Transthyretin Amyloid Cardiomyopathy (ATTR-CM) Treatment Preferences Among Patients And Cardiologists: Findings From Two Advisory Boards In The United States

PCR 26

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Background

- Transthyretin amyloid cardiomyopathy (ATTR-CM), if left untreated, is a progressive, life-threatening disease in which misfolded transthyretin proteins deposit in the myocardium, leading to heart failure.[1]
- In recent years, several disease-modifying therapies for ATTR-CM have been approved, with important differences in their mechanism of action, clinical trials, and frequency and routes of administration.[2]
- Given the heterogeneity of available therapies and the benefits of early treatment initiation, understanding patients' and physicians' preferences is critical to support shared decision-making and optimize clinical outcomes in ATTR-CM.

Methods

Study Design

- A cross-sectional study consisting of an online survey followed by two virtual advisory board meetings. Survey findings were presented at the meetings serving as the basis for the discussions. The cardiologist meeting was held in April 2025 and the patient meeting in August 2025.
- The online surveys and meetings were conducted with 9 patients with ATTR-CM and 10 cardiologists.

Participant Enrollment Criteria

Patients:

- Age 60 or older
- Self-reported diagnosis of ATTR-CM with cardiac symptoms
- Currently receiving any approved or clinical trial treatment for ATTR-CM

Cardiologists:

- Licensed to practice medicine in the US
- Board certified in cardiology
- Currently treating patients with ATTR-CM

Recruitment Procedures

- Cardiologists were recruited by the study sponsor using purposive sampling to include multiple practice settings and representation across geographic regions.
- Patients were recruited from the Amyloidosis Support Groups patient advocacy group via their private Facebook groups. Purposive sampling was used to achieve diversity in gender, race/ethnicity, age, ATTR-CM subtype, treatment status, time since diagnosis, and geographic region.
- Each participant was contracted individually and provided consent to participate.

Measures

- The online surveys included three object-case Best Worst Scaling (BWS) exercises to elicit, separately, the relative importance of:
  - Treatment benefits (11 items for patients;15 items for cardiologists),
  - Side effects (8 items)
  - Dosing Regimens (8 items for patients; 6 items for cardiologists)
- A 100-point allocation exercise was included to assess the relative importance of treatment access challenges. The exercise entailed assigning points to each challenge.
  - The patient exercise focused on 6 barriers to obtaining ATTR treatment; the cardiologist exercise focused on 5 barriers to managing ATTR-CM and prescribing treatment.
- Discussion questions in the meetings explored reasoning behind preferences for certain attributes and the ranking of access challenges, as well as patient experiences with ATTR-CM.

Analyses

- Descriptive statistics were reported for all study variables.
- For each BWS exercise, individual-level preference scores for each attribute were calculated as the number of times the attribute was selected as "most preferred" minus the number of times it was selected as "least preferred," divided by the number of times it was shown; these scores were transformed to a 0 to 1 (most preferred) scale.
- Point allocation results were presented as the mean number of points assigned to each challenge.
- Meeting transcripts were analyzed; key themes contextualizing the quantitative findings and reasoning behind preferences were identified.

Results

Participant Characteristics

Self-reported patient and cardiologist characteristics are shown in **Table 1** and **Table 2**.

Preferences and Meeting Discussion

Treatment Benefits BWS

Figure 1 shows the mean preference scores for treatment benefits and supporting quotes from participants.

- Both patients (0.82) and cardiologists (0.92) placed highest importance on reducing mortality.
- Having lab test and imaging results that indicated no disease progression was highly important for patients (0.71). In contrast, cardiologists found this as less important (0.05), stating that the results may not correlate with disease status.
  - Since treatment primarily delays progression but does not improve functional status, cardiologists emphasized the importance of setting realistic treatment expectations with their patients.
- For cardiologists, reducing the number of urgent encounters (0.88) and the need for a heart transplant (0.73) were both highly important.

Side Effects BWS

Patients and cardiologists (not shown) generally thought treatment side effects were minimal or easily managed. Both groups mentioned that it is difficult to differentiate symptoms of the disease itself from treatment side effects.

Dosing Regimens BWS

Figure 2 shows the mean preference scores for dosing regimens and supporting quotes from participants.

- Assuming equal efficacy, patients and cardiologists preferred more convenient regimens, the specifics of which varied among patients.
- Some participants stated their belief that a more convenient regimen would translate into better adherence.

Treatment Access Challenges Point Allocation

Figure 3 shows the rankings of treatment and access challenges and supporting quotes from participants.

- Key challenges included low awareness of diagnostic testing procedures among non-specialists, resulting in diagnostic delays (46) and difficulties obtaining insurance coverage for treatment (patients = 16; cardiologists = 42).
- Patients reported distress as they often need to serve as their own healthcare advocates because some healthcare providers lack knowledge about ATTR-CM.

Objectives

This research sought to understand patient and cardiologist preferences for ATTR-CM treatments, specifically:

- Quantify and understand the importance of key treatment attributes.
- Assess challenges with ATTR-CM in accessing treatment.

Conclusions

- Because treatment priorities can differ between ATTR-CM patients and cardiologist, promoting shared decision making can help ensure patient perspectives are considered in treatment decisions, which some participants believe could translate to better adherence to treatment.
- Providers should be aware of the high emotional burden of the disease as well as limitations from extra-cardiac symptoms. There is also a need for ensuring patients understand disease progression and have realistic treatment expectations.
- Delays in diagnosis and treatment initiation point to a need for potential new solutions, such as educating non-specialists about ATTR-CM testing and criteria for referrals.

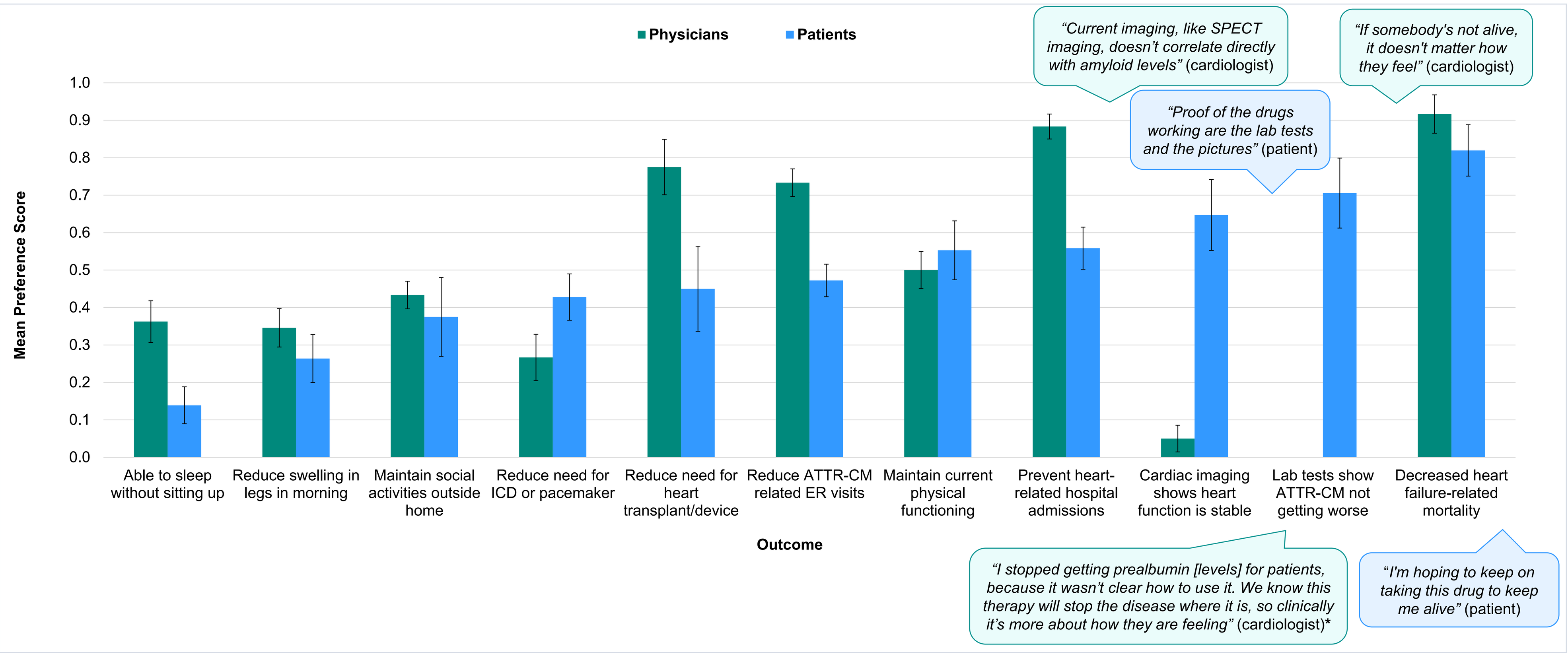
Table 1. Self-Reported Patient Characteristics

	N = 9
Age, mean years + standard deviation	69.9 + 3.06
Sex at birth, n	
Male	6
Female	3
ATTR-CM Type, n	
Variant/hereditary type	5
Wild type	4
Amyloidosis symptoms, n	
Only heart signs and symptoms	2
Both heart and nerve signs and symptoms	6
Don't know or not sure	1
Experience with treatment for ATTR-CM, n	
I have received regular intravenous (IV) infusions	2
I have received injections under the skin	4
I have regularly taken pills (tablets or capsules)	9
Years since ATTR-CM diagnosis, mean (min-max)	3.3 (1-9)

Table 2. Self-Reported Cardiologist Characteristics

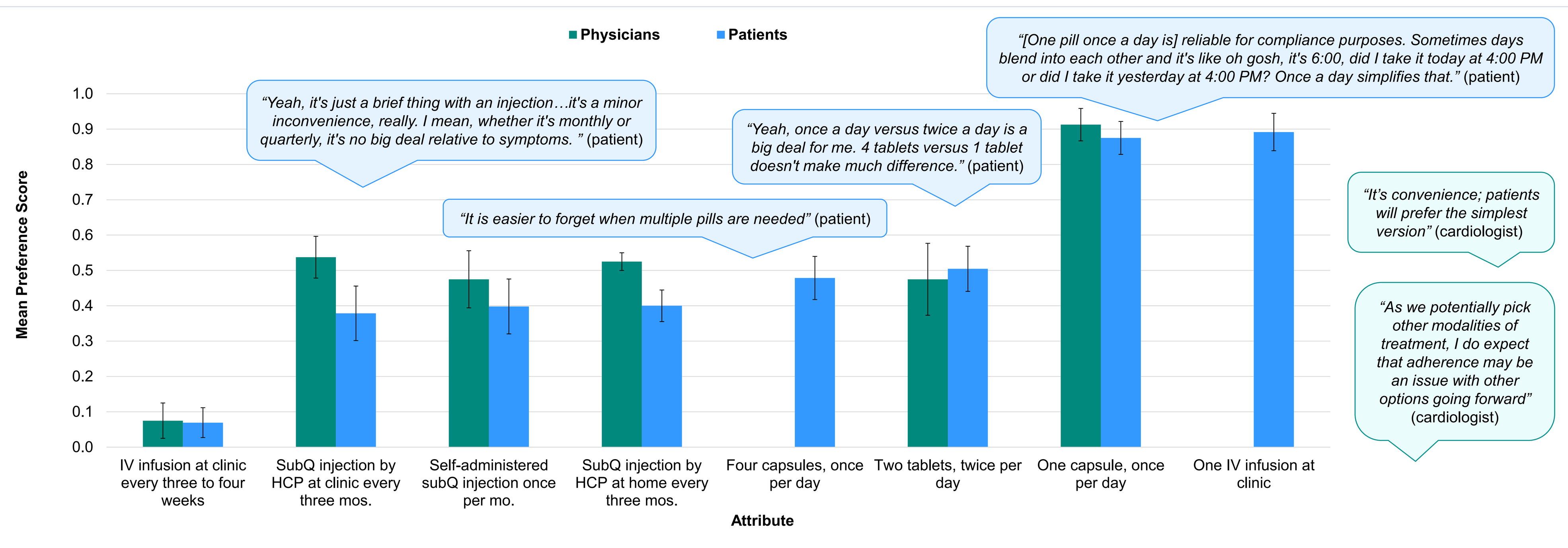
	N = 10
Age, mean years + SD	45.9 + 7.2
Years of practice managing/treating ATTR-CM , n	
2-5 years	3
6-10 years	6
11-20 years	1
Primary practice setting , n	
Non-teaching/community hospital-based practice	1
Teaching/university hospital-based practice	3
Academic cardiac specialist center	2
Non-academic cardiac specialist center	2
VA/DoD hospital	1
Other	1
Patients in practice with wild vs. hereditary ATTR-CM, %	
Wild Type	84%
Hereditary	16%

Figure 1. Mean Preference Scores for Treatment Outcomes



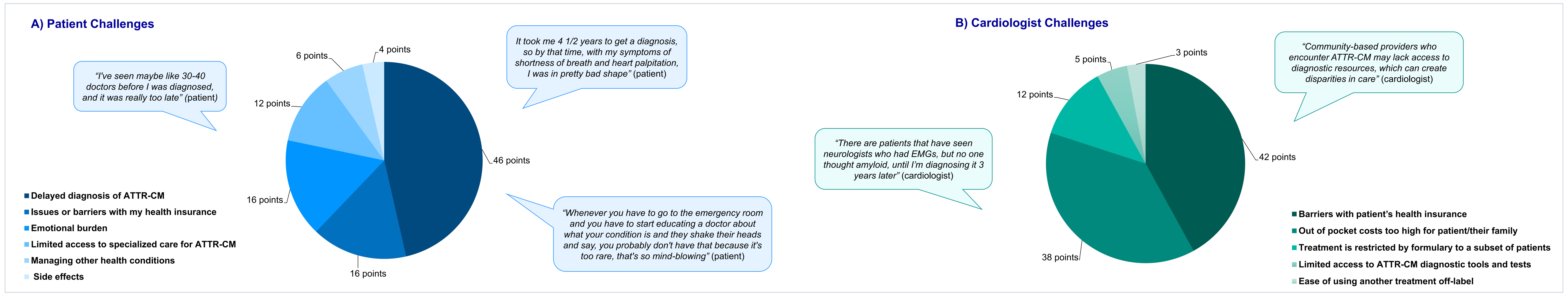
Note: Higher scores indicate higher importance. The figure does not include items focusing on improvements in functional status that were shown to cardiologists but removed in the patient survey due to cardiologist feedback of treatment primarily maintains, as opposed to improves, functional status.*"Lab tests show ATTR-CM not getting worse' item was not in the cardiologist survey, but lab tests were discussed during the cardiologist meeting. The error bars are the standard error of each mean preference score.

Figure 2. Mean Preference Scores for Dosing Regimen Attributes



Note: Higher scores indicate more preference. The "one IV infusion at clinic" and 'four capsules, once per day' were added to patient survey based on findings from the cardiologist meeting. Mo = month; Mons. = months. The error bars are the standard error of each mean preference score.

Figure 3. Mean Number of Points Assigned to Treatment Access Challenges by (A) Patients and (B) Cardiologists



Limitations

- The advisory board meetings were subject to potential selection bias and may not be reflective of the broader population of patients with ATTR-CM or cardiologists treating ATTR-CM.
- Choices made in the surveys may be subject to hypothetical choice bias. The BWS exercises did not include all attributes related to ATTR-CM treatments and those not included were held constant, which may lead to differences in choices than those made in a real-world setting.

References

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