EPH110

Real-world treatment patterns in adults living with primary chronic immune thrombocytopenia in the United States

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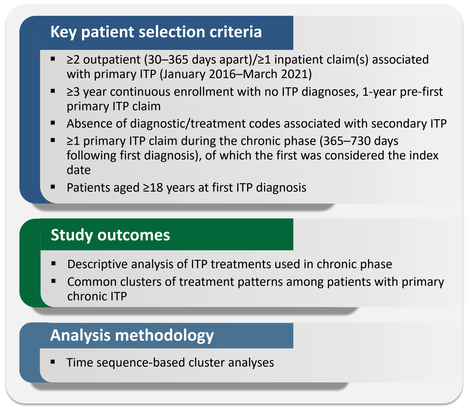
Introduction and Purpose

- Immune thrombocytopenia (ITP) is a rare autoimmune platelet disorder characterized by increased destruction and reduced production of platelets (platelet count of <100 x 10^9/L).[1]
- ITP presents varying unmet needs including 2 key factors:
  - Disease etiology: primary or idiopathic (occurring without an underlying cause)[2,3] and secondary (caused by comorbid/underlying conditions, which may be induced by drugs or systemic illness such as immunodeficiency or autoimmune conditions).[3,4]
  - The disease phase is based on the timing and continuation of symptoms (acute/newly diagnosed ITP: time of diagnosis to 3 months; persistent ITP: 3 to 12 months from initial diagnosis; chronic ITP: continuation of ITP after 12 months from initial diagnosis until its resolution).[3,5,6]
- Owing to such variability, diverse treatment strategies can be utilized for patients with ITP. According to clinical guidelines, steroids are the preferred initial treatment; however, a majority of patients relapse after an initial response and need a second line of treatment (like thrombopoietin receptor agonists [TPO RAs]).[7]
- Recent research has also reported an overuse of steroids in patients with ITP in the US,[8] which indicates potential unmet need. However, evidence is limited on the most common treatment patterns observed in the real-world, particularly in the chronic phase of the disease, in which patients are reported to experience pronounced disease severity.[9,10]
- The objective of this study was to assess treatment patterns during the chronic phase in adult patients with primary ITP, using a US claims database.

Methods

- The Komodo Health closed claims database (January 2015 to March 2023), containing complete medical and prescription claims information from >150 payers across all geographic regions of the US, was utilized for the analysis. The details of the study design are provided in **Figure 1**.

Figure 1. Study design



ITP, immune thrombocytopenia.

Time sequence-based cluster analysis

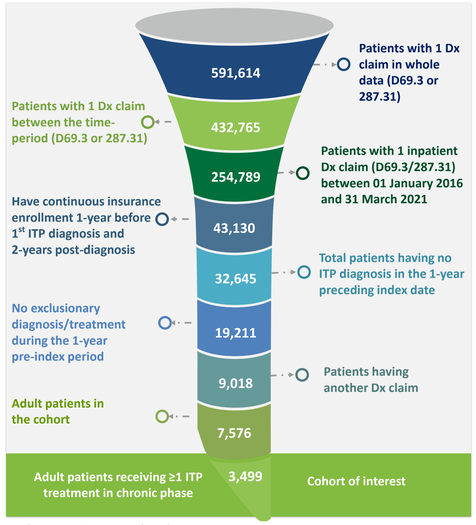
- The common treatment patterns among patients with primary chronic ITP were classified into clusters using time sequence-based cluster analysis (unsupervised learning method), which aims to group similar data based on their temporal patterns.[11]
- Clusters were identified using the 'Silhouette metric score', which assesses the quality of clustering by considering both intra-cluster cohesion and inter-cluster separation.[12]

Results

Patient selection

- After application of the inclusion criteria, 3,499 adults with primary ITP who received ≥1 ITP treatments during the chronic phase were included in the analysis (**Figure 2**).

Figure 2. Patient selection



Dx, diagnosis; ITP, immune thrombocytopenia.

Baseline demographics and characteristics of patients with primary ITP receiving ≥1 treatment during the chronic phase

- The mean (SD) age at index was 52.7 (17.6) years, with female predominance (n=2,174 [62%]; **Table 1**).
- The baseline demographics and characteristics were largely consistent with previously published data on real-world ITP populations in the US.[10]

Table 1. Baseline patient demographics and characteristics

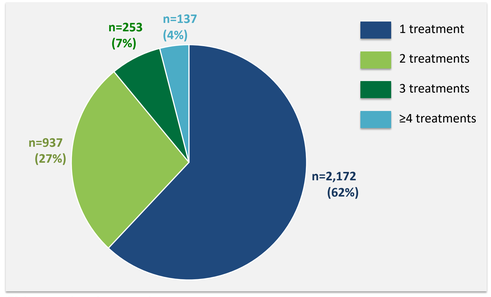
	N=3,499
Age, years, mean (SD)	52.7 (17.6)
Female, n (%)	2,174 (62)
Race, n (%)	
White	900 (26)
Hispanic or Latino	386 (11)
Black	140 (4)
Asian	50 (1)
Other	37 (1)
Multiple	35 (1)
Unknown	1,951 (56)
Insurance, n (%)	
Commercial	1,564 (45)
Medicare	554 (16)
Medicaid	509 (15)
Others[a]	872 (25)
CCI, mean (SD)	1.8 (2.2)
Comorbidities, n (%)	
Hypertension	1,703 (49)
Malaise and fatigue	1,107 (32)
Osteoarthritis	1,022 (29)
CPD	991 (28)
Diabetes without chronic complication	833 (24)
Thyroid disease	712 (20)
Dermatological condition	442 (13)
Diabetes with chronic complication	363 (10)
Ischemic heart disease	340 (10)

[a]Dual eligible, Self-insured, Multiple, Other, and Unknown.
CCI, Charlson Comorbidity Index; CPD, chronic pulmonary disease; SD, standard deviation.

Chronic phase treatment patterns

- Among 3,499 patients identified, almost 40% (n=1,327 [38%]) used ≥2 ITP treatment classes during the chronic phase (**Figure 3**).

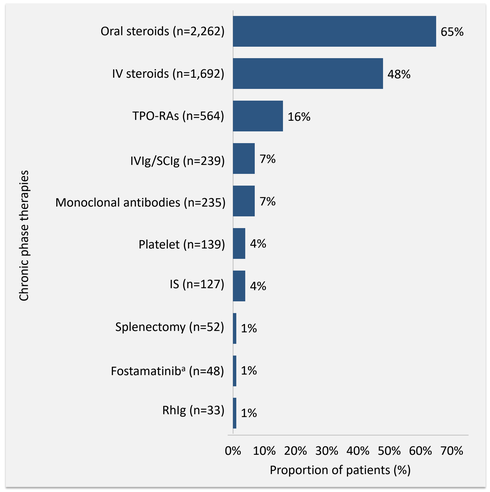
Figure 3. Proportion of patients with primary ITP utilizing 1, 2, 3, or ≥4 ITP treatment classes during the chronic phase (N=3,499)



ITP, immune thrombocytopenia.

- The most common treatments used at least once in the chronic phase were steroids (oral and intravenous [IV]), followed by TPO RAs (**Figure 4**).

Figure 4. Proportion of patients (out of N=3,499) who used each ITP treatment at least once during the chronic phase

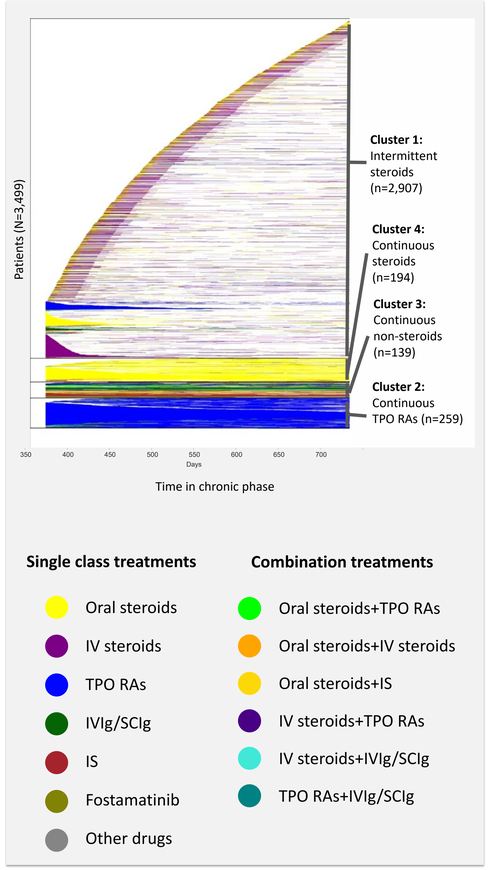


[a]Fostamatinib usage may have been limited among the study cohort (patient selection period: 2016-2021) due to its recent approval (FDA approval: April 2018).
IS, immunosuppressants; ITP, immune thrombocytopenia; IV, intravenous; IVIg, intravenous immunoglobulins; Rhlg, Rho(D) immunoglobulin; SCIg, subcutaneous immunoglobulins; TPO RAs, thrombopoietin receptor agonists.

Treatment pattern clusters

- Four common treatment clusters were identified among patients with primary chronic ITP. The most common was intermittent treatment with steroids (n=2,907 [83%]), followed by continuous treatment with TPO RAs (n=259 [7%]), continuous treatment with steroids (n=194 [6%]), and continuous treatment with non-steroids (n=139 [4%]; **Figures 5 and 6**).

Figure 5. Treatment clusters identified using time sequence-based cluster analysis



IS, immunosuppressants; IV, intravenous; IVIg, intravenous immunoglobulins; SCIg, subcutaneous immunoglobulin; TPO RAs, thrombopoietin receptor agonists.

Figure 6. Details on average treatment length of the four treatment clusters

Cluster 1: Intermittent steroids

Drug class	Average treatment length (days) n=2,907
Gap[a] (n=2,907)	174
Oral steroids (n=1,728)	16
IV steroids (n=1,453)	32
TPO RAs (n=232)	38
Oral steroids+IV steroids (n=327)	11
IVIg/SCIg (n=119)	28

Cluster 2: Continuous TPO RAs

Drug class	Average treatment length (days) n=259
Gap[a] (n=161)	29
Oral steroids (n=27)	9
IV steroids (n=21)	13
TPO RAs (n=259)	129
IV steroids+TPO RAs (n=59)	28
Oral steroids+TPO RAs (n=66)	17

Cluster 3: Continuous non-steroids

Drug class	Average treatment length (days) n=139
Gap[a] (n=82)	33
Oral steroids (n=38)	15
TPO RAs (n=40)	29
IS (n=63)	82
Oral steroids+IS (n=32)	56
IVIg/SCIg (n=32)	98

Cluster 4: Continuous steroids

Drug class	Average treatment length (days) n=194
Gap[a] (n=176)	45
Oral steroids (n=194)	83
IV steroids (n=29)	14
TPO RAs (n=10)	32
Oral steroids+IV steroids (n=43)	28
Oral steroids+TPO RAs (n=11)	16

[a]Gap refers to consecutive sets of days during which patients had no treatment corresponding to any of the ITP treatments assessed in this study during the 1-year observation period during the chronic ITP phase.
IS, immunosuppressants; IV, intravenous; IVIg, intravenous immunoglobulins; SCIg, subcutaneous immunoglobulin; TPO RAs, thrombopoietin receptor agonist.

Patient characteristics by treatment cluster

- A higher mean (SD) CCI score was observed among those continuously treated with steroids (cluster [C] 4: 2.5 [2.4]) or non-steroids (C3: 2.4 [2.1]) compared to others, indicating comorbidities were more common in these clusters.
- Patients continuously treated with TPO RAs (C2: 48%) or non-steroids (C3: 47%) had a substantially higher proportion using commercial insurance than those continuously treated with steroids (C4: 40%).
- Patients continuously treated with non-steroids (C3: 17%) or TPO RAs (C2: 9%) used a higher number (≥4) of chronic phase therapies compared to other clusters.

Conclusions

- **Among patients with primary chronic ITP, who predominantly utilized oral/IV steroids and TPO RAs during the chronic phase, the most common treatment pattern was intermittent treatment with steroids.**
- **The 3 other common treatment patterns in the chronic ITP phase were continuous treatment with TPO RAs, steroids, or non-steroids.**
- **Patients continuously treated with TPO RAs or non-steroids may experience pronounced burden, reflected by higher chronic phase treatment utilization as well as comorbidities.**
- **Future studies could help to better understand the impact of different treatment strategies on other burden in primary chronic ITP, including clinical, humanistic, and economic burden.**

Limitations

- This retrospective claims dataset did not capture key disease-related parameters such as severity. Thus, any clinical burden associated with treatment patterns were not evaluated.
- As the study population was identified from a US database, findings may not be generalized to patients from other geographical regions.

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