

Healthcare resource utilization for patients with transthyretin amyloid cardiomyopathy: a real-world study in Europe, Canada and Japan



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Material Presented

Teresa Kauf¹, Jade Garratt-Wheeldon², Alisha Braid², Katherine Smethers², Mark Oellerich³
¹Alnylam Pharmaceuticals, Cambridge, MA; ²Adelphi Real World, Bollington, UK; ³Alnylam Switzerland GMBH, Switzerland

Conclusions

- HCRU among ATTR-CM patients was high. Almost a third of NYHA II and half of NYHA III patients had been hospitalized, with many requiring ER admissions.
- Patients were closely monitored with frequent tests and consultations, highlighting complex disease management and potential burden for patients and healthcare systems.
- Optimizing treatment strategies may help to reduce HCRU and relieve burden.

Background

- Transthyretin amyloid cardiomyopathy (ATTR-CM) is a rare, progressive and fatal systemic disease, resulting from extracellular deposition of misfolded transthyretin fibrils in the cardiac tissues and other organs.¹
- ATTR-CM requires early recognition, comprehensive management and disease specific therapy.²
- Limited studies exploring real-world data for ATTR-CM exist, particularly regarding healthcare resource utilization in a real-world setting.

Objective



Describe healthcare resource utilization (HCRU) across New York Heart Association (NYHA) classes for transthyretin amyloidosis cardiomyopathy (ATTR-CM) patients in a real-world setting.

Methods

- Secondary analyses using data from the Adelphi ATTR Disease Specific Programme™, a cross-sectional survey of physicians and patients with ATTR-CM in Europe (France, Germany, Italy, Portugal, Spain, UK), Canada and Japan, conducted between September 2024 – February 2025.

- The DSP methodology has been published, validated, and proven to be consistent over time.³⁻⁶

- Physicians completed electronic record forms for consecutively consulting patients.

- Patients were grouped by NYHA classes at survey, defined in **Table 1**.

- Analyses were descriptive, missing data were not imputed.

Physicians provided data following consultation in routine clinical practice

Electronic record forms

Completed by physicians about their consulting patients, capturing sociodemographic & clinical characteristics (including NYHA class), and history of consultations, testing, and hospitalizations over the last 12 months



Physicians
• Primary specialty was cardiology
• Treated at least 4 patients with ATTR



Patients with ATTR-CM

Results

- Overall, 166 physicians provided data for 865 ATTR-CM patients with self-reported data (Canada: 100, France: 133, Germany: 120, Italy: 139, Japan: 120, Portugal: 15, Spain: 120, UK: 118).
- Patient demographic and clinical characteristics are summarized in **Table 2**.

Table 1. NYHA classifications

NYHA class	Description
I	No limitation of physical activity. Ordinary physical activity does not cause undue fatigue, palpitation or shortness of breath.
II	Slight limitation of physical activity. Comfortable at rest. Ordinary physical activity results in fatigue, palpitation, shortness of breath or chest pain.
III	Marked limitation of physical activity. Comfortable at rest. Less than ordinary activity causes fatigue, palpitation, shortness of breath or chest pain.
IV	Symptoms of heart failure at rest. Any physical activity causes further discomfort.

Table 2. Patient demographic and clinical characteristics, at time of survey

	All patients (n=865)	I (n=149)	II (n=551)	NYHA class III (n=148)	IV (n=17)
ATTR phenotype, n (%)					
ATTR amyloidosis cardiomyopathy (ATTR-CM)	754 (87)	128 (86)	484 (88)	129 (87)	13 (76)
ATTR amyloidosis mixed phenotype (ATTR-CM + ATTR-PN)	111 (13)	21 (14)	67 (12)	19 (13)	4 (24)
Age (years)					
Mean (SD)	72.7 (12.0)	67.2 (12.5)	74.1 (10.6)	73.1 (14.1)	75.1 (16.3)
Patient sex, n (%)					
Male	636 (74)	123 (83)	392 (71)	106 (72)	15 (88)
Female	229 (26)	26 (17)	159 (29)	42 (28)	2 (12)
Time since diagnosis (years)					
Median (IQR)	1.4 (0.7 - 2.4)	1.3 (0.6 - 2.2)	1.4 (0.7 - 2.4)	1.4 (0.7 - 2.6)	1.6 (0.7 - 3.7)
Prescribed treatment at survey, n (%)					
Tafamidis	571 (86)	90 (73)	384 (90)	89 (87)	8 (80)
Vutrisiran	16 (2)	6 (5)	6 (1)	4 (4)	0 (0)
Patisiran	26 (4)	8 (7)	14 (3)	4 (4)	0 (0)

- During the 12 months prior to survey, 32% of patients had been hospitalized one or more times for their ATTR (excluding admissions for routine treatment administration). Of those patients, 67% had been admitted via the emergency room (ER) for one or more hospitalizations. Hospitalization and emergency room admissions are summarized in **Figure 1**.
- The number of tests/assessments conducted to monitor ATTR-CM are summarized in **Figure 2**
- The total number of consultations with healthcare professionals, in relation to ATTR-CM, are summarized in **Figure 3**.

Figure 1. Frequency of hospitalizations, and the proportion of hospitalized patients admitted via the ER, over the 12 months prior to survey, across NYHA classes

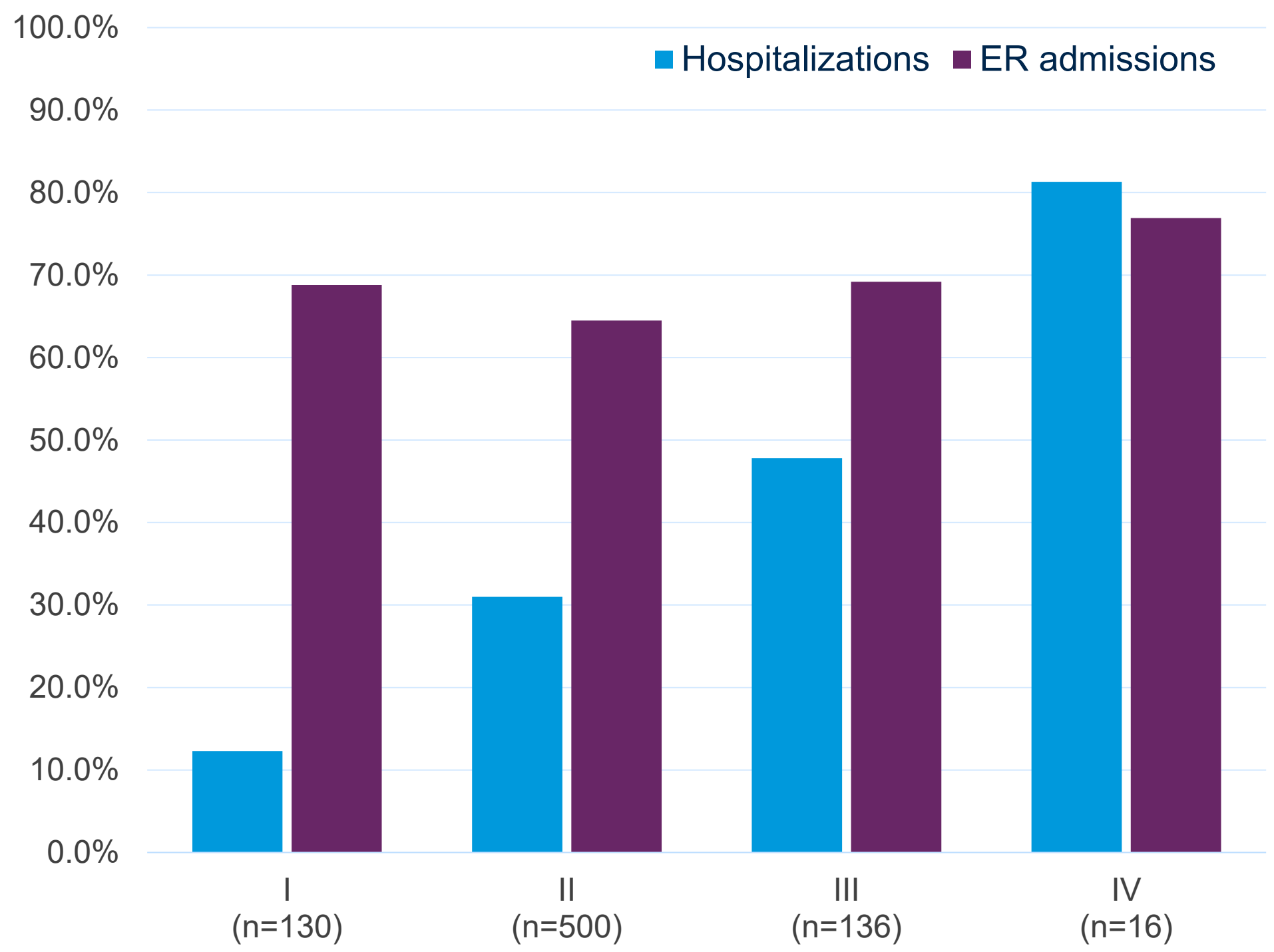


Figure 2. Total number of tests/assessments conducted to monitor ATTR-CM, over the 12 months prior to survey, across NYHA classes

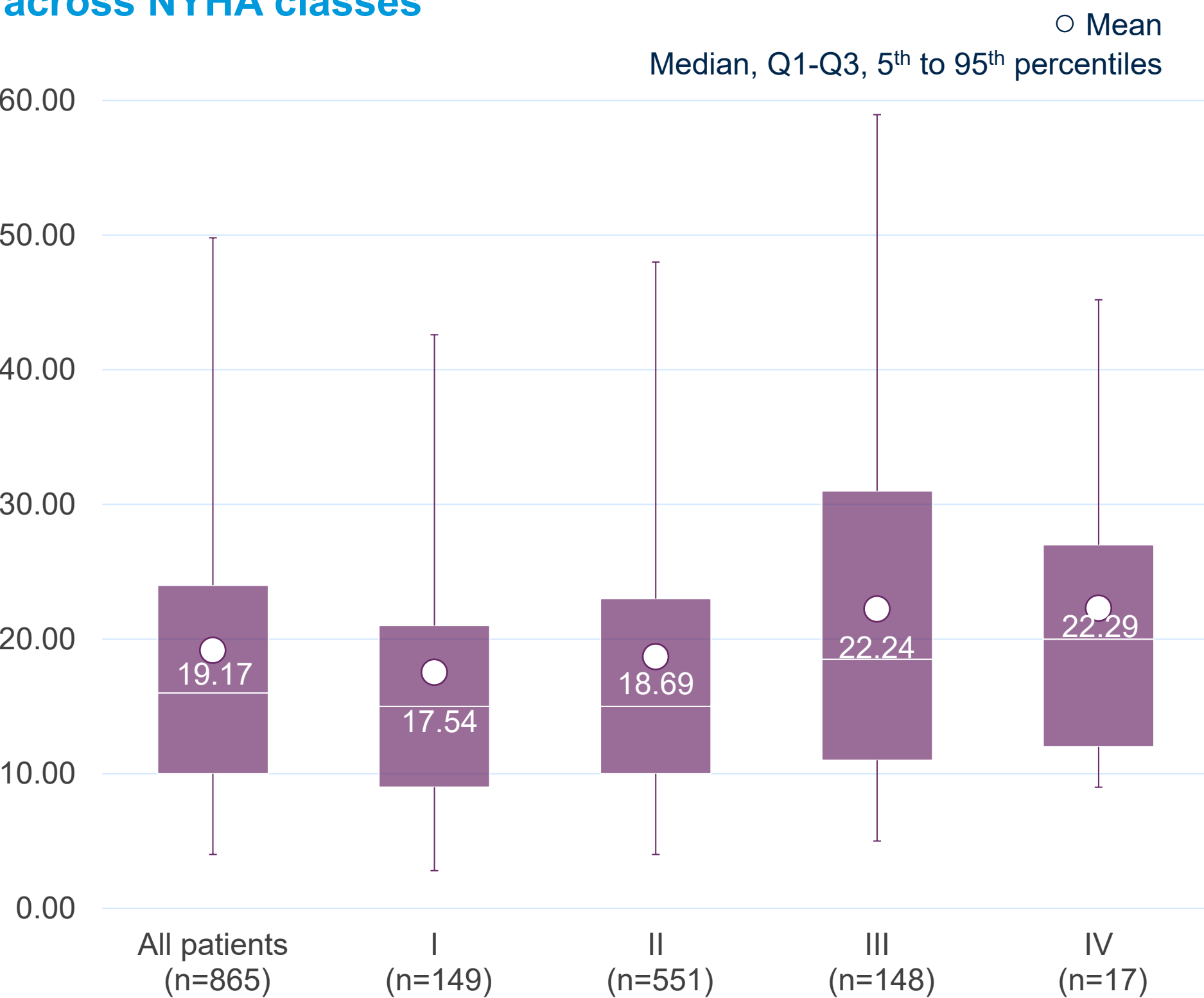
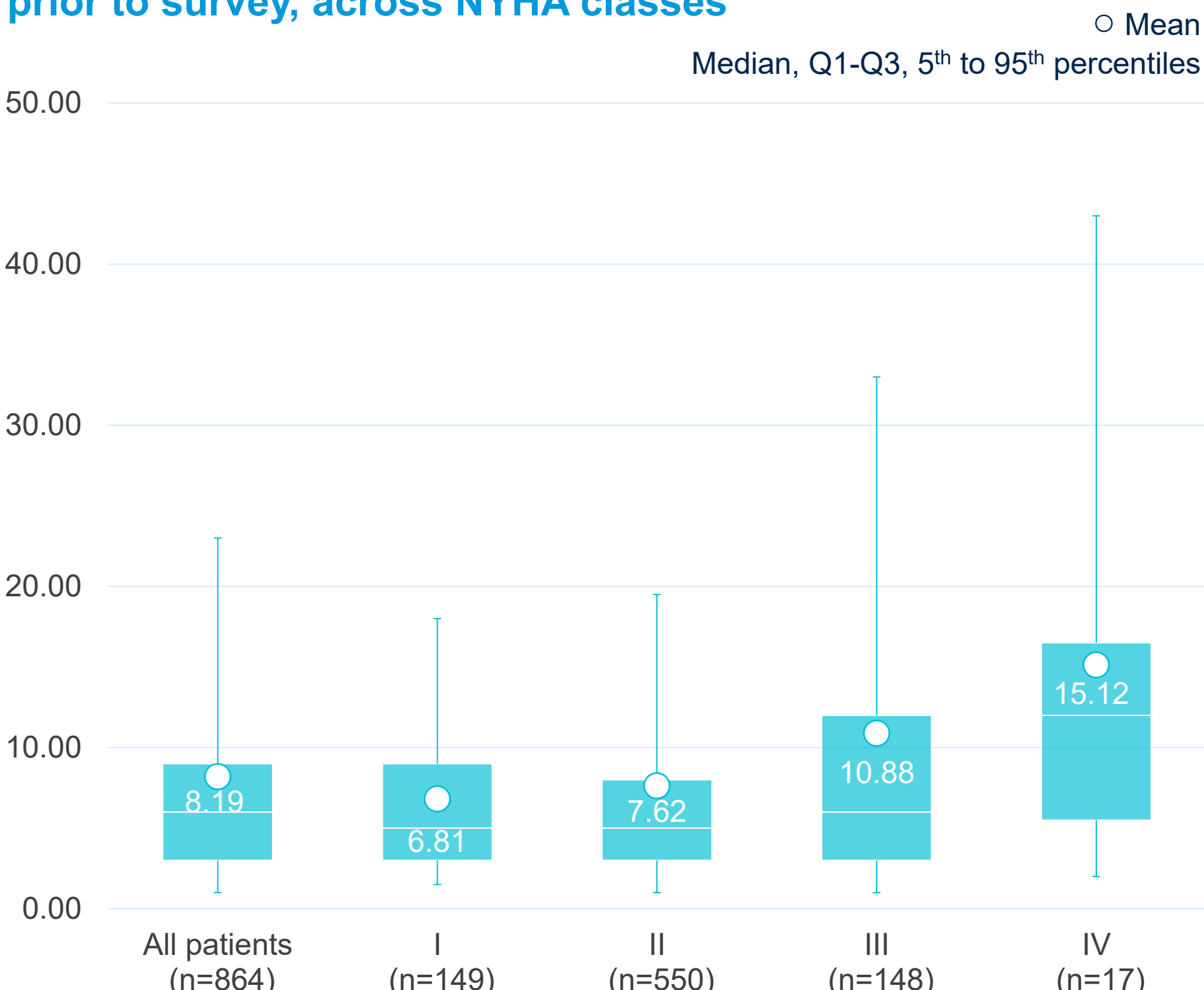
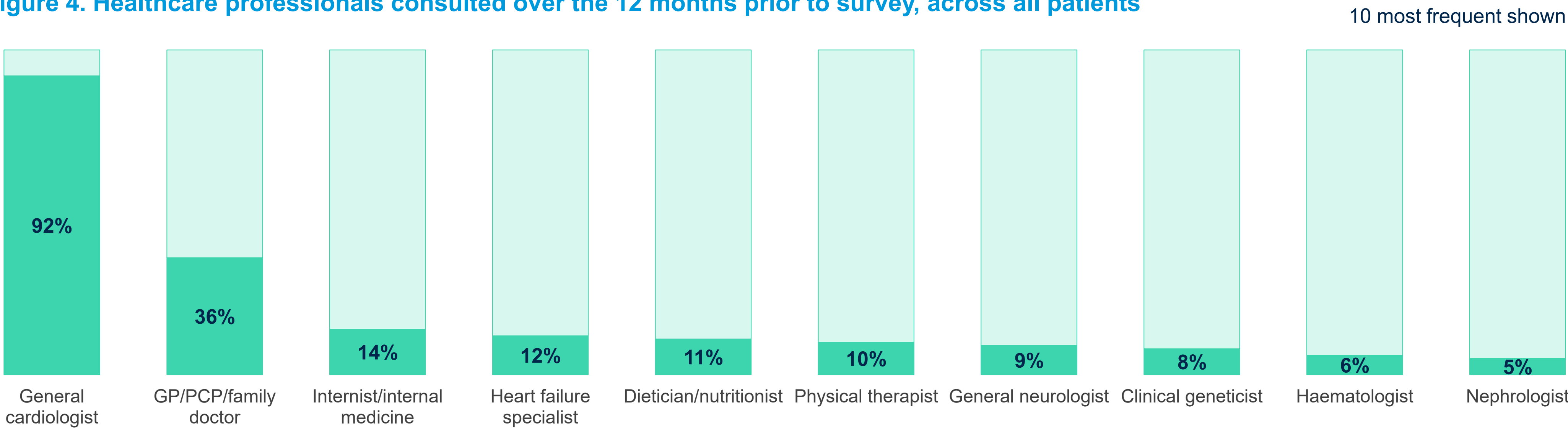


Figure 3. Total number of consultations with any healthcare professional in relation to ATTR-CM, over the 12 months prior to survey, across NYHA classes



The most frequently consulted healthcare professional types are summarized in **Figure 4**.

Figure 4. Healthcare professionals consulted over the 12 months prior to survey, across all patients



Limitations

- This study was based on a pragmatic rather than true random sample; physician and patient participation is influenced by willingness to complete record forms.

References

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Disclosures: TK, MO are employees of Alnylam Pharmaceuticals and own shares in Alnylam Pharmaceuticals; JGW, AB, KS are employees of Adelphi Real World.

If you are seeking additional scientific information related to Alnylam therapeutics, US HCPs may visit the Alnylam US Medical Affairs website at RNAiScience.com. Non-US HCPs should contact medinfo@alnylam.com.

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