**BACKGROUND**

- Sickle cell disease (SCD) is a genetic disorder in which hemoglobin is abnormal, causing cells to become rigid and less able to flow through blood vessels, leading to painful crises, organ damage, and early mortality.
- Children with SCD face increased risks for infections, stroke, heart disease, and certain cancers.
- Clinical guidelines for pediatric SCD patients recommend vaccinations, prophylactic penicillin, and ophthalmology screening.

**OBJECTIVE**

- To describe the use of vaccines, penicillin, TCD screening, and ophthalmology care in a large cohort of children with HbSS disease.

**METHODS**

Study Design and Data Sources

- This is a retrospective administrative claims database analysis among a sample of Commercial and Medicaid insured populations in the United States.
- These databases provide detailed outcomes measures including outpatient pharmacy claims, resource utilization, and associated costs for healthcare services performed in both outpatient and inpatient settings for over 40 million individuals.
- Identified US administrative claims data from January 1, 2009 through December 31, 2014 were extracted from the Truven Health MarketScan® Claims Databases and Medicaid Database.
- Only regional geographic data is available for Commercial patients in this dataset.

Data Analysis

- The proportion of patients with a pneumococcal vaccine among children 1-5 years of age was underreported in the Truven Health MarketScan® Research Databases.
- The high penicillin utilization among patients age 1-5 years was consistent with guideline recommendations and offered a partial validation of the dataset.

**RESULTS**

**Table 1. Annual Cohorts**

<table>
<thead>
<tr>
<th>Year</th>
<th>Commercial</th>
<th>Medicaid</th>
</tr>
</thead>
<tbody>
<tr>
<td>2009</td>
<td>346</td>
<td>400</td>
</tr>
<tr>
<td>2010</td>
<td>346</td>
<td>400</td>
</tr>
<tr>
<td>2011</td>
<td>346</td>
<td>400</td>
</tr>
<tr>
<td>2012</td>
<td>346</td>
<td>400</td>
</tr>
<tr>
<td>2013</td>
<td>346</td>
<td>400</td>
</tr>
<tr>
<td>2014</td>
<td>346</td>
<td>400</td>
</tr>
</tbody>
</table>

**Average Age, Mean (SD)**

<table>
<thead>
<tr>
<th>Commercial Patients</th>
<th>Medicaid Patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>9.5 (4.4)</td>
<td>8.2 (4.3)</td>
</tr>
</tbody>
</table>

**Table 2. Average Patient Characteristics (all years)**

<table>
<thead>
<tr>
<th>Geographic Location</th>
<th>Commercial Patients</th>
<th>Medicaid Patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>Urban</td>
<td>351 (31%)</td>
<td>1,064 (82%)</td>
</tr>
<tr>
<td>Rural</td>
<td>224 (20%)</td>
<td>238 (18%)</td>
</tr>
<tr>
<td>Unknown</td>
<td>7 (2%)</td>
<td>1 (0%)</td>
</tr>
</tbody>
</table>

**Proportion of patients with penicillin use was consistently greater among Medicaid patients than among Commercial patients.**

- For both payer populations, over 80% of patients age 1-5 years used penicillin.
- No marked differences in results across payers and age groups observed when stratified by urban/rural status.

**Figure 3. Average Prior Year TCD Use**

- Across payers and age groups, there was a proportional use of TCD utilization for stroke prevention.
- The proportion of patients with a pneumococcal vaccine among children 1-5 years of age was underreported in the Truven Health MarketScan® Research Databases.
- The high penicillin utilization among patients age 1-5 years was consistent with guideline recommendations and offered a partial validation of the dataset.

**LIMITATIONS**

- The limitations of this study are that it is a retrospective analysis of administrative claims data and therefore subject to the inherent limitations of claims-based research.
- Data are limited to those captured in claims and identification of a diagnosis codes only reflect the claims submitted by the physician for reimbursement.
- Potential for misclassification of SCD and HbSS: The proportion of patients with a pneumococcal vaccine among children 1-5 years was underestimated as this age group includes patients for which capture of booster vaccinations may be incomplete in the study period.
- Flu and pneumococcal vaccines may be underreported as vaccines provided by state/local agencies are not captured in these databases.
- Medication data indicate drugs administered in a physician’s office or filled through an outpatient pharmacy, but data do not represent if the patient used the medication as prescribed.

**SUMMARY**

- The high penicillin utilization among patients age 1-5 years was consistent with guideline recommendations and offered a partial validation of the dataset.
- Immunization against pneumococcus was surprisingly poor in this cohort.
- The proportion of patients with a pneumococcal vaccine among children 1-5 years of age was underreported in the Truven Health MarketScan® Research Databases.
- The high penicillin utilization among patients age 1-5 years was consistent with guideline recommendations and offered a partial validation of the dataset.

**CONCLUSIONS**

- Appropriate use of TCD screening for stroke prevention is especially warranted among children where automatic chronic transfusion therapy is a proven method of prevention against this potentially devastating complication.
- Further research is needed to better understand potential barriers to proper screening in addition to evaluating strategies to improve awareness, adherence and implementation of recommended screening in children with SCD.

**References**

4. Available at http://www.ncbi.nlm.nih.gov/pmc/articles/PMC5619754/

**Disclosures**

- This project was funded in full by Global Blood Therapeutics. Authors LB, JS, and SJC are employees of Global Blood Therapeutics, LLC, an employee of Vertex Pharmaceuticals Inc. CD is employee of Emory University, Forsyth Institute, and Global Blood Therapeutics. JK and RH are employees of Global Blood Therapeutics. IA, and RH are employees of Global Blood Therapeutics, LLC, an employee of Thomas Jefferson University. RF is an employee of Global Blood Therapeutics, LLC, an employee of Emory University, Forsyth Institute, and Global Blood Therapeutics. The authors have no relevant financial relationships to disclose.

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