BACKGROUND

- Immune thrombocytopenia purpura (ITP) is an acquired immune-mediated disease leading to low platelet counts and an increased risk of bleeding (Rodeghiero, et al. 2009).
- Estimated incidence for European and US populations are 3.3 per 100,000 persons per year and 1.6–2.6 per 100,000 persons per year, respectively (Frederiksen and Schmidt 1999; Terrell, et al. 2010).
- Bleeding related episodes (BREs) can be complicated by ITP in patients with certain comorbidities (Michel 2009).
- Patients with ITP may have severe symptoms and complications, which can be life-threatening (Lin, et al. 2017).
- Inconvenience (e.g. nose bleeds and heavy menstrual bleeding), and may disrupt the patient’s lifestyle and prevent them from taking part in everyday activities.
- Furthermore, the anxiety associated with the risk of bleeding can have a negative impact on quality of life (Michel 2009).
- Prevention of BREs is one of the goals of ITP therapy (Rodeghiero, et al. 2009).
- The overall aims of ITP treatment are to reduce patients’ risk of bleeding, decrease mortality, and increase QoL by elevating platelet counts, while minimizing treatment-related side effects.
- Current treatment guidelines cover many different agents:
  - First-line: Corticosteroids and immunoglobulins
  - Second-line: Thrombopoietin receptor agonists (TPO-RAs), rituximab, and splenectomy.
- Following first-line treatment with corticosteroids or immunoglobulins, there is no clearly defined pathway and treatment is personalized to each patient.
- TPO-RAs elotrombopag (EPAG) and romiplostim (ROMI) are the only drugs appropriately studied with double-blind randomized trials in ITP.
- TPO-RAs act to increase platelet production and are a valuable treatment option for patients who have relapsed on first-line therapy.
- They have demonstrated high efficacy and an acceptable safety profile, even in refractory patients.
- Although EPAG and ROMI are the most commonly used second-line treatments, no robust burden related studies have been conducted with patients in these real-world settings (Prow, et al. 2010; Neunert, et al. 2011).

OBJECTIVES

- The objective of this retrospective real-world evidence (RWE) study was to examine the burden of BREs in ITP patients treated with EPAG or romiplostim ROMI.

METHODS

DATA SOURCES

- TriNetX Live™ is a federated health research network of electronic medical records (EMR) from 26 US hospital institutions treating over 27 million patients
- TriNetX EMRs include:
  - Demographic data
  - Inpatient and outpatient services and procedures
  - Disease diagnoses
  - Prescription drugs
  - Laboratory results
- TriNetX Live™ provides real-time statistical results based on queries
- The “Query System” provided by the TriNetX Live™ platform was used to identify patients with specific ITP-10 codes.
- The data were queried in real-time in April 2017 for analyses of BREs to identify adult patients diagnosed with primary ITP

POPULATION

- Inclusion and exclusion criteria were set to select a population consistent with patients enrolled in EPAG and ROMI phase III trials
- The list of thrombocytopenia ICD-10 diagnoses was derived from a previously published study (Lin, et al. 2017). Table 1. Inclusion Criteria
- Exclusion Criteria
  - Patients with primary immune thrombocytopenia
  - Patients with secondary ITP, a history of HBV, HCV, or HIV, malignancy, idiopathic aplastic anemia, myelodysplastic syndrome, and myelofibrosis, and spleenectomy.

ICD-10 Condition

<table>
<thead>
<tr>
<th>Condition</th>
<th>EPAG (N=90)</th>
<th>ROMI (N=50)</th>
<th>P value</th>
<th>EPAG vs. ROMI</th>
</tr>
</thead>
<tbody>
<tr>
<td>D68.3</td>
<td>Immune thrombocytopenia purpura</td>
<td>20 (22%)</td>
<td>24 (48%)</td>
<td>0.026</td>
</tr>
<tr>
<td>D68.6</td>
<td>Thrombocytopenia, unspecified</td>
<td>21 (23%)</td>
<td>18 (36%)</td>
<td>0.039</td>
</tr>
</tbody>
</table>

Table 1: ICD-10 Diagnostic Codes for ITP

CONCLUSIONS

- This retrospective RWE study emphasizes the significant burden of BREs in ITP patients despite treatment which aims to prevent these episodes.
- BRE rates identified as 6% after controlling for confounding, were significantly higher in ROMI-treated compared to EPAG-treated patients.

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REFERENCES