

## BACKGROUND

- Transthyretin (ATTR) amyloidosis is a rare and often fatal disease caused by the misfolding of the protein transthyretin into an insoluble fibril that accumulates in various tissues and organs, causing dysfunction.
- ATTR primarily affects the heart and nervous system and can be categorized into two types, hereditary variant (ATTRv) and wild-type (ATTRwt).
- Approved therapeutics are able to slow down disease progression and improve survival, hospitalization rates and quality of life; however, the financial cost of these therapeutics can be an obstacle for patients.
- Financial toxicity is a patient-reported measure of the deleterious effect of financial stress caused by the cost of care on the well-being of patients and their families.

## OBJECTIVE

- To describe the factors associated with financial toxicity and the economic burden of ATTR care and treatment.

## METHODS

### Data Collection

- The Amyloidosis Research Consortium (ARC) conducted a United States (US) based online survey for patients with ATTR amyloidosis and caregivers from August 3, 2021, to January 31, 2022.
- Surveys collected demographic information, disease characteristics, healthcare resource utilization, financial impact of treatment, financial concerns related to health care, performance status, psychological distress, health-related quality of life, and impact of the COVID-19 pandemic.

### Outcomes and Statistical Analyses

- Financial toxicity was assessed using the Comprehensive Score for financial Toxicity-Functional Assessment of Chronic Illness Therapy (COST-FACIT), a validated patient-reported outcome measure of financial distress experienced by cancer patients.
- Patient demographics and disease characteristics were summarized and compared between financial toxicity status.
- Multivariate linear regression was used to assess the relationship between the COST score and ATTR type, controlling for age, US region of residence, race/ethnicity, education, urban setting of residence, household income, work status, time since ATTR diagnosis, number of insurance plans, and impact of COVID-19 on financial status.

## RESULTS

### Demographic Characteristics and Financial Toxicity

- A total of 452 eligible patients and caregivers completed the entire survey. Of those, 387 (86%) were patients and 65 (14%) were caregivers.
- The majority of respondents (65%) had ATTRwt, 75% were male, 45% were 65-75 years of age, 39% were older than 75, 87% were married or living with a partner, 92% were white, 51% had a post-graduate or graduate degree, and 46% reported a household income greater than \$100,000 (**Table 1**).
- 249 (55%) reported some level of financial toxicity (COST score <26). The mean COST score was 24.
- Mean (standard deviation (SD)) monthly cost of managing ATTR was \$728.69 (\$1,711.67) and the mean (SD) monthly cost of ATTR treatment was \$645.41 (\$2,880.24).
- Respondents experiencing financial toxicity more often: had ATTRv (41% vs 27%), were younger (22% vs 8% <65 years old), non-white (11% vs 5%), and had a household income <\$100,000 (65% vs 40%).
- Those experiencing financial toxicity were also less likely to be retired (68% vs 83%) and have multiple insurance coverage (27% vs 44%).

**Table 1. Demographic Characteristics of ATTR Patients and Caregivers, Overall and by Financial Toxicity Status**

	Overall	Not Experiencing Financial Toxicity (COST Score ≥26)	Experiencing Financial Toxicity (COST Score <26)	
<b>Demographic Characteristics, N (%)</b>	<b>N = 452</b>	<b>N = 203</b>	<b>N = 249</b>	<b>p-value</b>
<b>Sex</b>				
Female	114 (25.2)	48 (23.6)	66 (26.5)	
Male	338 (74.8)	155 (76.4)	183 (73.5)	
<b>Age</b>				<0.001
<65	74 (16.4)	17 (8.4)	57 (22.9)	
65-75	202 (44.7)	91 (44.8)	111 (44.6)	
>75	176 (38.9)	95 (46.8)	81 (32.5)	
<b>Race</b>				
White	412 (91.2)	193 (95.1)	219 (88.0)	
African American	25 (5.5)	3 (1.5)	22 (98.8)	
Hispanic	9 (2.0)	3 (1.5)	6 (2.4)	
Asian	4 (0.9)	4 (2.0)	0 (0.0)	
Other	2 (0.4)	0 (0.0)	2 (0.8)	
<b>Education</b>				<0.001
High school or less	25 (5.5)	6 (3.0)	19 (7.6)	
Some college or college degree	198 (43.8)	68 (33.5)	130 (52.2)	
Postgraduate or graduate degree	229 (50.7)	129 (63.5)	100 (40.2)	
<b>Household Income</b>				<0.001
≤\$29,999	24 (5.3)	4 (2.0)	20 (8.0)	
\$30,000 to \$49,999	50 (11)	11 (5.4)	39 (16)	
\$50,000 to \$99,999	169 (37)	65 (32)	104 (42)	
\$100,000 to \$349,999	180 (40)	97 (48)	83 (33)	
≥\$350,000	29 (6.4)	26 (13)	3 (1.2)	
<b>Region</b>				
Midwest	73 (16)	32 (16)	41 (16)	
Northeast	128 (28)	64 (32)	64 (26)	
South	150 (33)	58 (29)	92 (37)	
West	101 (22)	49 (24)	52 (21)	
<b>Urban Setting</b>				
In an urban or city area	104 (23)	45 (22)	59 (24)	
In a suburban area next to a city	211 (47)	90 (44)	121 (49)	
Small town or rural area	137 (30)	68 (33)	69 (28)	
<b>Multiple Insurance Coverage</b>				
Yes	157 (35)	90 (44)	67 (27)	<0.001
<b>Work Status</b>				0.004
Not employed	22 (4.9)	3 (1.5)	19 (7.6)	
Employed, working part-time	26 (5.8)	10 (4.9)	16 (6.4)	
Employed, working full-time	62 (14)	23 (11)	39 (16)	
Retired	342 (76)	167 (82)	175 (70)	

### Health Care Resource Utilization

- Respondents experiencing financial toxicity had a higher proportion of receiving co-pay assistance (53% vs 33%), having to delay the start of treatment either due to cost or insurance coverage issues (31% vs 9%), having to use either savings (47% vs 24%), borrow money (9% vs 1%) or make trade-offs (28% vs 4%) to pay for their treatment.
- The majority (69%) of respondents (82% of those experiencing financial toxicity vs 54% not experiencing financial toxicity) offset the cost of treatment by either using savings, borrowing money, making trade-offs, or using co-pay assistance to pay for ATTR treatment (**Figure 1**).
- Respondents not experiencing financial toxicity were more likely to be on more than one treatment (27% vs 20%).

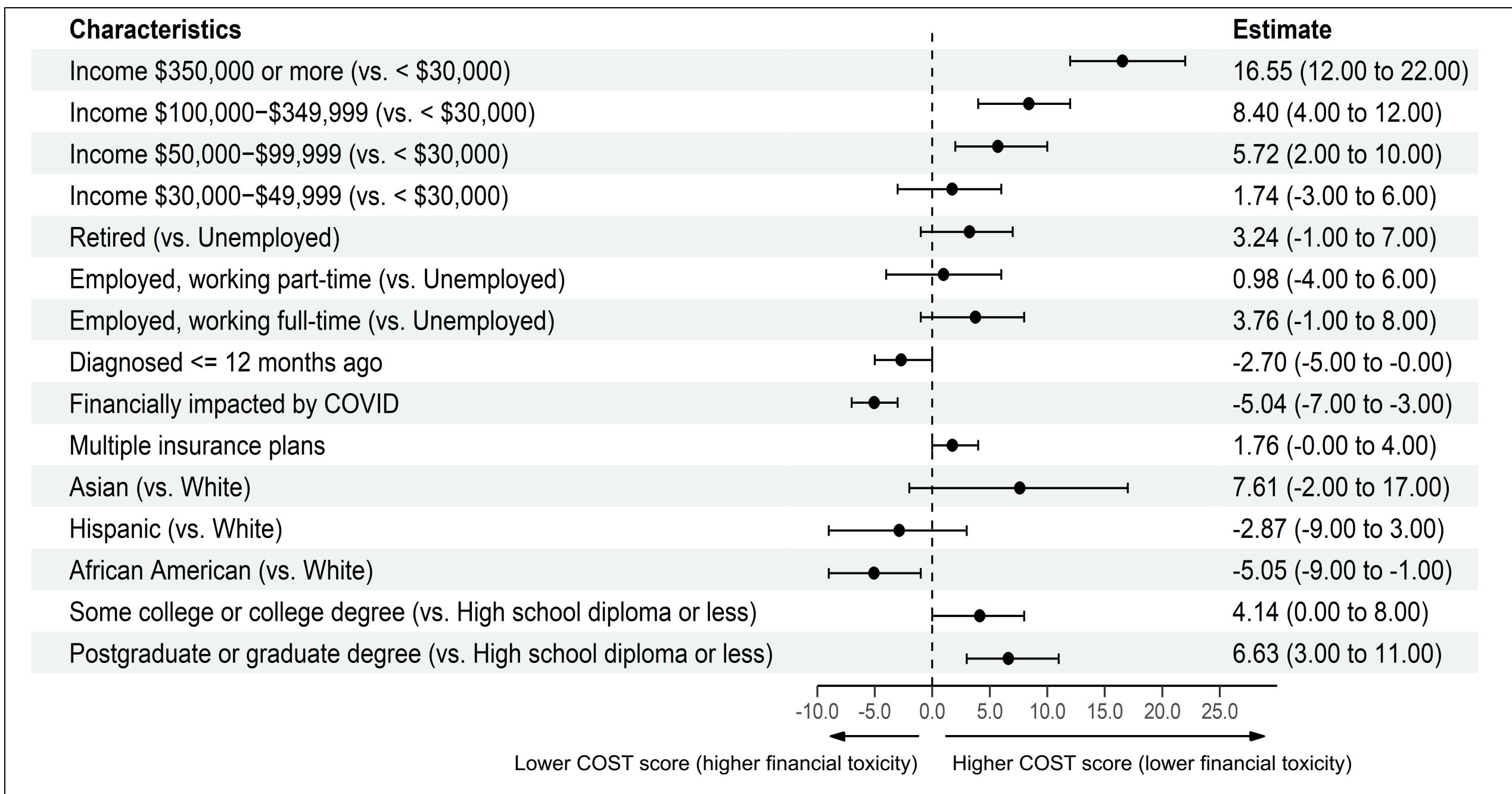
**Figure 1. Health Care Resource Utilization of ATTR Patients and Caregivers, Overall and by Financial Toxicity Status**



### Multivariate Linear Regression

- African American respondents scored on average 5.1 points lower on the COST vs. white respondents (**Figure 2**), indicating higher levels of financial toxicity.
- Respondents diagnosed with ATTR ≤12 months prior to survey completion had 2.4 points lower average COST scores (higher financial toxicity) vs those diagnosed over 12 months ago.
- Respondents with household income >\$50,000 had lower levels of financial toxicity. Participants also scored higher on COST-FACIT if they had multiple insurance plans or lived in the Midwestern region of the US.

**Figure 2. Linear Regression Coefficients of Association between Patient Characteristics and Financial Toxicity Score**



## DISCUSSION

- As expected, higher income, educational levels, and multiple insurance plans had associations suggesting a protective impact against financial distress.
- Despite overall high levels of education, income, and insurance coverage, high rates of financial toxicity were observed in this sample, suggesting a high cost burden of amyloidosis treatments.
- African American patients had higher levels of financial distress even after controlling for key sociodemographic and patient characteristics.
- Respondents not experiencing financial toxicity were more likely to be on more than one treatment, consistent with a higher level of sophistication among these patients/caregivers and their physicians or pharmacists in order to obtain coverage for multiple treatments.

## CONCLUSIONS

- Despite the benefits of newly approved disease-modifying drugs for the treatment of ATTR, the financial distress among this patient population is significant and comparable to patients with cancer<sup>1,2</sup>
- Access to clinical trials and co-pay assistance programs may not be enough to offset the cost of managing and treating ATTR, as many patients have to make trade-offs for paying for treatment, either using savings and/or borrowing money to pay for treatment and leading to increased financial distress.

## REFERENCES

- Esselen et al. Int J Gynecol Cancer. 2021;31(6):801-806.
- de Souza et al. Cancer. 2017;123(3):476-484.

## DISCLOSURES

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