### Background

- hATTR is a genetic, progressive, and fatal form of amyloidosis caused by extracellular deposition of transthyretin amyloid fibrils.
- Diagnosis of hATTR remains a challenge, and there are no FDA-approved therapies for the treatment of hATTR.
- Liver transplantation is sometimes used with or without accompanying heart transplant, but this treatment is limited to few patients.
- Current literature on the economic burden of amyloidosis is limited, and there is no current estimate of the cost of hATTR.

### Objective

To estimate hATTR-related healthcare utilization and costs.

### Methods

- Retrospective study using Truven Health Analytics MarketScan® Commercial and Medicare Supplemental databases and the IQVIA Real-World Data Adjudicated Claims - databases from 1/1/2012-12/31/2016.
- Patient identification:
  - Patients ≥18 years at index date (defined below) who were newly diagnosed with hATTR.
  - Diagnosis of hATTR defined as: ≥2 medical claims with a relevant diagnosis code for amyloidosis (ICD-9-CM 270.31-270.39, 271.30; ICD-10-CM E85.0-E85.82, E86.89, E89.0) between 7/1/2012-12/31/2016 (index); PLUS ≥1 additional occurring at any time during the study period (2012-2016):
    - ≥15-days of diflunisal use without >30 day gap; liver transplant; OR claim with ICD-10-CM codes E85.1 or E85.2.
  - Data of first claim with diagnosis code for amyloidosis was defined as the index date.
  - Patients had continuous enrollment in the 6 months prior to index diagnosis (baseline period) and during ≥3 months post-index.
  - Patients having a diagnosis code for amyloidosis during baseline were excluded to ensure new diagnosis.
  - Observation period: Newly diagnosed patients were followed 270 days post-index to enrollment or study end, whichever came first.
  - Study measures:
    - First-year healthcare utilization and costs reported by quarter among patients still enrolled.
      - Hospitalization, outpatient services (e.g., ED and physician office visits), therapeutic procedures and devices, and pharmacy utilization.
      - Total, outpatient, inpatient (ED and non-ED services), organ transplant-related, and outpatient pharmacy costs.
  - Baseline characteristics (e.g., age, gender, comorbidities) and year of diagnosis.
- Statistical analysis:
  - 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.

### Results

<table>
<thead>
<tr>
<th>Table 1: Patient Identification</th>
<th>Database</th>
<th>Cases</th>
<th>1st Quarter</th>
<th>2nd Quarter</th>
<th>3rd Quarter</th>
<th>4th Quarter</th>
</tr>
</thead>
<tbody>
<tr>
<td>Charlson = 0</td>
<td>MarketScan</td>
<td>IQVIA</td>
<td>12,620</td>
<td>10,434</td>
<td>11,462</td>
<td>10,606</td>
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<td>Charlson = 1</td>
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<td>725</td>
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<td>IQVIA</td>
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<td>50</td>
<td>43</td>
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<tr>
<td>Charlson = 3</td>
<td>MarketScan</td>
<td>IQVIA</td>
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<td>26</td>
<td>33</td>
<td>26</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Table 2: Patient Characteristics</th>
<th>Mean (SD)</th>
<th>272 days</th>
<th>360 days</th>
<th>90 days</th>
<th>180 days</th>
<th>270 days</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age (years)</td>
<td>75.4 (10.5)</td>
<td>272 days</td>
<td>360 days</td>
<td>90 days</td>
<td>180 days</td>
<td>270 days</td>
</tr>
<tr>
<td>Gender (male)</td>
<td>52.5%</td>
<td>272 days</td>
<td>360 days</td>
<td>90 days</td>
<td>180 days</td>
<td>270 days</td>
</tr>
<tr>
<td>Charlson Comorbidity Index</td>
<td>1.2 (2.5)</td>
<td>272 days</td>
<td>360 days</td>
<td>90 days</td>
<td>180 days</td>
<td>270 days</td>
</tr>
</tbody>
</table>

### Conclusion

- Patients with hATTR use significant healthcare resources and costs over time, even in the first year after diagnosis.
- This study adds to the limited understanding about the burden faced by patients with hATTR. However, further research over a longer time period with a larger sample is warranted to assess whether costs increase with disease progression, including near the end of life, similar to other fatal diseases.
- Limitations:
  - Quarterly outcomes could not be compared directly due to changing samples based on enrollment; however, we found that the quarterly samples had similar demographics and comorbidities and believe utilization and costs in each quarter would reflect a representative of the full sample of 452 patients.
  - Claims are meant for reimbursement, not research, so misclassification of patients with hATTR is possible.

This study examines only direct healthcare costs and does not include indirect costs such as decreased quality of life or productivity, which add to the picture of burden.

### References