Data were collected within the context of routine care visits from September 2024 to January 2025.
- Participating cardiologists completed a physician survey (including questions on their study site and professional characteristics), as well as PRFs for each patient included in the DSP. At the time of consultation, patients were also invited to complete voluntary paper-based surveys.
- Survey instruments included questions on patient characteristics, diagnostic/treatment experiences, symptoms, disease burdens, and HRQoL.

- HRQoL was assessed using EuroQoL 5-dimension 5-level (EQ-5D-5L) and EuroQoL visual analogue scale (EQ-VAS) questionnaires, alongside descriptive information collected in the DSP.^{8–10}

Analysis

- Results were descriptively reported and stratified by country. Sample statistics were reported using frequencies and percentages (categorical variables), or mean, standard deviation (SD), median, and range (numeric variables).
- For EQ-5D-5L and EQ-VAS, aggregate scores were computed as specified by the questionnaire documentation.
- Analyses were performed using Microsoft Excel and IBM® SPSS® Data Collection Survey Reporter v7.5.

RESULTS

Patient characteristics

- A total of 61 cardiologists (Germany: 30; Spain: 31) provided data on 120 patients from each country (Table 1).

Table 1. Patient demographics (all patients included in the DSP)

Characteristics	All patients (N=240)	Germany (n=120)	Spain (n=120)
Mean age, years (SD)	71.8 (12.4)	67.2 (12.0)	76.4 (11.0)
Male, n (%)	188 (78.3)	98 (81.7)	90 (75.0)
Phenotype, n (%)	N=240	N=120	N=120
ATTR-CM only	224 (93.3)	112 (93.3)	112 (93.3)
Mixed phenotype (with ATTR-PN)	16 (6.7)	8 (6.7)	8 (6.7)
Genotype, n (%)	N=205	N=102	N=103
Wild-type	100 (48.8)	48 (47.1)	52 (50.5)
Hereditary	48 (23.4)	27 (26.5)	21 (20.4)
Not tested	57 (27.8)	27 (26.5)	30 (29.1)
Missing†	35	18	17
NYHA functional class at survey, n (%)	N=240	N=120	N=120
I	27 (11.3)	11 (9.2)	16 (13.3)
II	155 (64.6)	74 (61.7)	81 (67.5)
III	54 (22.5)	34 (28.3)	20 (16.7)
IV	4 (1.7)	1 (0.8)	3 (2.5)
Treatment status, n (%)	N=240	N=120	N=120
Receiving treatment at survey	179 (74.6)	112 (93.3)	67 (55.8)
Previous treatment but not at survey	3 (1.3)	2 (1.7)	1 (0.8)
No previous treatment	58 (24.2)	6 (5.0)	52 (43.3)
Employment status, n (%)	N=237	N=120	N=117
Retired	198 (83.5)	94 (78.3)	104 (88.9)
Working full-time	17 (7.2)	10 (8.3)	7 (6.0)
Working part-time	10 (4.2)	5 (4.2)	5 (4.3)
On long-term disability/sick leave	6 (2.5)	5 (4.2)	1 (0.9)
Unemployed	4 (1.7)	4 (3.3)	0
On short-term disability/sick leave	1 (0.4)	1 (0.8)	0
Homemaker	1 (0.4)	1 (0.8)	0
Missing†	3	0	3
Patient not working due to ATTR-CM, n (%)	N=210	N=107	N=103
Yes	18 (8.6)	14 (13.1)	4 (3.9)

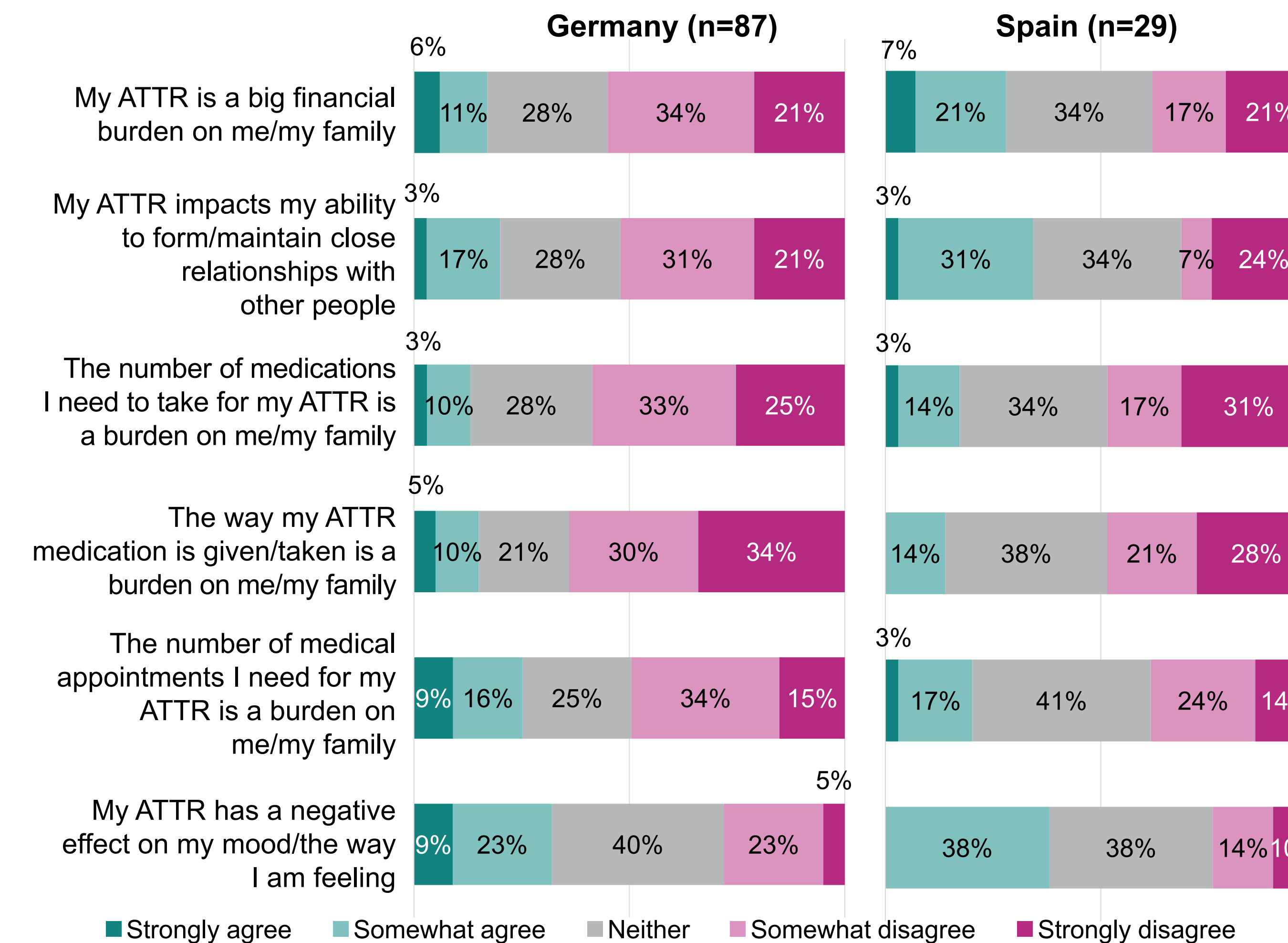
†Missing n numbers not included in N numbers for those characteristics. ATTR-CM, transthyretin amyloid cardiomyopathy; ATTR-PN, transthyretin amyloid polyneuropathy; DSP, Adelphi Real World ATTR I Disease Specific Programme; NYHA, New York Heart Association; SD, standard deviation.

- Overall, 118 of 240 patients (Germany: 89; Spain: 29) completed optional surveys. Among those surveyed, the mean age of patients was 66.5 years in Germany and 72.0 years in Spain. Most patients were male (Germany: 86.5%; Spain: 82.8%).

Patient burden of disease and impact on QoL

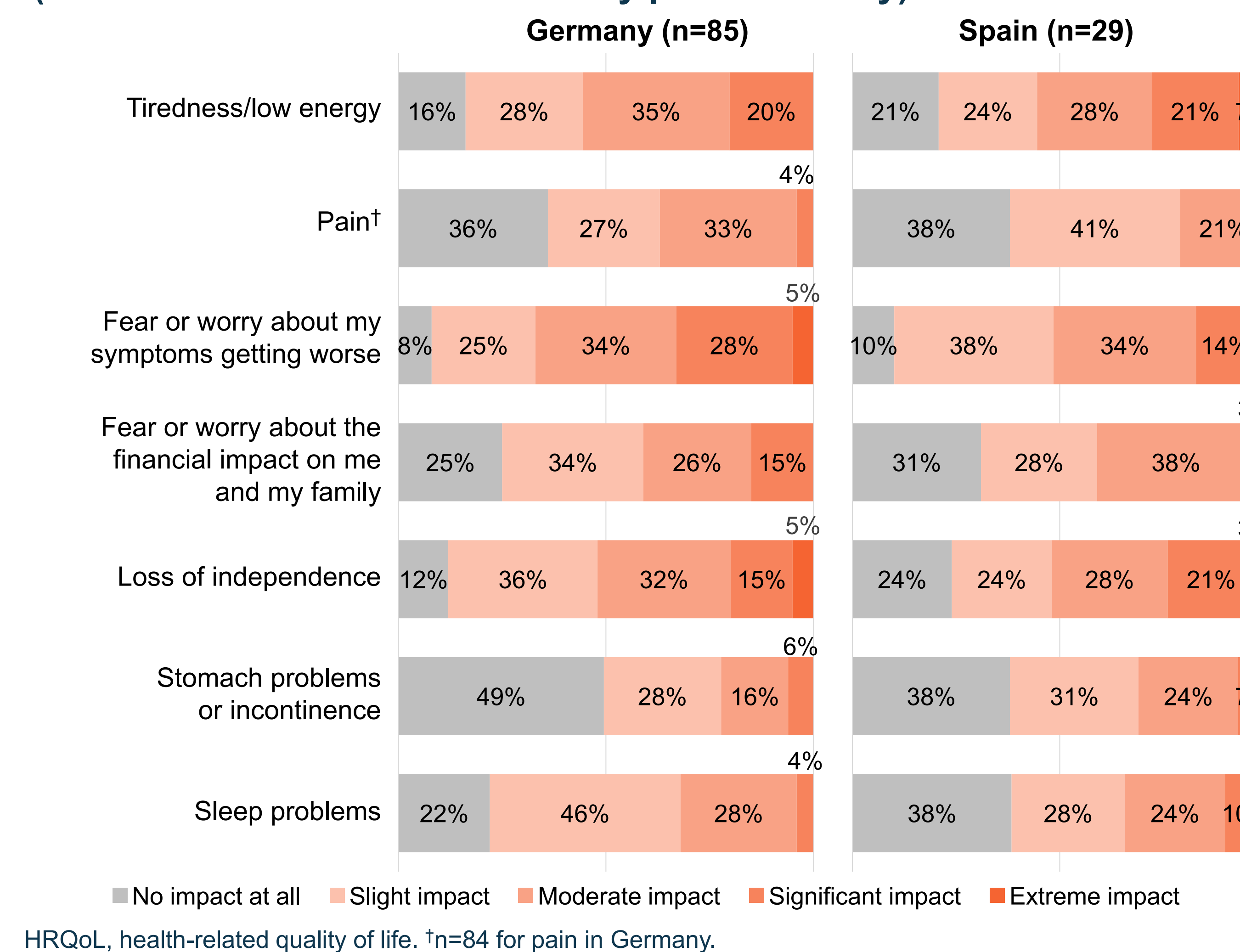
- Patients reported the highest burden (moderate-to-severe impact on QoL) in association with anxiety related to worsening symptoms (63%), fatigue (55%), loss of independence (52%), and fear of financial impacts (41%).
- While more patients in Germany than Spain faced self-reported burdens, trends in the ranking of symptoms were generally consistent across countries (Figures 1 and 2).
- Overall, 72% of patients in Germany and 69% of patients in Spain reported having no or slight problems with each of the five EQ-5D domains, with mean index scores of 0.82 in Germany and 0.80 in Spain.
- The mean (SD) EQ-VAS scores were 61.8 (16.3) in Germany and 63.4 (20.3) in Spain.

Figure 1. Patient-reported burdens associated with ATTR (results derived from the voluntary patient survey)



ATTR, transthyretin amyloidosis.

Figure 2. Patient-reported impact of symptoms on HRQoL (results derived from the voluntary patient survey)

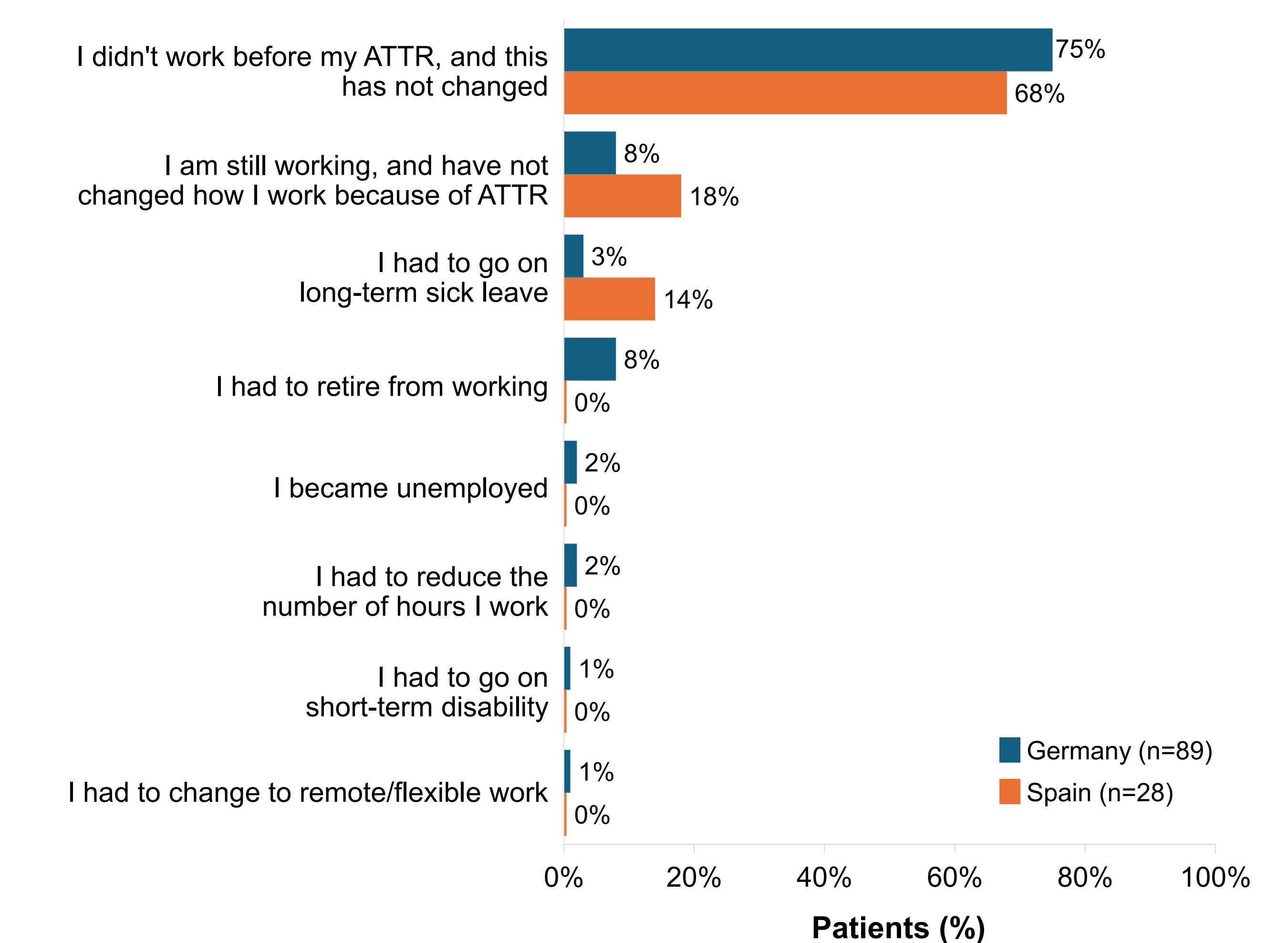


HRQoL, health-related quality of life. †n=84 for pain in Germany.

Economic burden of disease

- Among patients completing the voluntary survey, 74% were not working at the time of ATTR-CM diagnosis (Germany: 75%; Spain: 68%; Figure 3). This may be attributable to advanced age.
- According to survey, approximately 10% of patients were employed at diagnosis and experienced no change in employment after ATTR-CM diagnosis (Germany: 8%; Spain: 18%), while 16% reported a change in employment status/conditions (Germany: 17%; Spain: 14%) (Figure 3).
- Many physicians reported that fear/worry about the financial impacts of ATTR-CM negatively affected patients' QoL (overall: 67%; Germany: 69%; Spain: 64%).
- In both countries, the highest out-of-pocket expenditure was on travel to appointments, incurring a median (interquartile range [IQR]) monthly cost of 15.0 (10.0–20.0) EUR in Germany and 14.0 (0–42.5) EUR in Spain.

Figure 3. Patient-reported impact of ATTR on employment (results derived from the voluntary patient survey)



ATTR, transthyretin amyloidosis.

Access-related impact of disease

- While 64% of patients reported no discomfort seeking help for their condition, the most commonly reported access burdens were long wait times for appointments (Germany: 13%; Spain: 31%), transportation issues (Germany: 13%; Spain: 27%), and patient reluctance for further testing (Germany: 8%; Spain: 8%).

CONCLUSIONS

- Patients with ATTR-CM reported the highest burden in association with anxiety relating to worsening symptoms, fatigue, loss of independence, and worry about the financial impact of ATTR-CM.
- While most patients with ATTR-CM in Germany and Spain were no longer employed at the time of diagnosis, psychological symptoms related to ATTR-CM progression, challenges accessing care, and loss of autonomy are key issues continuing to affect patients.
- Improving healthcare provider awareness of ATTR-CM may enable earlier access to care and treatment, which could help alleviate the societal, economic, and access-related burdens for patients.

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