BACKGROUND

Immune thrombocytopenia purpura (ITP) is an acquired immune-mediated disease, leading to low platelet counts and an increased risk of bleeding (Brown et al., 2009). ITP can be asymptomatic but some patients experience symptoms ranging from spontaneous bruising to serious mucocutaneous bleeding (Prown, et al. 2010; Newell et al., 2011).

One of the goals of ITP treatment is to prevent major bleeding (Brown, et al., 2012). Patients with ITP require pharmacological therapies, whole blood transfusions, and hospitalizations due to bleeding, which incurs substantial costs (Sahib, et al., 2009).

In addition, side effects due to ITP treatments can be a burden on patients (Brown, et al. 2012).

Once treatment with epoetin alfa and romiplostim are the most commonly used second-line ITP treatments, limited published data using real-world evidence exists on the cost burden of ITP.

OBJECTIVES

The objective of this retrospective real-world evidence (RWE) study was to examine resource use and costs in ITP patients treated with epoetin alfa (EPAG) or romiplostim (ROMI).

METHODS

DATA SOURCES

This is a retrospective study using data collected from suspects of a syndicated network of electronic medical records (EMR) from 26 US hospital institutions treating over 27 million patients, was searched

• TRIHEx with

 o Inpatient and outpatient services and procedures
 o Disease diagnoses
 o Prescription drugs
 o Laboratory results

The data were queried in real time in April 2017 for analyses of BRETs to identify abnormal platelets.

In order to set up mutually exclusive cohorts without confounding factors, adult patients diagnosed with primary ITP and treated with epoetin alfa or romiplostim were included on:

• Prior steroid treatment: prednisone, methylprednisolone, dexamethasone,

• History of Hematopoietic B, C, H or Human Immunodeficiency Virus

• Malignancy

• Hematologic condition: severe aplastic anemia, myelodysplastic syndrome, and myelofibrosis

• Spleenectomy

The list of secondary disorders (hematologic conditions, virus infections, and malignancy conditions) was derived from a previously published study (Lin, et al., 2017).

After excluding confounders, patients were divided into mutually exclusive cohorts treated with EPAG or ROMI.

RESOURCE USE AND COSTS

A total of four years of patient data were analyzed through TRIHEx

The rates of rescue therapy use, adverse events (AEs) and use of routine health care were collected over 12 months following treatment initiation

The rates of platelet transfusion, IVig, and IV methylprednisolone were recorded as rescue therapy use

The list of AEs of interest was constructed based on the FDA labels for EPAG and ROMI

The rates of office visits, ER visits, and hospitalizations were collected as routine healthcare use

UNIT COSTS

- Treatment costs for EPAG and ROMI per patient per year were calculated using drug costs per week and administration costs per week

- Unit costs obtained from various public sources were applied to the rates of rescue therapy use to calculate the costs per patient per year for each resource

- Annual total costs per ITP patient receiving EPAG or ROMI as second-line were calculated by adding total treatment costs, total costs for rescue therapies, total AE costs, and costs for routine healthcare use

- 95% confidence intervals (CIs) were calculated for each resource use, except the drug use, and used to calculate the CIs for the total costs.

RESULTS

TREATMENT COSTS PER YEAR

A total of 1,030 patients were identified after cohort matching: Among them, 650 and 380 patients received EPAG and ROMI as second-line therapy, respectively.

- Total treatment costs per patient per year were calculated based on unit costs for drugs and administration

- Treatment and administration costs were lower for the EPAG cohort compared to the ROMI cohort

RESCUE MEDICATION COSTS

- ROMI cohort had higher rates of rescue medication use over 12 months following drug initiation compared to EPAG

- ROMI cohort showed higher total costs for rescue therapies per patient per year compared to EPAG

ROUTINE HEALTHCARE COSTS

- Although EPAG cohort had higher rates of office visits and emergency department visits as compared to ROMI; the ROMI cohort showed higher rates of diagnostic testing, and hospitalizations

- Total health care costs were higher for ROMI compared to EPAG, mainly driven by hospitalization costs

ADVERSE EVENT COSTS

- ROMI cohort showed higher rates of AEs compared to EPAG with the exception of diarrhea and upper respiratory tract infections, leading to higher overall AE related costs

TOTAL COSTS

- Total annual treatment per patient costs were $71,632 for EPAG and $84,432 for ROMI

- ROMI cohort showed higher costs for all four sub-categories: treatment and administration, rescue medications, adverse events, and health care costs

- Treatment cost accounted for approximate 87% and 86% of the total cost for EPAG and ROMI, respectively

Table 1: Treatment costs per patient per year (US$)

<table>
<thead>
<tr>
<th>EPAG (N=650)</th>
<th>ROMI (N=380)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Drug Costs Per Week</td>
<td>2,392</td>
</tr>
<tr>
<td>Administration Costs Per Week</td>
<td>0</td>
</tr>
<tr>
<td>TOTAL TREATMENT COSTS</td>
<td>62,202</td>
</tr>
</tbody>
</table>

Table 2: Rates of rescue medications and costs (US$)

<table>
<thead>
<tr>
<th>EPAG (N=650)</th>
<th>ROMI (N=380)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Platelet Transfusion</td>
<td>61.6% (4.40%;8.30%)</td>
</tr>
<tr>
<td>IV Ig</td>
<td>6.15%</td>
</tr>
<tr>
<td>N/Methylprednisolone</td>
<td>10.5%</td>
</tr>
<tr>
<td>Costs for rescue medications per patient per year, US$</td>
<td>278</td>
</tr>
<tr>
<td>IV Methylprednisolone</td>
<td>60</td>
</tr>
</tbody>
</table>

Table 3: Total costs in ITP for EPAG vs. ROMI (US$)

<table>
<thead>
<tr>
<th>EPAG (N=650)</th>
<th>ROMI (N=380)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total Costs</td>
<td>62,202</td>
</tr>
<tr>
<td>Total AE Costs</td>
<td>4,927</td>
</tr>
<tr>
<td>Total Health Care Costs</td>
<td>5,743</td>
</tr>
<tr>
<td>Total Health Care (95% CI)</td>
<td>(60,81;72,73)</td>
</tr>
<tr>
<td>Total AEs</td>
<td>2,382</td>
</tr>
<tr>
<td>Total ADVERSE EVENT COSTS (95% CI)</td>
<td>4,927 (7,46;8,34)</td>
</tr>
<tr>
<td>Total Costs Contributed by Other Costs</td>
<td>12%</td>
</tr>
<tr>
<td>Total Costs Contributed by Drug Costs</td>
<td>87%</td>
</tr>
</tbody>
</table>

CONCLUSIONS

This retrospective RWE study reports annual economic burden of ITP treatment. Annual total costs of treatment were substantially lower for EPAG vs. ROMI.

LIMITATIONS

This study includes limitations regarding different electronic medical records with limited longitudinal data.

REFERENCES

5. For a complete list of references please contact: AnnaForsythe@pshta.com

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