OBJECTIVE
• Hereditary transthyretin (hATTR) amyloidosis is a rare, progressive, multisystem, and fatal form of amyloidosis caused by extracellular deposition of transthyretin amyloid fibrils primarily synthesized by the liver.1,2
• The economic burden of hATTR amyloidosis remains difficult to quantify due to the disease’s rarity and the wide variety of clinical presentations.1,2
• We found estimates of the disease cost in existing literature.
• The objective of this study was to estimate healthcare resource utilization (HCRU) and costs associated with newly diagnosed hATTR amyloidosis.

METHODS
• Retrospective study using IBM MarketScan® Commercial and Medicare Supplemental databases (from 01/01/2014-12/31/2017).
• Patient identification
• Included adult patients (≥18 years of age at index) newly diagnosed with hATTR amyloidosis
  • Medical claim with diagnosis codes (ICD-9-CM 277.3, ICD-10-CM E85.1, E85.2; E85.3 includes right-hand and valid type) identified during the index (01/01/2015-12/31/2016) AND occurrence of qualifying criteria for hATTR any time during the study period
  • >15 days diffusion era without 30-day gap ≥1 liver transplant (patients with claim code E85.3 or E85.2 at any time did not require additional qualifier)
• Study index: date of first claim in the 6 month period with diagnosis code for amyloidosis
• Patients enrolled 1 year prior (baseline) and followed 1 year post index (post follow-up)
• To ensure a new diagnosis, patients with amyloidosis diagnosis in baseline period excluded
• The fixed 12-month follow-up period would have excluded those who died within 12 months of diagnosis, which might have resulted in an underestimate of cost.
• Charlson Comorbidity Index (CCI)
• Demographics (age, gender, region, insurance type)
• Total, inpatient, outpatient medical (ED and non-ED services), and outpatient pharmacy costs

RESULTS
• Among 31,025 newly diagnosed patients, mean age was 59.2 (SD:15.2), 54.1% were female, and baseline Charlson Comorbidity Index was 2.2 (2.5) (Table 1).
• We found no estimates of the disease cost in existing literature.
• The objective of this study was to estimate healthcare resource utilization (HCRU) and costs associated with newly diagnosed hATTR amyloidosis.

CONCLUSIONS
• Patients newly diagnosed with hATTR amyloidosis have substantial HCRU and costs in the first year following diagnosis, with the largest proportion of costs occurring in the first quarter after diagnosis.
• Further research should examine later costs associated with disease progression and end-of-life care.
• This study has potential limitations:
  - The reported estimates of economic burden associated with hATTR amyloidosis may be underestimated as until recently there were no ICD codes specifically for hATTR amyloidosis, and our approach to patient identification has not been validated using medical records.
  - However, the majority of patients in the final sample were included because they had codes for hATTR amyloidosis (ICD-10-CM E85.3 or E85.2) increasing our confidence that the correct population was identified.
  - The fixed 12-month follow-up period would have excluded those who died within 12 months of diagnosis, which might have resulted in an underestimate of cost.
  - Additionally, this study examines only direct healthcare costs and has not included indirect costs such as decreased quality of life or productivity, which add to the picture of disease burden.

REFERENCES
4. The annual mean (SD) total cost was $64,066 (214,317), with inpatient services contributing the majority of the expenses ($34,461, 24.9%). Follow-up was associated with increased median prescription fills (29 vs. 29.7) (quarterly values shown in Table 2).

Table 1. Patient Demographics and Baseline Charlson Comorbidity Index

<table>
<thead>
<tr>
<th>Age, years, mean (SD)</th>
<th>Gender, n (%)</th>
<th>Region, n (%)</th>
<th>Insurance type, n (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>59.2 (15.2)</td>
<td>54.1% Female</td>
<td>36.8% West</td>
<td>52.2% PPO/POS</td>
</tr>
<tr>
<td>18-34</td>
<td>7.6%</td>
<td>64 (34.6%)</td>
<td>122 (65.9%)</td>
</tr>
<tr>
<td>35-54</td>
<td>21.8%</td>
<td>68 (36.8%)</td>
<td>21 (11.4%)</td>
</tr>
<tr>
<td>55-64</td>
<td>47.0%</td>
<td>62 (33.5%)</td>
<td>56 (30.1%)</td>
</tr>
<tr>
<td>≥65</td>
<td>24.5%</td>
<td>63 (34.1%)</td>
<td>32 (17.2%)</td>
</tr>
</tbody>
</table>

Table 2. Healthcare Utilization During 1-Year Follow-up and Stratified by Quarter

<table>
<thead>
<tr>
<th>Post Q1</th>
<th>Post Q2</th>
<th>Post Q3</th>
<th>Post Q4</th>
<th>Year Follow-up</th>
</tr>
</thead>
<tbody>
<tr>
<td>Inpatient hospitalizations, n (%)</td>
<td>103 (55.6)</td>
<td>103 (55.6)</td>
<td>103 (55.6)</td>
<td>103 (55.6)</td>
</tr>
<tr>
<td>Inpatient days among all patients, mean (SD)</td>
<td>8 (7.1)</td>
<td>8 (7.1)</td>
<td>8 (7.1)</td>
<td>8 (7.1)</td>
</tr>
<tr>
<td>Inpatient days among patients with diagnosis code E85.3 or E85.2, mean (SD)</td>
<td>12.4 (7.2)</td>
<td>12.4 (7.2)</td>
<td>12.4 (7.2)</td>
<td>12.4 (7.2)</td>
</tr>
</tbody>
</table>

Figure 1. Comorbidities in 1-Year Follow-up

Figure 2. Healthcare Costs (Adjusted to 2017 US$) During 1-Year Follow-up

Table 3. Cost of Healthcare During 1-Year Follow-up and Stratified by Quarter

<table>
<thead>
<tr>
<th>Post Q1</th>
<th>Post Q2</th>
<th>Post Q3</th>
<th>Post Q4</th>
<th>Year Follow-up</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total, inpatient, outpatient medical, and outpatient pharmacy costs, mean (SD)</td>
<td>$64,066 (214,317)</td>
<td>$64,066 (214,317)</td>
<td>$64,066 (214,317)</td>
<td>$64,066 (214,317)</td>
</tr>
</tbody>
</table>


Statistical analysis
• Descriptive statistics, including means, standard deviations (SD), and relative frequencies and percentages for continuous and categorical data, respectively, reported.
• All data transformations and statistical analyses performed using SAS® version 9.4.