BACKGROUND

• Immune thrombocytopenic purpura (ITP) is an acquired immune-mediated disorder
  leading to low platelet counts and an increased risk of bleeding (Rodríguez, et al. 2015).

• Bleeding related episodes (BREs) are the most clinically important complication of
  ITP, they result in significant morbidity and mortality (Lin, et al. 2017, Neunert, et al. 2015).

• BRES also affect quality of life, their treatment requires hospitalizations or ER visits

• When patients have severe bleeding, transfusion is administered in a hospital
  setting to rapidly increase platelet levels (Arnold 2015).

• Current treatment guidelines include corticosteroids and immunoglobulins in first
  line, and thrombopoietin receptor agonists eltrombopag (EPAG) and romiplostim
  (ROMI), rituximab (RITUX), and splenectomy in second-line therapy. Although EPAG,
  ROMI, and RITUX are the most commonly used second-line ITP treatments, there is limited published real-world evidence on health care resource use (HCRU) attributed to ITP patients on these therapies and treated for BRES.

OBJECTIVES

• The objective of this retrospective real-world evidence (RWE) study was to assess
  the HCRU rates due to BRES in ITP patients treated with EPAG, ROMI, or RITUX.

METHODS

DATA SOURCES

• TriNetX, a syndicated network of electronic medical records (EMR) from 26 US
  hospital institutions and over 27 million patients, was searched.

• TriNetX EMR include:
  o Inpatient and outpatient services and procedures
  o Disease diagnoses
  o Prescription drugs
  o Laboratory results

• The data were queried in real time in May 2017 for analyses of BRES identified
  in adult patients diagnosed with primary ITP

TIME HORIZON

• We identified all patients with ITP from 4 years prior to the query date and compared
  the cumulative HCRU across different cohorts

POPULATION

• Inclusion and exclusion criteria were set to identify a population consistent with
  primary ITP as defined in ICD-10-CM (World Health Organization, 2016).

• The list of thrombocytopenia ICD-10 diagnoses was derived from the previously
  published study (Lin, et al. 2017),
  o D69.3 (ITP)
  o D69.4 (Other primary thrombocytopenia)
  o D69.6 (Thrombocytopenia, unspecified)

• Inclusion Criteria:
  o Patients ≥ 18 years old
  o Patients with primary immune thrombocytopenia
  o Patients treated with EPAG, ROMI, or RITUX
  o Patients with prior steroid treatment (prednisone, methylprednisolone, dexamethasone, hydrocortisone or triamcinolone) while on or before initiating ITP, EPAG, or ROMI were included

• Exclusion Criteria:
  o Patients with secondary ITP, or a history of HCV, HIV, malignancy, severe
  aplastic anemia, myeloproliferative syndrome, myelofibrosis and splenectomy were excluded.

• Analyses based on real-world evidence have certain limitations:
  - which may potentially lead to a substantial financial burden.

• A considerable proportion of patients with mild ITP were hospitalized or
  visited the ER and this cohort was associated with the highest rates of office visits (67% vs. 60% for severe BRES). This retrospective RWE study emphasized the significantly higher HCRU in patients with severe BRES as compared to patients without BRES.

• Severe BRES add additional burden of hospitalizations and emergency room visits, which may potentially lead to a substantial financial burden.

LIMITATIONS

• Analyses based on real-world evidence have certain limitations:
  - Interpretation of results should be considered while interpreting the results. No imputations of missing data were attempted in this study and as such, missing HCRU data may have lead to underestimation of true rates.

• The frequency of each event was not included in the EMR and therefore was not
  considered in the computation of total HCRU, hence, HCRU rates may have been underestimated.

• Multivariate adjustment for baseline characteristics was not conducted in this study to
  ensure cohort comparability. Such adjustment could have controlled for any possible confounding variables and should be considered in future studies.