Real-World Treatment Patterns and Preferences in Patients With Transthyretin Amyloid Cardiomyopathy From Germany and Spain

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INTRODUCTION

- Transthyretin amyloid cardiomyopathy (ATTR-CM) is a progressive disease characterised by the accumulation of misfolded amyloid fibrils in the myocardial extracellular space.[1,2]
- The buildup of amyloid in ATTR-CM leads to cardiac dysfunction, heart failure, and repeated hospitalisations.[2–4] The condition is fatal, with a median survival of 3–4 years after disease onset, when left untreated.[3]
- Early diagnosis and treatment of ATTR is important to stabilise amyloid deposition, prevent irreversible organ damage and improve patient outcomes.[5,6]
- The first disease-modifying treatment indicated for ATTR-CM – the transthyretin (TTR) stabiliser tafamidis – was granted market authorisation in 2020.[7] In 2025, a second TTR stabiliser, acoramidis, received market authorisation in Europe based on positive results from the ATTRibute-CM trial (NCT03860935).[8] Acoramidis is an oral TTR stabiliser that achieves near-complete (≥90%) TTR stabilisation.[9]
- Despite the evolving therapeutic landscape, there are limited data on real-world treatment patterns and preferences in patients with ATTR-CM.
- Consequently, our study set out to describe the real-world treatment history and preferences of patients with ATTR-CM in Germany and Spain.

METHODS

Study objectives

- We described the treatment history and treatment preferences in a sample of 240 real-world patients with ATTR-CM in Germany and Spain.

Study design

- This was a descriptive analysis of survey data from the Adelphi Real World ATTR I Disease Specific Programme (DSP)™, a cross-sectional survey of cardiologists and their consulting patients with ATTR-CM, with elements of retrospective data collection.[10] The study design is shown in **Figure 1**.
- German and Spanish cardiologists treating at least one patient with ATTR-CM were asked to include patients who were ≥18 years of age and had a confirmed diagnosis of ATTR-CM from a cardiologist within the context of routine care visits from September 2024 to January 2025.
- Participating cardiologists completed a physician survey that captured information on their professional and demographic characteristics, and attitudes/perceptions regarding disease management and treatment options.
- Each cardiologist completed electronic patient record forms for 1–10 consecutively consulted eligible patients which captured data on sociodemographic profile, diagnostic pathways, disease characteristics, treatment history and preferences. At the time of consultation, patients were invited to complete voluntary paper-based surveys, which captured information on patient characteristics, treatment and diagnostic history, health-related quality of life, and symptoms.

Analysis

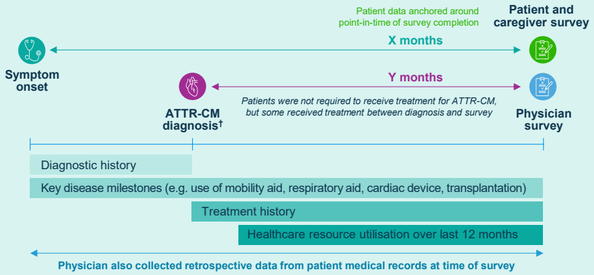
- Analyses were descriptive, with results stratified by country. Sample statistics were reported using frequencies and percentages for categorical variables, and mean, standard deviation (SD), median, and range for numeric variables.
- Analyses were performed using Microsoft Excel and IBM® SPSS® Data Collection Survey Reporter v7.5.

Figure 1. Study overview

Eligibility:

- Physicians from Germany and Spain were eligible if their primary specialty was cardiology, and they were responsible for treating at least one patient with ATTR-CM
- Patients were eligible for inclusion if they were aged ≥18 years and had a confirmed diagnosis of ATTR-CM or a mixed ATTR-CM/ATTR-PN phenotype

Completeness of timeline varies by availability of patient information



†Or diagnosis of mixed ATTR-CM/ATTR-PN phenotype.
ATTR-CM, transthyretin amyloid cardiomyopathy; ATTR-PN, transthyretin amyloid polyneuropathy.

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RESULTS

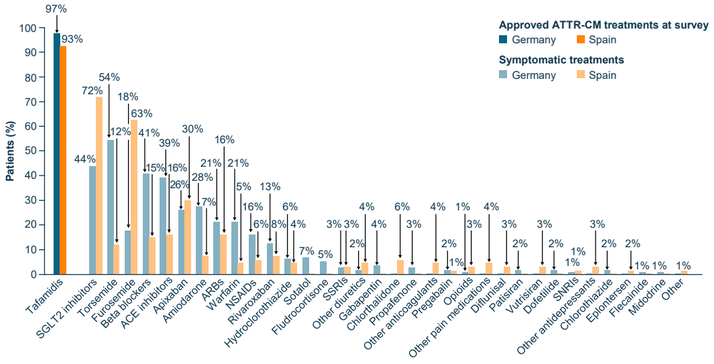
Patient characteristics

- A total of 61 cardiologists (Germany: 30, Spain: 31) provided data on 240 patients with ATTR-CM (Germany: 120, Spain: 120). A total of 118 patients (Germany: 89, Spain: 29) provided information on treatment preferences.
- The mean (SD) age of patients was 67 (12) years in Germany and 76 (11) years in Spain. Overall, 22% of patients were female (Germany: 18%, Spain: 25%).

Treatment history

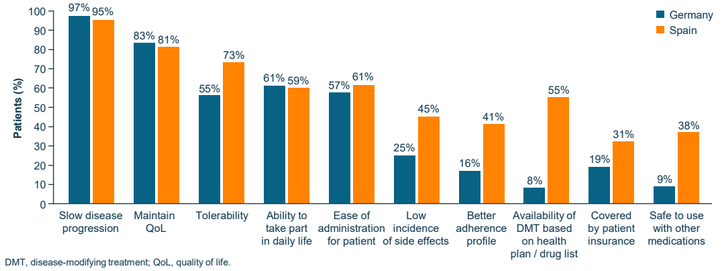
- Among the 120 patients included in each country, 93% (Germany) and 56% (Spain) were undergoing disease-modifying therapy or symptomatic treatments at the time of survey.
- Mean (SD) time from diagnosis to treatment initiation: 7 (20) months in Germany; 10 (15) months in Spain.
- Only one drug – tafamidis – was approved for the treatment of ATTR-CM at the time of survey. Of patients on treatment at survey, almost all were taking tafamidis (Germany: 97%; Spain: 93%) (**Figure 2**).
- Among treated patients, the most common symptomatic treatments used were sodium-glucose cotransporter 2 inhibitors (Germany: 44%; Spain: 72%), torsemide (Germany: 54%; Spain: 12%), and furosemide (Germany: 18%: Spain: 63%).

Figure 2. Most commonly prescribed treatments for patients in Germany (n=112) and Spain (n=67)



Tafamidis could be Vyndaqel or Vyndamax. Patisiran and vutrisiran were approved for ATTR-PN at the time of the survey. ACE, angiotensin-converting enzyme; ARB, angiotensin receptor blocker; ATTR-CM, transthyretin amyloid cardiomyopathy; ATTR-PN, transthyretin amyloid polyneuropathy; NSAID, non-steroidal anti-inflammatory drug; SGLT2, sodium-glucose cotransporter 2; SNRI, serotonin norepinephrine reuptake inhibitor; SSRI, selective serotonin reuptake inhibitor.

Figure 3. Top 10 reasons for the selection of DMT among providers in Germany (n=110) and Spain (n=64)



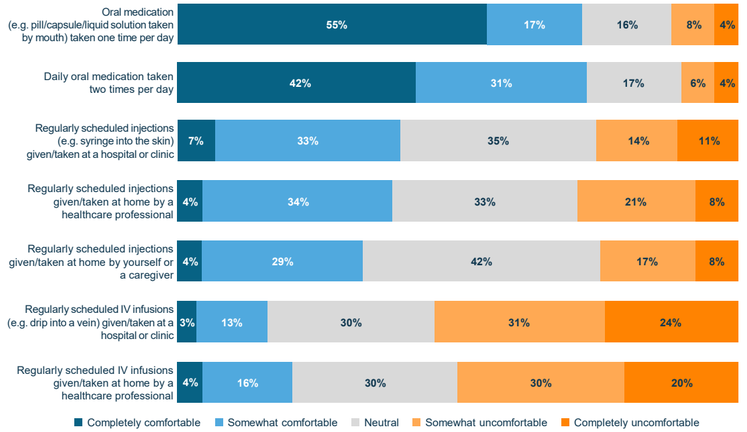
DMT, disease-modifying treatment; QoL, quality of life.

- Among patients prescribed treatment, two in Germany (2%) and one in Spain (1%) discontinued tafamidis.
- The most common reason for patients never having been prescribed treatment was patient choice in Germany (67%) and patient ineligibility in Spain (38%).
- Slowing disease progression was the most common driver of treatment selection among providers (Germany: 97%; Spain: 95%; **Figure 3**).

Treatment preferences

- Providers reported feeling very satisfied with the effects in 66% of cases (Germany: 78%; Spain: 48%). In both countries, poor outcomes related to disease progression (35%), quality of life (30%), and symptom management (30%) were the most common reasons for lack of complete satisfaction with treatment.
- Patient preferences around hypothetical treatments are summarised in **Figure 4**. Almost twice as many patients felt completely or somewhat comfortable with (once or twice daily) oral medications as compared with regularly scheduled injections.

Figure 4. Patient-reported comfort levels with receiving hypothetical treatments for ATTR-CM associated with different routes of administration (n=118)



ATTR-CM, transthyretin amyloid cardiomyopathy; IV, intravenous.

CONCLUSIONS

- Treatment varied in Germany and Spain, with over 90% of patients on treatment in Germany, but only around half of patients on treatment in Spain at the time of survey.
  - This likely reflects differences in country-specific factors affecting access to treatment. For example, access to tafamidis was delayed in Spain compared with Germany, due to approval complexities. Access to tafamidis in Spain is also region-specific and based on strict age and disease criteria.[11]
- Most patients on treatment were taking tafamidis, the only approved treatment for ATTR-CM at the time of survey. Slowing disease progression was the most common driver of treatment choice.
- More patients felt completely or somewhat comfortable with receiving once/twice daily oral medications as compared with regular injections or IV infusions, reinforcing the value of oral therapeutics in ATTR-CM.