Epidemiology Of Hereditary Transthyretin (hATTR) Amyloidosis: A Real-World Analysis Of A US Commercially Insured Population

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INTRODUCTION

- hATTR amyloidosis is a rare genetic, progressive, and fatal disease caused by build-up of misfolded transthyretin protein (amyloid) in organs and tissues[1]
- The recent incidence of hATTR amyloidosis in the US is not well documented[2,3]
  - A 1996 estimate reported the US incidence to be one in 100,000 individuals[4]
  - Rates are most likely underestimated due to a lack of awareness and diagnostic uncertainty[2,5]
- This study's objective was to generate a recent US estimate of diagnosed incidence of hATTR amyloidosis, focusing on patients with hATTR-associated polyneuropathy and/or mixed phenotype

METHODS

Study Design

- Retrospective study using IBM® MarketScan® Commercial and Medicare Supplemental databases* from 01/01/2013 – 12/31/2017

Patient Identification

- Included adult patients, ≥18 years, who were newly diagnosed with hATTR amyloidosis (incident cases) in 2016
  - ≥1 medical claim with a relevant diagnosis code for amyloidosis (ICD-10-CM: E85.0-4, E85.89, E85.9; excludes light chain and wild type) in the calendar year (CY) of 2016 AND ≥1 occurrence of qualifying criteria for hATTR any time during study period:
    - ≥15 days diflunisal use without >30-day gap OR
    - liver transplant OR
    - claim with code E85.1 or E85.2
  - All disease-free enrollees (continuously enrolled and without a diagnosis code of amyloidosis in CY2015) were included

Study Measures

- Annual diagnosed incidence in 2016 calculated as follows:
  - Number of new patient cases of hATTR in CY 2016 divided by total at-risk (disease-free) patient years from January 1, 2016, until diagnosis (cases) or enrollment end (non-cases) in CY 2016
  - Incidence reported as cases per million person-years (PMPY)
    - Enrollment was continuous during at-risk period

Statistical Analysis

- Incidence rates stratified by age group and gender
- All data transformations and statistical analyses were performed using SAS© version 9.4

* MarketScan is a trademark of IBM Corporation in the United States and other countries.

RESULTS (Table 1, Figures 1-3)

- Annual diagnosed incidence of hATTR in 2016 was 9.0 patients PMPY
- Incident cases were concentrated in older age groups (65+ years: 23.3, 55-64 years: 14.6, 35-54 years: 5.8, 18-34 years: 2.2 PMPY) and slightly more common among females than males (9.6 vs. 8.3 PMPY)

Table 1. hATTR Amyloidosis 2016 Incidence by Demographic Groups

	N	Number of Cases per Million Person-Years
All	142	9.0
Age group		
18-34	9	2.2
35-54	35	5.8
55-64	53	14.6
65+	45	23.3
Gender		
Female	80	9.6
Male	62	8.3

RESULTS continued

Figure 1. Age Distribution among New Cases of hATTR in 2016

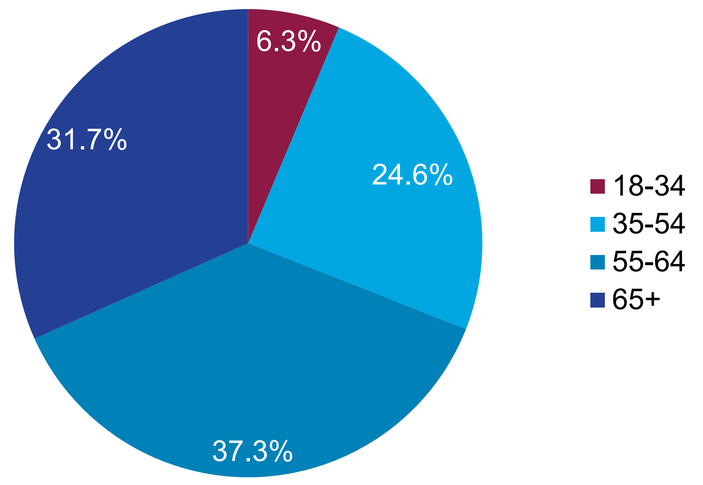


Figure 2. Gender Distribution among New Cases of hATTR in 2016

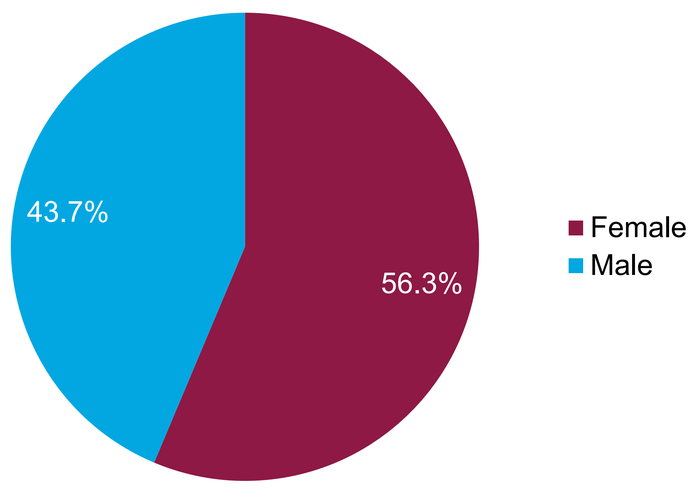
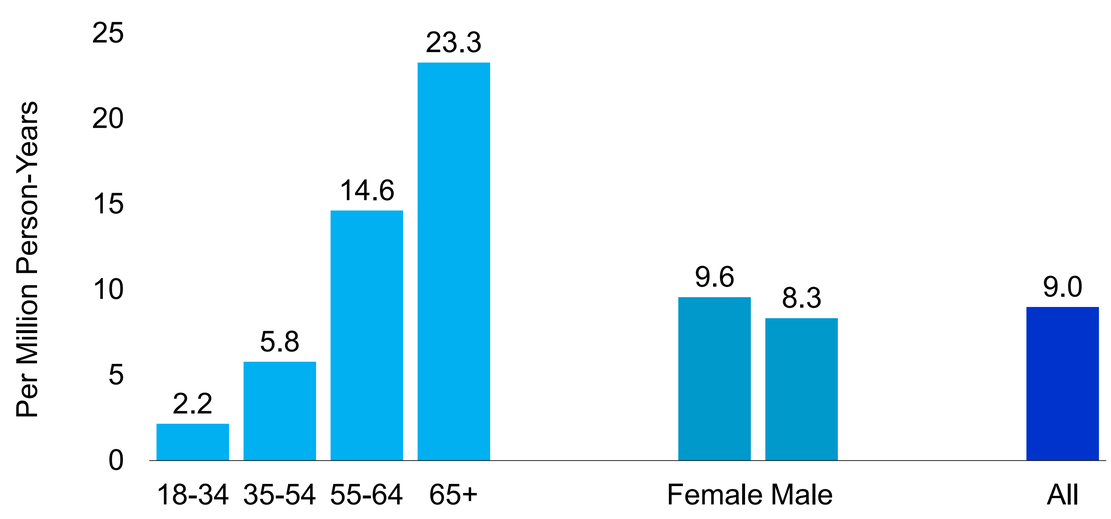


Figure 3. 2016 hATTR Incidence



LIMITATIONS

- Estimation of hATTR incidence using claims has not been previously validated; such estimation is difficult due to diagnostic challenges such as lack of awareness of the disease and, until recently, the absence of medical coding specific to hATTR
- This database has an underrepresentation of senior patients (65+);[6] because seniors have the highest rate of new cases of hATTR, the overall incidence in 2016 is likely underestimated

CONCLUSIONS

- The epidemiology of hATTR amyloidosis is not well understood or quantified
- We ascertained, using claims, a small but meaningful number of new patients diagnosed with hATTR in the US in 2016: a diagnosed incidence rate of 9.0 cases PMPY
- Consistent with previous studies, new cases are predominately of advanced age
- Future estimation of prevalence is planned

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