

# Disability, Resource Utilisation, and Caregiver Support Among a Real-World Sample of Patients with Transthyretin Amyloidosis with Polyneuropathy

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## Introduction

- Transthyretin amyloidosis with polyneuropathy (ATTR-PN) is a rare, progressive disease characterized by the deposition of misfolded transthyretin in the peripheral nervous system, leading to neuropathy and sensorimotor symptoms. Cardiomyopathy also presents in mixed phenotype patients<sup>1</sup>.
- Patients with ATTR-PN often experience symptoms that impair their ability to undertake physical tasks, causing disability and the requirement for assistance from a caregiver in day-to-day life<sup>1</sup>.
- Severity of the functional limitations of patients with ATTR-PN can be monitored using Polyneuropathy Disability (PND) scores. Scores range from PND 0 to IV, with the highest score indicating the patient is bedridden and/or requires a wheelchair<sup>2</sup>.
- Data which characterise real-world disability burden, healthcare resource utilisation (HCRU), and need for caregiver support among patients with ATTR-PN are limited.

## Objective



Describe HCRU, caregiver support and healthcare consultations relating to physical function among patients with ATTR-PN or mixed phenotype ATTR, across different PND scores.

## Methods

- Secondary analyses were performed using data from the Adelphi ATTR Disease Specific Programme™, a cross-sectional survey of ATTR-treating physicians and their patients in Brazil, China, France, Germany, Italy, Spain, UK, and the United States, October 2024-May 2025.
- The DSP methodology has been published and validated<sup>3-6</sup>.
- Patients were grouped according to their PND score at time of survey, PND scoring definitions are provided in **Table 1**.
- Analyses were descriptive.

Physicians provided data on up to 10 consecutively consulting patients, following consultation in routine clinical practice



## Electronic record forms

- Completed by physicians, capturing:
  - Demographics & clinical characteristics (including PND scores)
  - Consultation history (last 12 months)
  - Mobility aid use, and home modifications made, due to ATTR-PN
  - Caregiver support received



## Physicians

- Primary specialty in neurology or cardiology
- Treated at least 4 ATTR patients per month



## Patients with ATTR-PN

## Results

- Overall, 229 physicians reported data for 1,179 patients (PND 0: 104 [8.8%], I: 413 [35.0%], II: 366 [31.0%], IIIA: 184 [15.6%], IIIB: 82 [7.0%], IV: 30 [2.5%]).
- Patient sociodemographic and clinical characteristics are reported in **Table 2**.

**Table 1. PND scoring system definitions**

PND score	Description
0	No impairment
I	Sensory disturbances, preserved walking capability
II	Impaired walking capability, but able to walk without a stick or crutches
IIIA	Walking with the help of one stick or crutch
IIIB	Walking with the help of two sticks or crutches
IV	Confined to a wheelchair or bedridden

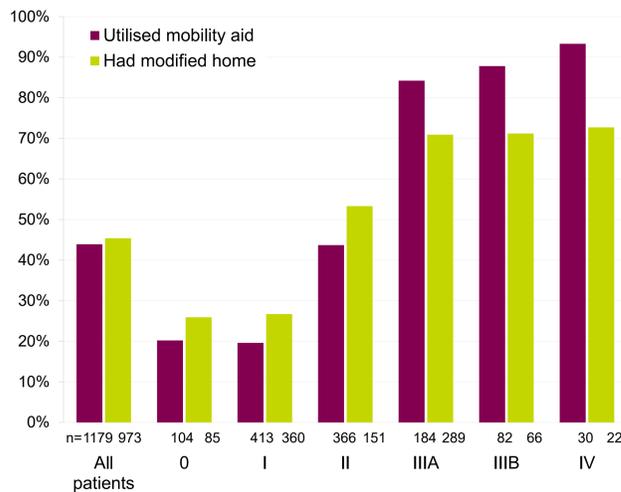
**Table 2. Physician-reported patient characteristics across PND scores, at the time of survey**

	All patients (n=1179)	0 (n=104)	I (n=413)	II (n=366)	IIIA (n=184)	IIIB (n=82)	IV (n=30)
Age (years), mean (SD)	58.2 (15.7)	57.0 (14.9)	55.1 (15.7)	57.8 (15.2)	63.8 (16.2)	62.4 (14.0)	66.2 (12.5)
Sex - male, n (%)	772 (65.5)	68 (65.4)	272 (65.9)	235 (64.2)	128 (69.6)	52 (63.4)	17 (56.7)
ATTR phenotype, n (%)							
ATTR-PN	687 (58.3)	52 (50.0)	261 (63.2)	204 (55.7)	108 (58.7)	52 (63.4)	10 (33.3)
Mixed	492 (41.7)	52 (50.0)	152 (36.8)	162 (44.3)	76 (41.3)	30 (36.6)	20 (66.7)
Genotype, n (%)	n=970	n=86	n=351	n=301	n=146	n=64	n=17
Hereditary	592 (61.0)	49 (57.0)	222 (63.2)	182 (60.5)	83 (56.8)	39 (60.9)	17 (77.3)
Wild-type	117 (12.2)	14 (16.3)	44 (12.5)	45 (15.0)	9 (6.2)	5 (7.8)	0 (0.0)
Unconfirmed (not tested)	261 (26.9)	23 (26.7)	85 (24.2)	74 (24.6)	54 (37.0)	20 (31.3)	5 (22.7)
Disease duration (years), median (IQR)	n=931 3.1 (1.7-5.4)	n=83 2.3 (1.0-5.0)	n=346 2.9 (1.2-4.9)	n=288 3.3 (1.8-5.8)	n=133 3.6 (2.2-6.0)	n=64 4.7 (2.9-7.2)	n=17 5.7 (3.3-9.4)

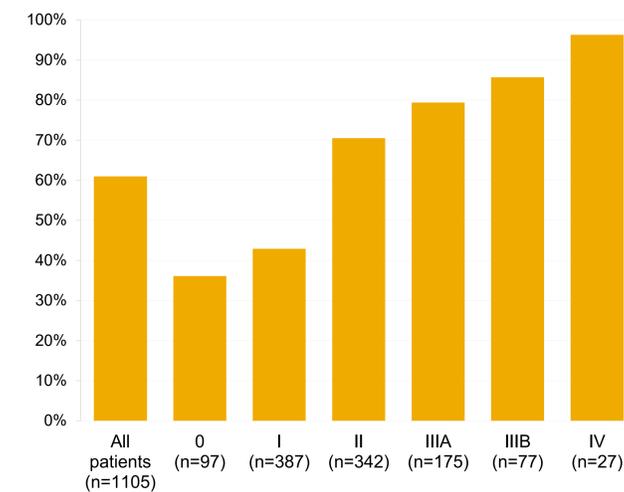
IQR; Interquartile Range, PND; Polyneuropathy Disability, SD; Standard deviation.

- Utilisation of mobility aids and home modifications due to ATTR-PN are summarised in **Figure 1**.
  - The 3 most frequently utilised mobility aids were: cane/walking stick (31.0%), wheeled walker (7.0%), walking frame (4.6%).
  - The 3 most frequent home modifications were: installation of grab bars or railings (18.1%), a shower chair (17.6%), or ramps (11.2%).
- Utilisation of caregiver support is summarised in **Figure 2**.

**Figure 1. Proportion of patients using mobility aids and home modifications at the time of survey, across PND scores**

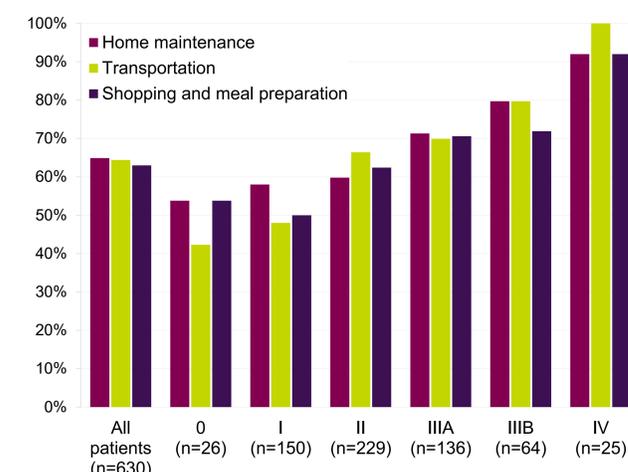


**Figure 2. Proportion of patients receiving caregiver support at the time of survey, across PND scores**

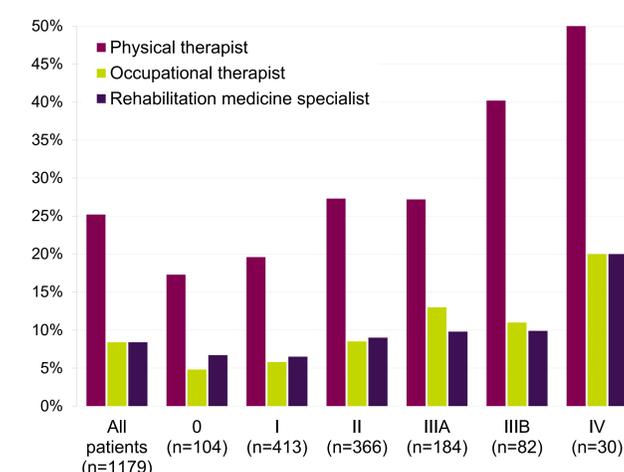


- Among those with a caregiver, activities that frequently required additional support included home maintenance, transportation, and shopping and meal preparation (**Figure 3**).
- Utilisation of physical therapy, occupational therapy, and rehabilitation medicine, within the 12 months prior to the survey, is summarised in **Figure 4**.

**Figure 3. Proportion of patients requiring support with home maintenance, transportation, and shopping and meal preparation, across PND scores**



**Figure 4. Proportion of patients consulting with supportive therapists within in the 12 months prior to survey, across PND scores**



## Conclusions



High levels of disability and impairment were observed in patients with ATTR-PN or mixed phenotype.



Many patients at more advanced stages of disease progression required physical therapy or other supportive approaches to help manage their condition.



Most patients required caregiver support with their activities of daily living, and many had modified their homes to better accommodate their condition.



Earlier detection and treatment of ATTR-PN may help to reduce the burden on both patients and caregivers.

## Limitations

- This study was based on a pragmatic rather than true random sample; in addition to the inclusion criteria, while minimal, physician and patient participation was influenced by willingness to complete the questionnaires.

## Disclosures

- GC, KR, and SG are employees of AstraZeneca and have ownership, options, and/or interests in AstraZeneca stock.
- JW, JGW, and AB are employees of Adelphi Real World.
- This study used data from the Adelphi Real World ATTR DSP, an independently conducted survey. The DSP is a wholly owned Adelphi Real World product. AstraZeneca were one of multiple subscribers to the DSP, and funded the analysis described here.

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