Benefit-Risk Comparison in Patients with Immune Thrombocytopenia (ITP) Receiving Eltrombopag or Romiplostim: Real World Evidence (RWE) from 26 Hospital Institutions

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Identification of BREs and Statistical Analysis

BREs were identified as (Altomare, et al. 2016):
- Actual bleeding events based on bleeding codes and/or
- Uses of rescue therapy (intravenous immunoglobulin (IVIg) administration, intravenous (IV) steroid administration, or platelet transfusion, (RM)),
- Severe BREs were defined as BREs requiring platelet transfusions (BREP),
- Relapse: AEs only selected based on FDA prescribing information for EPAG and ROMI. Following an initial query for AEs that occurred at any time following the initiation of EPAG or ROMI, only AEs that occurred in >10% of patients were selected for further analysis.
- Incidence proportion of patients who experienced BREs, RM, use, platelet transfusions, and, and only AEs that occurred in >10% of patients were selected for further analysis.
- Incidence proportion of patients with each outcome and their 95% confidence intervals were descriptively compared for each outcome using 2-tailed p<0.05

RESULTS

- Among 1070 identified ITP patients (EPAG 680, ROMI 390) mean age was 60 years (SD: 18) and 51% were male.
- All patients diagnosed with primary ITP (ICD-10 D69.5) and treated with EPAG and ROMI were similar in age, prior steroid treatment, history of HBV/HCV/HIV, severe aplastic anemia, myelodysplastic syndrome, myeloﬁbrosis and splenectomy.

Bleeding Episodes

<table>
<thead>
<tr>
<th>BREs</th>
<th>EPAG (n=680)</th>
<th>ROMI (n=390)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Severe Bleeding</td>
<td>16.4%</td>
<td>22.1%</td>
</tr>
<tr>
<td>All Bleeding</td>
<td>26.0%</td>
<td>27.0%</td>
</tr>
<tr>
<td>Triggers</td>
<td>28.3%</td>
<td>21.6%</td>
</tr>
</tbody>
</table>

Relevant AEs were selected based on FDA prescribing information for EPAG and ROMI. Relevant AEs were selected based on FDA prescribing information for EPAG and ROMI.

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LIMITATIONS

- Restrictions associated with having access to only proportion populations lead to some important limitations:
  - The absence of regression analysis was addressed by manually examining differences in proportions across typical covariate variables, such as demographics. This method is imprecise because it requires an importance judgement on behalf of the researcher.
  - Differences in risk were addressed by manually examining differences between arms in imprecise because it requires an importance judgement on behalf of the researcher.
  - AUROCs may include data subject to change as patients progress through treatment. Some incorrect data may be not be recorded against conflicting data, or may become unavailable.
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CONCLUSIONS

- This is the first comparison of benefits and risks in patients with ITP treated with EPAG or ROMI that suggests a potential advantages of the EPAG use, especially the lower rates of RM use.

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