PCR115

Health-related quality of life for patients with transthyretin amyloid cardiomyopathy: a real-world study in Europe, Canada and Japan

For US HCPs Only

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Conclusions

- **ATTR-CM had a notable impact on patients HRQoL, with average scores for the EQ-5D and KCCQ being numerically lower among more severe NYHA classes.**
- **Symptoms of fatigue and shortness of breath were bothersome for most patients.**
- **The poor QoL outcomes reported at later stages of disease highlight the importance of early diagnosis, and timely initiation of treatments to maintain stability and slow progression.**

Background

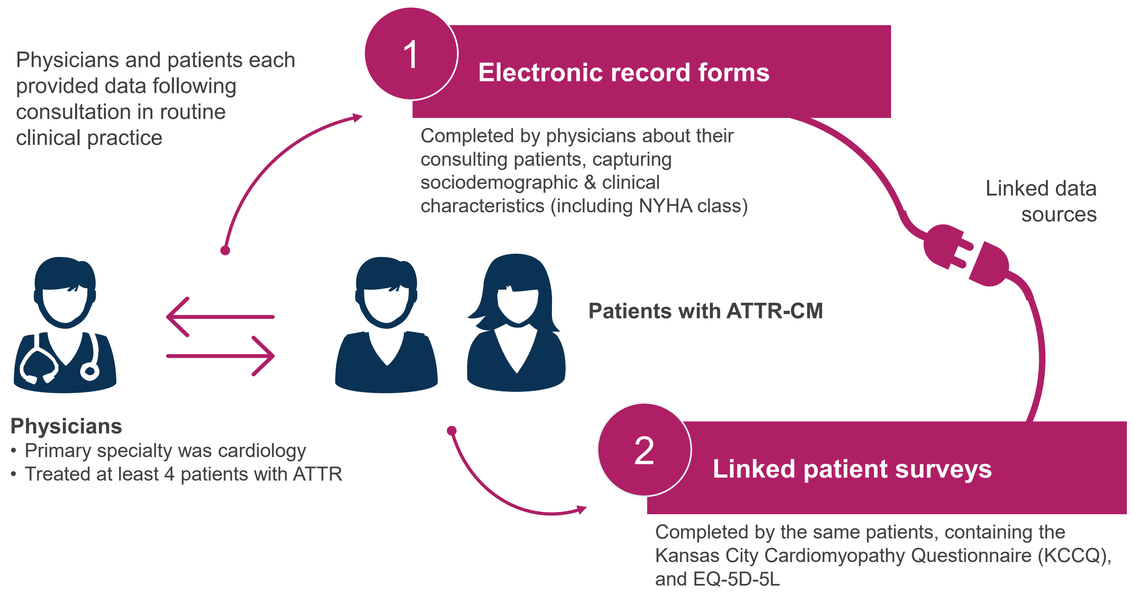
- Transthyretin amyloid cardiomyopathy (ATTR-CM) is a progressive, and fatal disease characterized by the accumulation of misfolded transthyretin (TTR) protein in the heart.[1-2]
- The physical symptoms of ATTR CM can have a large impact on activities of daily life and affect health-related quality of life (HRQoL).[1-2]
- Few studies have explored health related quality of life (HRQoL) for ATTR-CM in real-world clinical settings, at different stages of disease progression.

Objective

Describe health-related quality of life (HRQoL) across New York Heart Association (NYHA) classes for transthyretin amyloidosis cardiomyopathy (ATTR-CM) patients in a real-world setting, as reported by patients

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, 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, who completed separate surveys of their own on a voluntary basis.
- Patients were grouped by NYHA class at survey as I, II, and III (class IV excluded from analyses due to small group size). NYHA classes are defined in **Table 1**.
- Analyses were descriptive, missing data were not imputed.



Results

- Overall, 68 physicians provided data for 235 ATTR-CM patients with self-reported data (Canada: 11, France: 45, Germany: 89, Italy: 27, Japan: 32, Spain: 29, UK: 2).
- Patient demographic and clinical characteristics are summarised in **Table 2**. There were too few NYHA class IV patients (n=3) to report separately.

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=235)	NYHA class I (n=39)	NYHA class II (n=157)	NYHA class III (n=36)
ATTR phenotype, n (%)				
ATTR amyloidosis cardiomyopathy (ATTR-CM)	220 (94)	38 (97)	144 (92)	35 (97)
ATTR amyloidosis mixed phenotype (ATTR-CM + ATTR-PN)	15 (6)	1 (3)	13 (8)	1 (3)
Age (years)				
Mean (SD)	70.1 (11.6)	65.9 (10.6)	71.4 (10.4)	69.9 (14.8)
Patient sex, n (%)				
Male	182 (77)	34 (87)	118 (75)	27 (75)
Female	53 (23)	5 (13)	39 (25)	9 (25)
Time since diagnosis (years)				
Median (IQR)	1.3 (0.8 - 2.4)	1.5 (0.9 - 2.7)	1.3 (0.8 - 2.3)	1.2 (0.7 - 2.8)
Prescribed treatment at survey, n (%)	**n=212**	**n=35**	**n=141**	**n=34**
Tafamidis	196 (92)	32 (91)	130 (92)	32 (94)
Vutrisiran	4 (2)	2 (6)	0 (0)	2 (6)
Patisiran	3 (1)	1 (3)	2 (1)	0 (0)

- Patients reported a mean (SD) EQ-5D health utility of 0.82 (0.17), and EQ-VAS score of EQ-VAS score of 63.52 (18.03). EQ-5D health utility and EQ-VAS scores are summarised in **Figure 1 & 2**.
- Patients reported a mean (SD) KCCQ overall summary score of 63.80 (18.11), and quality-of-life (QoL) score of 59.52 (19.34). KCCQ scores are summarised in **Figures 3 & 4**.
- Patients reported that they had been bothered by symptoms of fatigue (89%), shortness of breath (84%), and swelling in legs/feet (66%), over the prior 2 weeks.

Figure 1. EQ-5D health utilities across NYHA classes (Germany value sets)

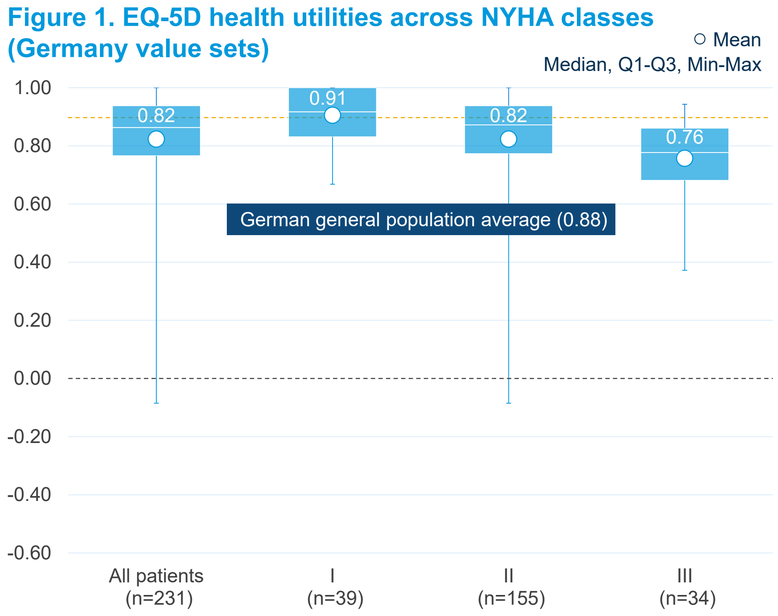


Figure 2. EQ-VAS scores across NYHA classes

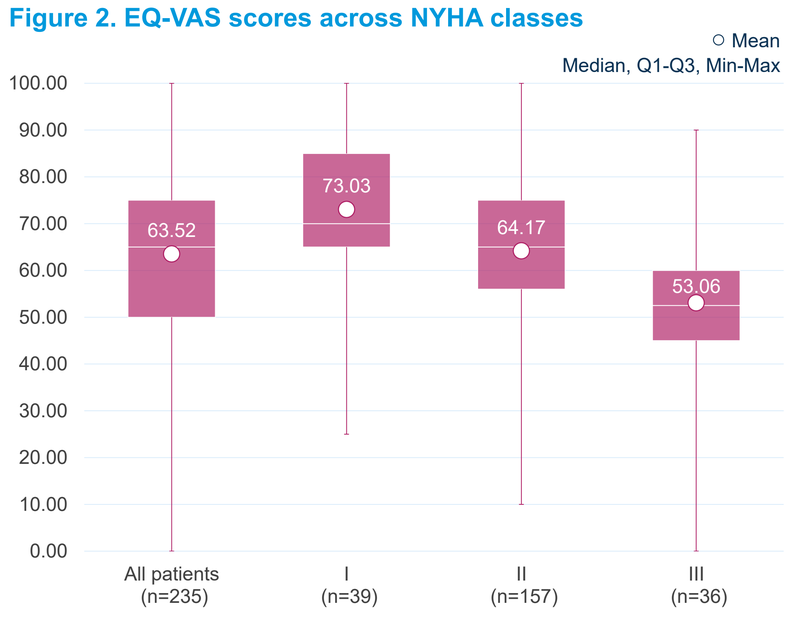


Figure 3. KCCQ overall summary score across NYHA classes

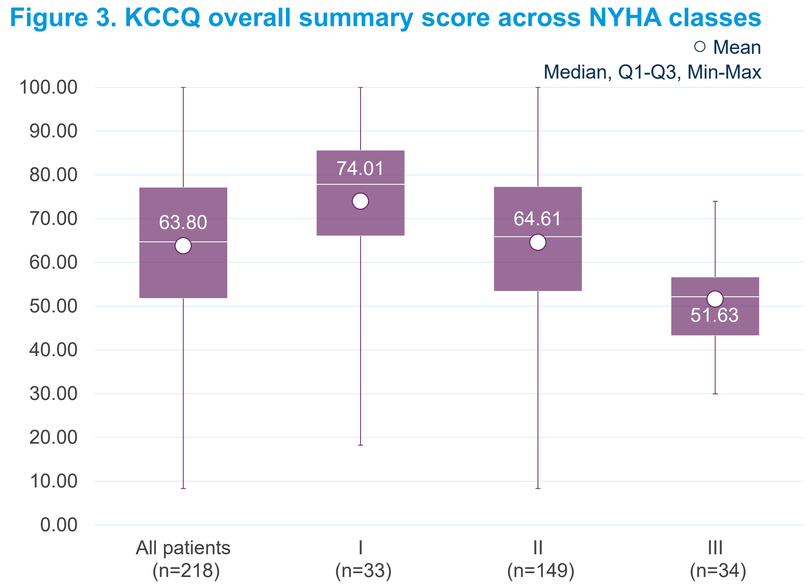
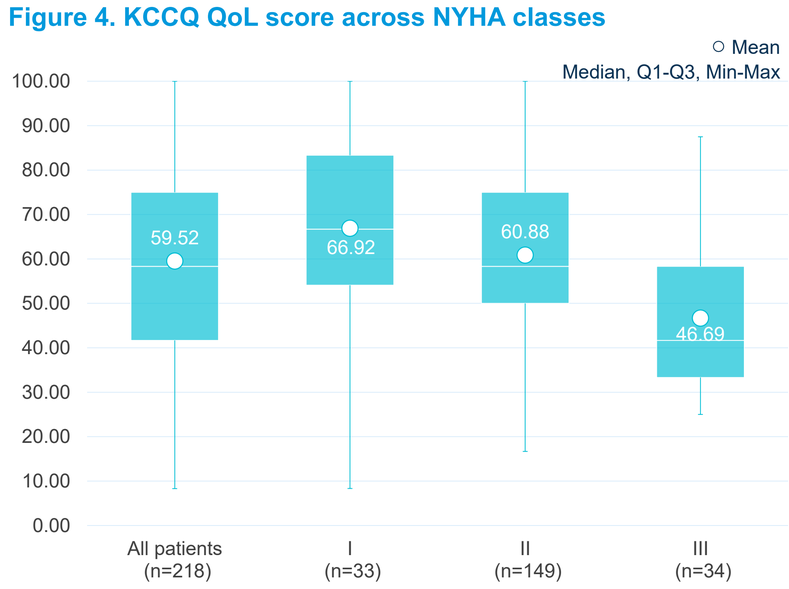


Figure 4. KCCQ QoL score across NYHA classes



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.

Acknowledgements

- Data was collected by Adelphi Real World via the ATTR Disease Specific Programme™, an independent survey whereby all data are the intellectual property of Adelphi Real World. Alnylam Pharmaceuticals subscribed to access this data source.
- Adelphi Real World and Alnylam Pharmaceuticals would like to thank the physicians and patients that participated in this survey.

References

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Disclosures: TK, MO are employees of Alnylam Pharmaceuticals and own shares in Alnylam Pharmaceuticals; **JW, BK, SW** 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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