HEALTHCARE UTILIZATION AND COST AMONG PATIENTS WITH HEREDITARY TRANSTHYRETIN AMYLOIDOSIS (hATTR)

Spencer Guthrie, MBA1; Sheila R. Reddy, PhD, RPh2; Ryan Tieu, MS3; Jennifer Munday, PhD, MPH4; Marian Tarbox, MPP5; Michael S. Broder, MD, MS6; MS7; Michael R. Pollock7

1 Akcea Therapeutics, Cambridge, MA, USA; 2 Partnership for Health Analytic Research, LLC, Beverly Hills, CA, USA

OBJECTIVE

• hATTR is a rare genetic, progressive and fatal disease caused by build-up of misfolded transthyretin protein (amyloid) in organs and tissues.1
• Diagnosis and treatment of hATTR remains challenging.2
• FDA-approved therapies do not currently exist.

METHODS

• Retrospective study using Truven Health Analytics MarketScan® Commercial and Medicare Supplemental databases and the IQVIA™ Real-World Data Adjudicated Claims - US databases from 1/1/2012-12/31/2016 (study period).
• Patient identification (Figure 1): Patients ≥18 years old who were newly diagnosed with hATTR:
  • ≥1 medical claim with a relevant diagnosis code (ICD-9-CM 277.30-31, 277.38; ICD-10-CM E85.0-4, E85.82-89, E85.9) between 7/1/2012-9/30/2016 (ID period) and ≥1 additional qualifying criteria occurring any time during study period: ≥2 days different usage with >30-day gap, liver transplant, or claim with ICD-10-CM codes E85.1 or E85.2.
  • Study index: date of first claim in the ID period with a diagnosis code for amyloidosis.
• Patients enrolled during baseline period and followed for ≥3 months post-index until enrollment or study end, whichever occurred first.
• Study measures:
  • Baseline age, gender, comorbidities, and year of diagnosis.
  • Outcome: HCRU and costs reported quarterly (every 3 months; Q1 through Q4).
• This study was sponsored by Akcea Therapeutics, Inc.

RESULTS

• Patients with ED visit during study period: ≥15 days diflunisal use within 6 months continuous (N = 277, ≥30 days continuous (N = 231), had claim for hATTR (Table 2)
  • N = 349
  • 129 (29.9) had claim for amyloidosis by estimating healthcare resource utilization (HCRU) and costs for amyloidosis.
• Descriptive statistics, including means, standard deviations (SD), and relative frequencies and percentages for continuous and categorical data, respectively, were reported.
• All data transformations and statistical analyses were performed using SAS version 9.4.

CONCLUSION

• Patients newly diagnosed with hATTR have substantial HCRU and costs in the first year after diagnosis.
• As in common fatal diseases, progression in hATTR is associated with high costs. This may be particularly true in the terminal phase.
• This study has potential limitations:
  • Results for each quarter represent slightly different populations, however patient characteristics are similar in all examples.
  • Our study presents an incomplete assessment of the burden of hATTR, as we had no data on indirect costs (e.g., due to productivity losses or caregiver burden) or on quality of life.

REFERENCES


Figure 1. Patient Identification

Figure 2. Baseline Characteristics

Figure 3. Therapeutic Procedures and Devices in the First Year after hATTR Diagnosis

Figure 4. Healthcare Costs in the First Year after hATTR Diagnosis

Table 1. Patient Characteristics

Table 2. Healthcare Utilization in the First Year after hATTR Diagnosis

Table 3. Healthcare Costs in the First Year after hATTR Diagnosis

Baseline

• Among 432 qualifying newly diagnosed patients (Figure 1), mean age was 57.5 (SD: 14.1), 52.9% were female, and baseline Charlson comorbidity index was 1.9 (2.9) (Table 1).
• By one year post-index, enrollment decreased to 179.

Selected baseline comorbidities are shown in Figure 2.

Cardiovascular-related comorbidities varied: dyspnea (27.3%), edema (17.1%), congestive heart failure (15.7%), ventricular hypertrophy (11.6%), and restrictive cardiomyopathy (10.5%); ischemic stroke, aortic stenosis, and hypertrophic cardiomyopathy occurred in ≥10% of cases. Nearly a quarter of patients experienced diabetes (24.5%). In nervous system-related comorbidities, 15.5% experienced neuropathy (4.0% peripheral, 4.0% peripheral, while low back pain experienced cognitive impairment or incontinence (3.3% and 3.7%, respectively).

For gastrointestinal-related comorbidities, 11.1% experienced diarrhea and 14.4% constipation.

Figure 3. Therapeutic Procedures and Devices in the First Year after hATTR Diagnosis

Table 3. Healthcare Costs in the First Year after hATTR Diagnosis

RESULTS

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Claims are meant for reimbursement, not research, so misclassification of hATTR is possible.