Treatment Patterns and Preferences in Transthyretin Amyloid Cardiomyopathy in the United States: Results From a Real-World Survey

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BACKGROUND

- **Transthyretin (TTR) amyloid cardiomyopathy (ATTR-CM) is a progressive, life-threatening disease caused by destabilization of TTR tetramers into misfolded monomers that aggregate into toxic amyloid fibrils, which deposit throughout the body, predominantly in the heart[1]**
- **The therapeutic landscape for ATTR-CM in the United States (US) continues to evolve since the first oral TTR stabilizer, tafamidis, was approved in May 2019.[2] Since then, another TTR stabilizer and a new class, TTR silencers, have emerged:**
  - **Acoramidis, an oral TTR stabilizer, was approved in November 2024[3]**
  - **Vutrisiran, an injectable TTR silencer, was approved in March 2025[4]**
- **The role of patients in determining treatment choice is also evolving**
  - **Recent guidance emphasizes shared decision-making (SDM) that incorporates patient preferences and priorities when developing a treatment plan for ATTR-CM[5,6]**
- **As more treatments become available, understanding physician and patient preferences is critical to help inform treatment selection**
- **Here, we describe real-world ATTR-CM treatment patterns, physician satisfaction, and patient administration preferences captured in the Adelphi ATTR Disease Specific Programme™ (DSP) Survey from August 2024 to February 2025**

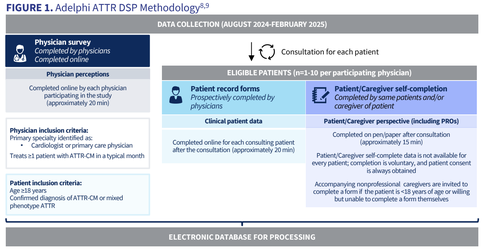
METHODS

- The Adelphi ATTR DSP is a cross-sectional survey of physicians who treat ATTR-CM and patients diagnosed with ATTR-CM in the US
- Physician-reported demographics, prescribed treatments, treatment satisfaction, and patient-reported treatment administration preferences were analyzed descriptively

Study Design and Data Source

- This was a secondary analysis of the Adelphi ATTR DSP, which consisted of linked physician and patient/caregiver cross-sectional surveys with elements of retrospective data collection
- The DSP methodology has previously been described,[7,8] validated,[9] demonstrated to be representative and consistent over time,[10] and implemented globally across many different disease areas[7] (**Figure 1**)
  - Each physician completed a patient record form for up to their next 10 patients
  - Each patient was then invited to fill out a voluntary self-completion form (the patient could choose not to participate)
  - Participating patients were treated in accordance with usual medical practice; no additional assessments or tests were conducted because of their participation
  - Physician-reported patient data were linked to patient-reported patient data via unique, pseudonymized physician- and patient-coded numbers
- The Adelphi ATTR DSP was conducted between August 2024 and February 2025 in the US

FIGURE 1. Adelphi ATTR DSP Methodology[8,9]



ATTR, transthyretin amyloidosis; ATTR-CM, transthyretin amyloid cardiomyopathy; PRO, patient-reported outcome.

Inclusion Criteria

- Eligible physicians were cardiologists or primary care physicians who had treated ≥4 patients with ATTR-CM
  - Patients were eligible if they were treated by a physician participating in the survey, were adult, and had a confirmed diagnosis of ATTR-CM or mixed phenotype (defined as ATTR-CM + ATTR peripheral neuropathy [ATTR-PN] noted in their medical record)
  - Patients with ATTR-PN were excluded

CONCLUSIONS

- **Physicians were not fully satisfied with treatment for >55% of their patients with ATTR-CM, highlighting unmet treatment needs in the period before newer therapies were approved and reached routine clinical use (August 2024-February 2025)**
- **Despite the majority of patients having New York Heart Association class I or II staged disease, a time in the disease history when treatment initiation is critical for optimal long-term benefits, many patients elected not to begin treatment, representing a continued unmet need for patient counseling in the era when SDM is being encouraged[5,6]**
- **The majority of patients (74%) reported some degree of dissatisfaction with their current treatment; all (100%) were most comfortable with oral ATTR-CM therapy**
- **These findings provide real-world insight into patient and physician preferences and priorities for ATTR-CM treatment, which may facilitate SDM and the selection of therapy**
- **Further research is needed to understand patient and physician preferences as more therapeutic options become available for treating ATTR-CM**

METHODS

Study Measures: Demographic and Clinical Characteristics

- Physician surveys, physician-populated patient record forms, and patient self-completion forms captured patient and physician demographics and patient clinical characteristics and preferences

Objectives

- To describe treatment patterns and associated outcomes, including:
  - Frequency of prescription for the proportion of patients prescribed ATTR-CM treatment at the time of the survey. Among those not prescribed treatment at the time of the survey, the reasoning was captured
  - Levels of satisfaction with current treatment, as reported by both physicians and patients, as well as reasoning for dissatisfaction
- To describe patient-reported levels of comfort receiving treatment across modes of administration

Analysis

- All statistical analyses were descriptive in nature (ie, there was no hypothesis testing)

RESULTS

Physicians and Patients

- Of the 34 participating physicians, the majority were general cardiologists or heart failure specialists (55.8%). Among their overall ATTR caseload, physicians consulted a mean (SD) of 21.6% (33.4%) patients in an academic hospital, 7.1% (14.0%) in a community hospital, and 71.3% (34.0%) in an office
- Among 256 patients treated by the participating physicians, the mean (SD) age was 63.9 y (13.2), 60.9% were male, and 55.9% were White. ATTR phenotype was 79.7% cardiac and 20.3% mixed. Median (interquartile range) disease duration (n=164) was 1.9 y (1.3-3.0) (**Table 1**); 58 of these patients provided self-reported data

TABLE 1. Patient Demographic and Clinical Characteristics

Characteristic	Patients	
	N	Value
Age, mean (SD), y	256	63.9 (13.2)
Biological sex,[a] n (%)	256	
Male		156 (60.9)
Female		100 (39.1)
Intersex		0
Body mass index, mean (SD), kg/m²	255	25.4 (3.9)
Race/Ethnicity,[b] n (%)	256	
White		143 (55.9)
Black or African American		81 (31.6)
South or Central American Native		13 (5.1)
East or Southeast Asian		9 (3.5)
Middle Eastern or North African		6 (2.3)
South Asian (Indian subcontinent)		3 (1.2)
American Indian, Indigenous American, or Alaska Native		1 (0.4)
Other		1 (0.4)
NYHA class at time of survey, n (%)	256	
I		56 (21.9)
II		139 (54.3)
III		51 (19.9)
IV		10 (3.9)
Patient treated at an amyloidosis clinic, n (%)	256	49 (19.1)
ATTR phenotype	256	
ATTR-CM		204 (79.7)
Mixed phenotype[c]		52 (20.3)
Disease type, n (%)	180	
Hereditary ATTR-CM		56 (31.1)
Wild-type ATTR-CM		20 (11.1)
Unconfirmed (no genetic test performed)		104 (57.8)
Diagnostic journey and disease history from onset of signs/symptoms, median (IQR), y		
Time from onset to first consultation	159	0.2 (0.0-0.3)
Time from first consultation to diagnosis	175	0.3 (0.1-0.5)
Time from diagnosis to initiation of treatment	172	0.2 (0.1-0.6)
Time from diagnosis to survey	256	1.4 (0.8-2.2)
Time from onset to survey	164	1.9 (1.3-3.0)

[a]As stated in the patient's medical record; [b]Survey question was, "How would you describe the patient?"; multiple selections were allowed. [c]Confirmed diagnosis of ATTR-CM or mixed phenotype (defined as ATTR-CM + ATTR-PN peripheral neuropathy).
ATTR, transthyretin amyloidosis; ATTR-CM, transthyretin amyloid cardiomyopathy; ATTR-PN, transthyretin amyloid polyneuropathy; IQR, interquartile range; NYHA, New York Heart Association.

RESULTS

TABLE 2. Treatment History Among Patients With ATTR-CM

Characteristic	Patients	
	N	Value
Patient has received treatment for ATTR symptoms, n (%)	256	
Yes, currently prescribed treatment		155 (60.5)
No, but has previously been prescribed treatment		17 (6.6)
No, has never received any prescribed treatment		84 (32.8)
Number of distinct treatment lines ever received	256	
Mean (SD)		0.7 (0.6)
At least 1 treatment line, n (%)		172 (67.2)
More than 1 treatment line, n (%)		14 (5.5)
Number of distinct treatment lines ever received among patients receiving treatment at time of survey	155	
Mean (SD)		1.1 (0.4)
More than 1 treatment line, n (%)		10 (6.5)
Reason why patient has never received treatment,[a] n (%)	84	
Patient does not want treatment for their ATTR		30 (35.7)
Patient is waiting to start their first treatment regimen		22 (26.2)
Patient's medical insurance did not cover the cost of treatment		17 (20.2)
Patient not eligible for treatment		17 (20.2)
Patient newly diagnosed and will be prescribed treatment at future consultation		4 (4.8)
Other (specify)		2 (2.4)
Reason why patient is not receiving treatment at time of survey,[a] n (%)	17	
Patient requested to stop treatment		9 (52.9)
Patient is waiting to start a new treatment regimen		4 (23.5)
Patient did not respond adequately to prior treatment		3 (17.6)
Patient stopped treatment due to side effects		3 (17.6)
Other (specify)		2 (11.8)
Patient's physician would have preferred to use a different disease-modifying treatment than that prescribed, n (%)	145	5 (3.4)

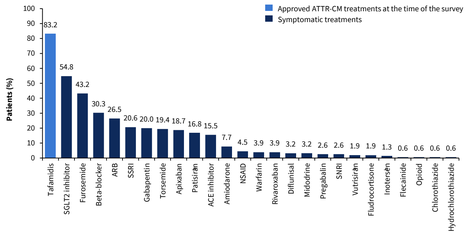
[a]Multiple selections could be made; percentages may not sum up to 100%.
ATTR, transthyretin amyloidosis; ATTR-CM, transthyretin amyloid cardiomyopathy.

- Treatment was received by 60.5% of patients, 6.6% had prior treatment, and 32.8% were treatment naïve (**Table 2**)

Treatment Patterns of US Survey Respondents (August 2024-February 2025)

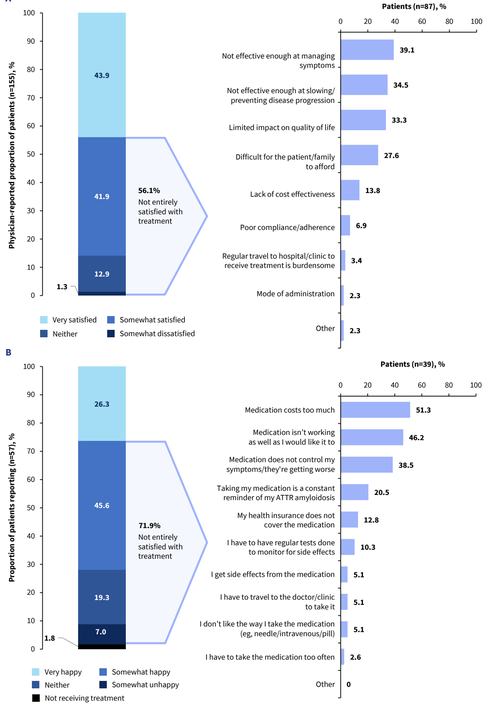
- Among patients receiving treatment at the time of the survey (N=155), the TTR stabilizer, tafamidis, was the most frequently prescribed (n=129 [83.2%]); no patients in this survey had received acoramidis (**Figure 2**)
- While no TTR silencer was approved for ATTR-CM at the time of this study, a few options were available and approved for use in ATTR-PN; some TTR silencer use was noted (16.8% patisiran, 1.9 % vutrisiran, and 1.3% inotersen) (**Figure 2**)

FIGURE 2. Treatment at Time of Survey Among Patients Treated for ATTR-CM (n=155)[a]



[a]If a patient received >1 treatment, that patient was counted once for each treatment they received. [b]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, nonsteroidal anti-inflammatory drug; SGLT2, sodium-glucose cotransporter 2; SNRI, serotonin norepinephrine reuptake inhibitor; SSRI, selective serotonin reuptake inhibitor.

FIGURE 3. Physician-Reported (A) and Patient-Reported (B) Satisfaction With Current Prescribed Treatment for ATTR-CM, and Reasons Why Physician/Patient Was Not Entirely Satisfied With This Treatment[1]

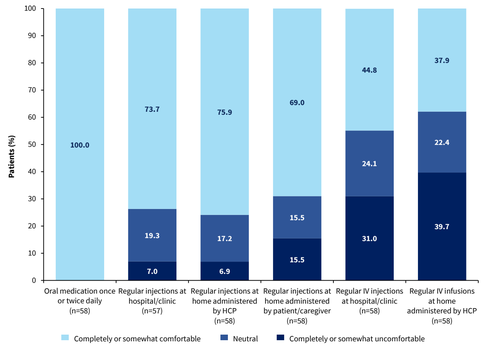


ATTR, transthyretin amyloidosis; ATTR-CM, transthyretin amyloid cardiomyopathy.

RESULTS

- Physicians were less than "very satisfied" with treatment for 56.1% of patients due to (**Figure 3A**):
  - Level of symptom management (39.1%)
  - Limited impact on disease progression (34.5%)
  - Limited impact on quality of life (33.3%)
  - Difficulty affording treatment (27.6%)
- The majority (71.9%) of patients reported being less than "very happy" with their current treatment due to (**Figure 3B**):
  - Expense (51.3%)
  - Expected better improvement (46.2%)
  - Symptoms persist or exacerbate (38.5%)

FIGURE 4. Patient-Reported Preference With Regard to Mode of Administration of Treatment for ATTR-CM



ATTR-CM, transthyretin amyloid cardiomyopathy; HCP, healthcare professional; IV, intravenous.

- All patients (100% [n=58]) were "completely" or "somewhat" comfortable with once- or twice-daily medication. Patients "completely" or "somewhat" comfortable receiving injectable treatment ranged from 69.0% to 75.9%, and receiving intravenously from 37.9% to 44.8% (**Figure 4**)

DISCUSSION

Between August 2024 and February 2025:

- One-third of patients had never been prescribed treatment for ATTR despite receiving an ATTR-CM diagnosis; of these, one-quarter were awaiting treatment start
- Common reasons for lack of treatment satisfaction among both physicians and patients involved suboptimal symptom management and high cost of treatment
- Overall, less than half of physicians were very satisfied with treatment and less than one-third of patients were very happy with treatment
- Patients were most comfortable with the prospect of oral therapy

LIMITATIONS

- This is a survey whose cross-sectional design does not allow for determination of causal relationships and is limited in sample size
- Though consecutive patient inclusion reduces selection bias, the DSP reflects a pragmatic physician sample and pseudo-random patient participation
- Retrospective reporting may introduce recall bias, mitigated by physician access to medical records and short recall periods in the patient self-completion form
- This study occurred during a time period when the second oral TTR stabilizer, acoramidis, was recently approved but not yet available nor part of routine clinical practice, and the TTR silencer, vutrisiran, had not yet been approved for ATTR-CM. Future studies are needed to assess treatment patterns and preferences in a more contemporary cohort

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ETHICAL APPROVAL: All DSPs were performed in compliance with local regulations, and patients provided their informed consent prior to participation; all data were deidentified and pseudonymized.
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