Impact of tafamidis use in Colombian patients diagnosed with ATTR-CM on health-related quality of life during two years of follow-up: A real world study in Colombia.

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BACKGROUND

- Cardiac amyloidosis (CA) is a restrictive cardiomyopathy secondary to amyloid deposits, which are misfolded protein fragments, in cardiac tissue[1,2]. The prevalence of CA in patients with heart failure is estimated at 13.7% (95%CI 7.9 – 19.8) according to a meta-analysis collecting studies worldwide[3].
- Tafamidis was the first treatment used to treat certain forms of transthyretin amyloid (ATTR) amyloidosis. Tafamidis is disease-modifying transthyretin kinetic stabilizer for treating transthyretin amyloidosis[4].
- The pooled analysis from pivotal study (ATTR-ACT study) showed that tafamidis of 80 mg and 20 mg versus placebo were associated with a reduction in all-cause of mortality (29.5% vs 42.9%, HR 0.70; CI 0.51-0.96) and cardiovascular hospitalizations (RR 0.68; CI 0.56-0.81) at 30 months[5].
- Tafamidis was approved in July 2022 in Colombia.

OBJECTIVE

- To describe the impact of tafamidis use on quality of life and hospitalization rates in Colombian patients diagnosed with transthyretin amyloid cardiomyopathy (ATTR-CM) both hereditary and wild-type over a two-years follow-up period.

METHODS

- We conducted a prospective, longitudinal, non-interventional study across five healthcare institutions in Colombia. Adult patients with a confirmed diagnosis of ATTR-CM who had been prescribed with tafamidis prior to March 2024 were consecutively enrolled.
- Follow-up began on the index date (first tafamidis prescription) and continued until one of the following occurred: patient death, withdrawal from the study, treatment discontinuation, loss to follow-up, or completion of 24 months of follow-up.
- The study was performed at five Colombian Health Provider Institution whose population included patients with ATTR-CM diagnosis in the study period.
- Quality of life was evaluated when the agreed to participate and signed the informed consent. Quality of life, hospitalization for any cause, and cardiovascular medication were measured 24 months after prescription of tafamidis. Twelve months before the index date, treatments, and frequency of hospitalization for any cause were measured.
- Health-related quality of life was assessed using the Kansas City Cardiomyopathy Questionnaire (KCCQ) at baseline and at 6, 12, 18, and 24 months. A ≥10-point increase was considered clinically meaningful. Hospitalizations were evaluated during the 12 months prior to treatment initiation and follow-up.
- Clinical and demographic information at baseline were abstracted from the medical record while data related to hospitalizations, cardiovascular functionality, quality of life, and death were collected directly from the patients contacted every three months by phone calls through validated questionnaires and structured interviews.
- Descriptive statistical consisted on frequencies and percentages for binary and categorical variables, while medians, means, standard deviation (SD), and interquartile ranges (IQRs) for continuous variables.
- Linear quantile mixed models developed by Geraci et al[6] were used to estimate the adjusted median of KCCQ score by domain during follow-up.

RESULTS

- Thirty-two patients were enrolled in the study. The mean follow-up was 21.6 months. The mean age was 75.1 ± 6.7 years, and predominantly male (84.4%) (Table 1).
- Wild-type ATTR was the most common form (59.4%). Left ventricular ejection fraction was <60% in 75% of patients. According to the New York Heart Association (NYHA) classification, 12 patients were in class II, 9 in class III, and 8 in class I and 3 without data (Table 2).

Table 1. Demographic characteristics at index date

	Absolute Frequency	Total
	32	%
Demographic characteristics		
Mean Age±	75.1 ± 6.7	
Gender n (%)		
Male	27	84.4
Female	5	15.6
Race n (%)		
Mixed race	25	78.2
Black	3	9.4
Non information	4	12.5
Other	0	0.0

Table 2. Clinical characteristics at index date

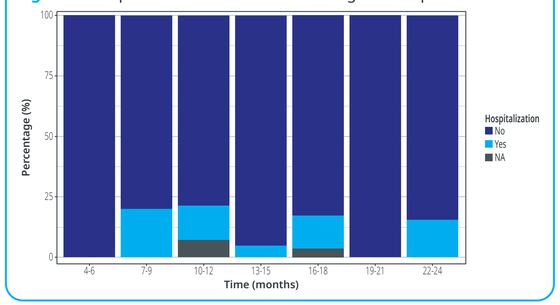
	Absolute Frequency	Total
	32	%
Genotype		
ATTRwt	19	59.4
ATTRv	10	31.3
No data	3	9.4
Biopsy		
Yes	6	18.8
No	25	78.1
No data	1	3.1
GFR (Gromerular filtration rate)		
> 40	31	96.9
No data	1	3.1
Peruggini Score		
Grade 1	1	3.1
Grade 2	8	25.0
Grade 3	21	65.6
No data	2	6.3
Low voltage QRS		
Yes	12	37.5
No	15	46.9
No data	5	15.6
Waves Q pseudo–myocardial infarction		
Yes	10	31.3
No	17	53.1
No data	5	15.6
AV block disease		
Yes	10	31.3
No	17	53.1
No data	5	26.3

RESULTS (cont)

	Absolute Frequency	Total
	32	%
LEVF%		
<30	3	9.4
30 - 60	21	65.6
>60	4	12.5
No data	4	12.5
NYHA		
Class I	8	25.0
Class II	12	37.5
Class III	9	28.1
No data	3	9.4
Time from Diagnosis to Symptoms n, %		
0-3 months	12	37.5
4- 6 months	5	15.6
7 - 12 months	5	15.6
> 12 months	9	28.1
No data	1	3.1

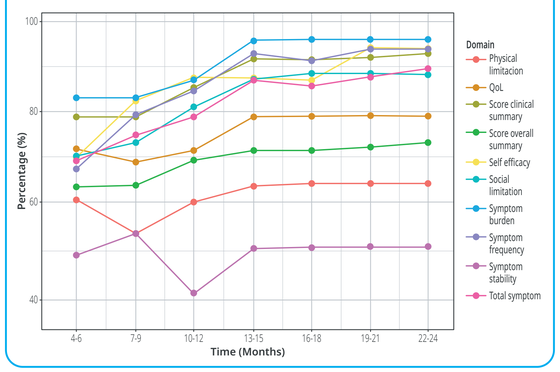
ATTRv: Hereditary transthyretin Cardiac amyloidosis.
ATTRwt: Wild-type Transthyretin Cardiac Amyloidosis

Figure 1. Hospitalization for all causes during follow-up



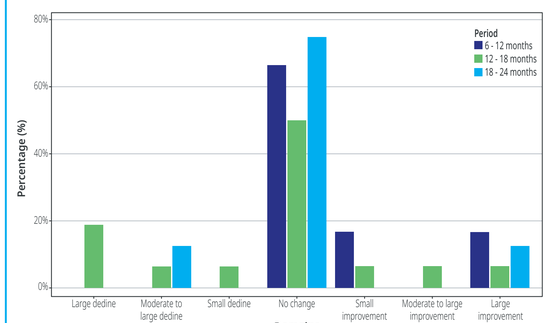
- During follow-up, ten patients experienced at least one hospitalization (31.3%), accounting for a total of 13 events, eight of which were due to cardiovascular causes (Figure 1).
- Four patients reported use of cardiovascular devices. Two in 19-21 months follow-up period, 1 in 7-9 months, and the other in 10 -12 months of follow-up. In all cases, the cardiovascular device was cardiac resynchronization therapy – pacemaker.

Figure 2. Median KCCQ domains during follow-up



RESULTS (cont)

Figure 3. Six-months changes by KCCQ Overall summary Score



- The changes of KCCQ score domains during the follow-up were positive in all cases. The reatest improvements were observed in symptom frequency, symptom burden, and social limitation domains. Domain scores stabilized after approximately 13–15 months of follow-up.
- Median KCCQ overall score increased from 71.6 at 4–6 months to 78.9 at 10–12 months.

CONCLUSION

- In this real-world cohort of Colombian patients with ATTR-CM, tafamidis use was associated with clinically meaningful improvements in HRQoL and a relatively low burden of hospitalizations over two years. These findings add real-world evidence and provide local data to support its use in routine clinical practice. However, this analysis was limited by the small sample size.

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