Characterization of adult patients with primary chronic immune thrombocytopenia using United States administrative claims data

Cécile Blein[a], Clémence Arvin-Berod[a], Syed Raza[a], Michael Yeakey[a], Arash Mahajerin[a], Dakshinamoorthy Amirthaganesan[b], Charlotte Ward[c], Mai Sato[d], Amit Goyal[e]

[a]argenx BVBA, Industriepark Zwijnaarde 7, Ghent, Belgium; [b]ZS Associates, Bengaluru, Karnataka, India; [c]ZS Associates, Two Bethesda Metro Center, Bethesda, MD, USA; [d]ZS Associates, 350 Fifth Avenue, New York, NY, USA; [e]ZS Associates, 210 Carnegie Center, Princeton, NJ, USA

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 or 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]
- When patients with different etiology and/or at different stages of disease are aggregated, identification of different degrees of burden and unmet needs can be challenging.
- Primary chronic ITP is associated with greater severity including higher hemorrhagic manifestations and treatment burden,[7,8] but current evidence is limited to identify other addressable unmet needs.
- This study was aimed to characterize adults with primary chronic ITP using patient data from 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

Key patient selection criteria

- ≥2 outpatient (30–365 days apart)/≥1 inpatient claim(s) associated with primary ITP (January 2016 to March 2021)
- ≥3 years continuous enrollment with no ITP diagnoses 1-year pre-first primary ITP claim
- Absence of diagnostic/treatment codes associated with secondary ITP
- ≥1 primary ITP claim during the chronic phase (365–730 days following first diagnosis), the first of which is considered the index date
- Patients aged ≥18 years at first ITP diagnosis

Study outcomes

- Characteristics of patients with primary chronic ITP
- Treatments used in primary chronic ITP
- Characteristics of treated versus untreated patients with primary chronic ITP

Statistical analysis

- Chi-square tests for categorical variables
- T-tests for continuous variables

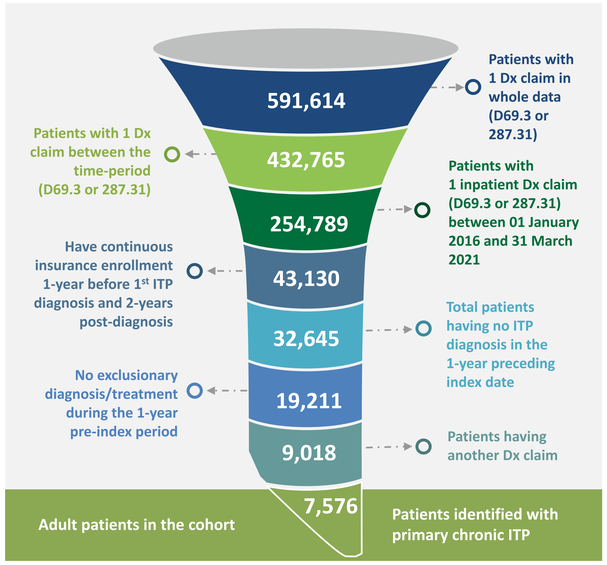
ITP, immune thrombocytopenia.

Results

Patient selection

- After application of the inclusion criteria, 7,576 adults with primary chronic ITP were included in the analysis (**Figure 2**).

Figure 2. Patient selection



Dx, diagnosis; ITP, immune thrombocytopenia.

Baseline patient demographics and characteristics

- The mean (SD) age at index was 52.5 (17.9) years, with female predominance (n=4,462 [59%]; **Table 1**).
- The baseline demographics and characteristics were largely consistent with previously published data on real-world ITP populations in the US.[9]

Table 1. Baseline patient demographics and characteristics

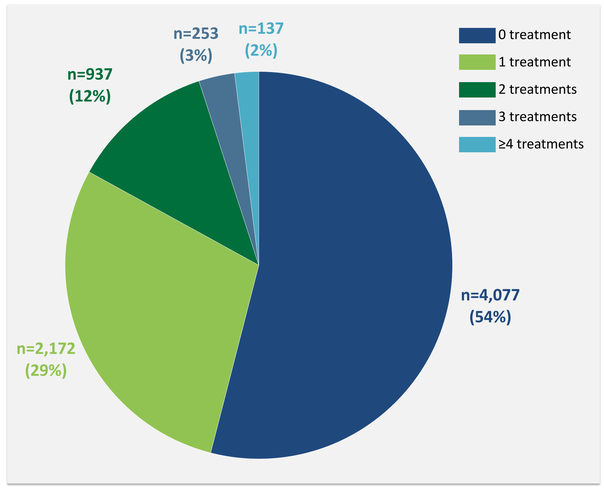
	N=7,576
Age, years, mean (SD)	52.5 (17.9)
Age, years, median (IQR)	54 (28)
Age, range, n (%)	
18–40	2,158 (28)
41–65	3,481 (46)
65+	1,937 (26)
Gender, n (%)	
Female	4,462 (59)
Male	3,114 (41)
Race, n (%)	
White	1,842 (24)
Hispanic or Latino	880 (12)
Black	275 (4)
Asian	105 (1)
Other	97 (1)
Multiple	50 (1)
Unknown	4,327 (57)
Insurance, n (%)	
Commercial	3,406 (45)
Medicare	1,215 (16)
Medicaid	1,051 (14)
Others[a]	1,904 (25)
CCI, mean (SD)	1.6 (2.0)
Comorbidities, n (%)	
Hypertension	3,432 (45)
Malaise and fatigue	2,089 (28)
Osteoarthritis	1,747 (23)
CPD	1,737 (23)
Diabetes without chronic complication	1,725 (23)
Thyroid disease	1,380 (18)
Dermatological condition	866 (11)
Diabetes with chronic complication	753 (10)

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

Treatments received for primary chronic ITP

- Overall, 4,077 (54%) patients were untreated in the chronic phase, whereas 3,499 (46%) patients received ≥1 ITP treatment (**Figure 3**).
- Among the 3,499 patients who were treated for primary chronic ITP, the majority (n=2,172 [62%]) had used a single treatment class as chronic phase therapy.

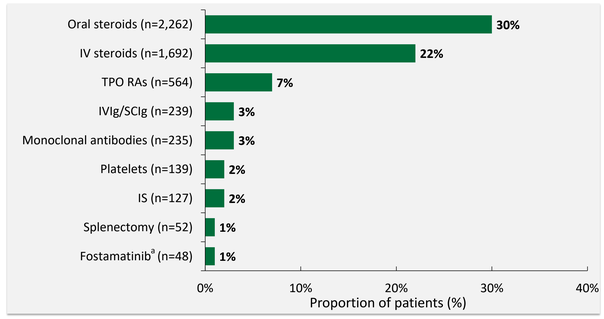
Figure 3. Number of ITP treatment classes utilized in chronic phase (N=7,576)



ITP, immune thrombocytopenia.

- Overall, oral (n=2,262; [30%]) and intravenous (IV) (n=1,692 [22%]) steroids were the most common treatments used in the chronic phase, followed by thrombopoietin receptor agonists (TPO RAs) (n=564, [7%]; **Figure 4**).

Figure 4. Proportion of patients (out of overall N=7,576) 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).
FDA, Food and Drug Administration; IS, immunosuppressants; IV, intravenous; IVIg, intravenous immunoglobulins; SCIg, subcutaneous immunoglobulins; TPO RAs, thrombopoietin receptor agonists.

Characteristics of treated and untreated patients with primary chronic ITP

- Between patients who were treated or untreated in the chronic phase, there were no significant differences in terms of age (p=0.39) and insurance status (p=0.47; **Table 2**).
- While differences in gender and race were statistically significant, their distribution was consistent across both cohorts.
- Patients in the treated cohort had a significantly higher Charlson Comorbidity Index (CCI) compared with those who did not receive treatment in the chronic phase, reflecting higher comorbidities (mean [SD] CCI: 1.8 [2.2] versus 1.4 [2.0], p<0.0001).

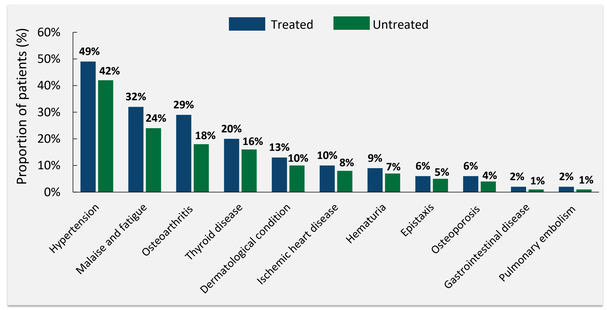
Table 2. Baseline demographics and characteristics of patients with primary ITP who were treated or untreated in the chronic phase

	Treated n=3,499	Untreated n=4,077	p-value
Age, years, mean (SD)	52.7 (17.6)	52.3 (18.2)	0.39
Age, years, median (IQR)	54 (26)	54 (30)	
Age, range, n (%)			
18–40	959 (27)	1,199 (29)	
41–65	1,670 (48)	1,811 (44)	
65+	870 (25)	1,067 (26)	
Gender, n (%)			<0.00001
Female	2,174 (62)	2,288 (56)	
Male	1,325 (38)	1,789 (44)	
Race, n (%)			0.00024
White	900 (26)	942 (23)	
Hispanic or Latino	386 (11)	494 (12)	
Black	140 (4)	135 (3)	
Asian	50 (1)	55 (1)	
Other	37 (1)	60 (1)	
Multiple	35 (1)	15 (0)	
Unknown	1,951 (56)	2,376 (58)	
Insurance, n (%)			0.468
Commercial	1,564 (45)	1,842 (45)	
Medicare	554 (16)	661 (16)	
Medicaid	509 (15)	542 (13)	
Others[a]	872 (24)	1,032 (26)	
CCI, mean (SD)	1.8 (2.2)	1.4 (2.0)	<0.0001

[a]Dual eligible, Self-insured, Multiple, Other, and Unknown.
CCI, Charlson Comorbidity Index; IQR, interquartile range; ITP, immune thrombocytopenia; SD, standard deviation.

- Consistent with CCI results, the proportion of patients with individual comorbidities including hypertension, malaise and fatigue, osteoarthritis, and thyroid disease were significantly higher in the treated cohort than the untreated cohort (p<0.00001; **Figure 5**).

Figure 5. Proportion of patients with comorbidities, stratified by those who were treated or untreated in the chronic ITP phase



For hypertension, malaise and fatigue, osteoarthritis, and thyroid disease, p<0.00001.
ITP, immune thrombocytopenia.

Conclusions

- **Overall, majority of the patients with primary chronic ITP were untreated in the chronic phase.**
- **Among patients with primary chronic ITP receiving treatment in the chronic phase, majority received oral/IV steroids and TPO RAs.**
- **Patients who utilized primary chronic ITP treatments, who were primarily receiving steroids or TPO RAs, had significantly higher comorbidities compared with those who were untreated in the chronic phase.**
- **Future studies enabling further investigation of treatment patterns and the associated cost/healthcare resource utilization (HCRU) burden are required to better understand the burden and unmet needs in primary chronic ITP.**

Limitations

- This retrospective claims dataset did not capture key disease-related parameters such as severity. Any potential clinical unmet needs among patients untreated for primary chronic ITP could not be assessed in this study as relevant clinical data were unavailable.
- As the study population was identified from a US database, the study findings may not be generalized to patients from other geographical regions.

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