Burden of illness for adults living with primary chronic immune thrombocytopenia in the United States

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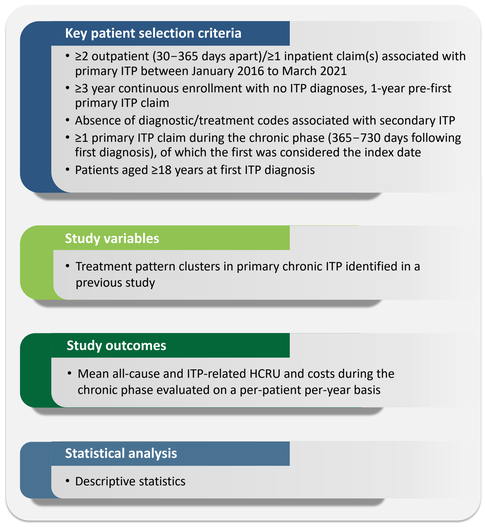
Introduction and Purpose

- Immune thrombocytopenic purpura (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) and secondary (caused by comorbid/underlying conditions, which may be induced by drugs or systemic illness such as immunodeficiency or autoimmune conditions).[1]
  - 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).[1]
- According to clinical guidelines, primary chronic ITP is managed with corticosteroids, intravenous immunoglobulin (IVIg), rituximab, thrombopoietin receptor agonists (TPO RAs), and splenectomy.[2] In our previous study, four major treatment clusters were identified in primary chronic ITP, of which the most common was intermittent treatment with steroids, followed by continuous treatment with TPO RAs, steroids, or non-steroids during the chronic phase.
- Healthcare resource utilization (HCRU) and costs contribute to overall economic burden affecting both patients and healthcare system, and better understanding its drivers can aid healthcare providers in selecting optimal management strategies.[3-5] In the case of primary chronic ITP, evaluating economic burden based on treatment patterns is crucial as costs and HCRU may be driven by different treatments.[5]
- This study aimed at assessing HCRU and cost among adults with primary chronic ITP in the United States (US), and explore any disparities observed based on their treatment strategies.

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 study design are provided in **Figure 1**.

Figure 1. Study design



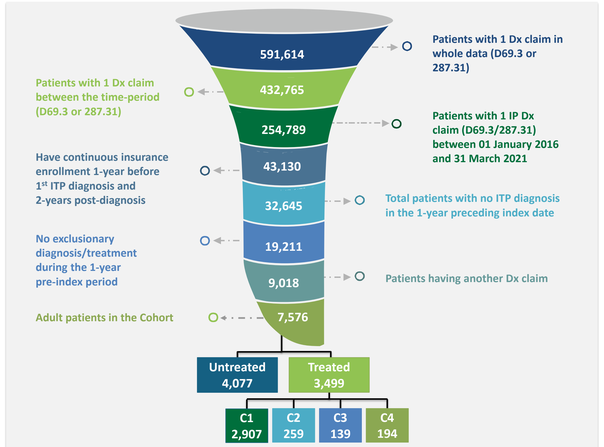
HCRU, healthcare resource utilization; ITP, immune thrombocytopenia.

Results

Patient selection

- Overall, 7,576 adult patients with primary chronic ITP were identified, of which 4,077 were untreated and 3,499 were treated for ITP during the chronic phase (**Figure 2**).
- Using time-sequence cluster analysis described in a previous study, the 3,499 treated patients were categorized into 4 treatment clusters according to their chronic phase treatment patterns:
  - Cluster 1 (C1): Intermittently treated with steroids (n=2,907)
  - Cluster 2 (C2): Continuously treated with TPO RAs (n=259)
  - Cluster 3 (C3): Continuously treated with non-steroids (n=139)
  - Cluster 4 (C4): Continuously treated with steroids (n=194)

Figure 2. Patient selection



C, cluster; Dx, diagnosis; IP, inpatient; ITP, immune thrombocytopenia.

Baseline patient demographics and characteristics

- The baseline patient demographics and clinical characteristics were comparable across sub-cohorts, with higher comorbidity burden observed among those receiving ITP treatments in the chronic phase (treated cohort; **Table 1**).

Table 1. Baseline patient demographics and characteristics

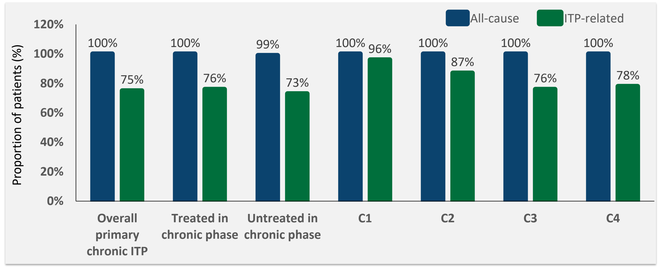
	Overall primary chronic ITP	Treated in chronic phase	Untreated in chronic phase	C1 (Int. steroids)	C2 (Continuous TPO RAs)	C3 (Continuous non-steroids)	C4 (Continuous steroids)
Patients, n	7,576	3,499	4,077	2,907	259	139	194
Age, years, mean (SD)	52.5 (17.9)	52.7 (17.6)	52.3 (18.2)	52.2 (17.7)	54.0 (18.0)	51.3 (16.0)	58.5 (16.1)
Female, n (%)	4,462 (59)	2,174 (62)	2,288 (56)	1,839 (63)	134 (52)	77 (55)	124 (64)
Insurance, n (%)							
Commercial	3,406 (45)	1,564 (45)	1,842 (45)	1,297 (45)	124 (48)	65 (47)	78 (40)
Medicare	1,215 (16)	554 (16)	661(16)	453 (16)	41 (16)	17 (12)	43 (22)
Medicaid	1,051 (14)	509 (15)	542 (13)	424 (15)	40 (15)	17 (12)	28 (14)
Other[a]	1,883 (25)	862 (23)	1,021 (25)	728 (25)	50 (19)	40 (28)	44 (23)
CCI, mean (SD)	1.6 (2.1)	1.8 (2.2)	1.4 (2.0)	1.8 (2.2)	1.8 (2.1)	2.4 (2.1)	2.5 (2.4)
Comorbidities, n (%)							
Hypertension	3,432 (45)	1,703 (49)	1,729 (42)	1,369 (47)	128 (49)	83 (60)	123 (63)
Malaise and fatigue	2,089 (28)	1,107 (32)	982 (24)	923 (32)	64 (25)	53 (38)	67 (35)
Osteoarthritis	1,747 (23)	1,022 (29)	725 (18)	849 (29)	68 (26)	41 (29)	64 (33)
CPD	1,737 (23)	991 (28)	746 (18)	815 (28)	60 (23)	45 (32)	71 (37)
Diabetes without chronic complication	1,725 (23)	833 (24)	892 (22)	667 (23)	79 (31)	41 (29)	46 (24)
Thyroid disease	1,380 (18)	712 (20)	668 (16)	609 (21)	40 (15)	27 (19)	36 (19)
Dermatological condition	866 (11)	442 (13)	424 (10)	356 (12)	45 (17)	20 (14)	21 (11)
Diabetes with chronic complication	753 (10)	363 (10)	390 (10)	278 (10)	44 (17)	17 (12)	24 (12)

[a]Self-insured, Multiple, Unknown.
C, clusters; CCI, Charlson Comorbidity Index; CPD, chronic pulmonary disease; Int, intermittent; SD, standard deviation; TPO RAs, thrombopoietin receptor agonists.

HCRU (all-cause and ITP-related) by cohort

- Overall, mean all-cause HCRU during the chronic phase was considerable with 75% of patients having at least 1 ITP-related outpatient visit **(Figure 3)**.
- While rare compared with outpatient visits, a considerable proportion of patients had at least 1 inpatient and/or emergency room visit in the chronic phase (**Table 2 and Figure 3**).

Figure 3. All-cause and ITP-related outpatient visits (patients with ≥1 visit count)



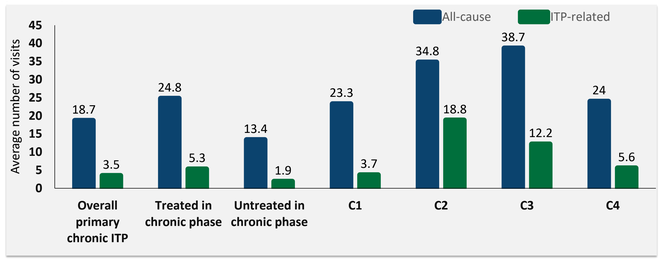
C, cluster; ITP, immune thrombocytopenia.

Table 2. All-cause and ITP-related inpatient, emergency room, and observation visits (patients with ≥1 visit count)

		IP visits, n (%)	ER visits, n (%)	Observation visits, n (%)
Overall primary chronic ITP	All-cause	1,718 (23)	2,212 (29)	117 (2)
	ITP-related	632 (8)	159 (2)	17 (0)
Treated in chronic phase	All-cause	599 (15)	875 (21)	41 (1)
	ITP-related	145 (4)	37 (1)	5 (0)
Untreated in chronic phase	All-cause	932 (32)	1,141 (39)	67 (2)
	ITP-related	395 (14)	97 (3)	9 (0)
C1	All-cause	66 (25)	71 (27)	2 (1)
	ITP-related	32 (2)	12 (5)	0 (0)
C2	All-cause	52 (37)	56 (40)	5 (4)
	ITP-related	30 (22)	3 (2)	2 (1)
C3	All-cause	69 (36)	69 (36)	2 (1)
	ITP-related	30 (15)	10 (5)	1 (1)
C4	All-cause	1,119 (32)	1,337 (38)	76 (2)
	ITP-related	487 (14)	122 (3)	12 (0)

C, cluster; ER, emergency room; IP, inpatient; ITP, immune thrombocytopenia.

Figure 4. All-cause and ITP-related outpatient visits (average number of visits per patient)



C, cluster; ITP, immune thrombocytopenia.

- From the perspective of average number of visits per patient, those continuously treated with TPO RAs (C2) had the highest outpatient visit burden, with 5.4-fold higher (18.8 visits) mean visits compared with the overall cohort (3.5 visits; **Figure 4 and Table 3**). This may be attributed in part to certain TPO RAs requiring administration in an outpatient setting.

Table 3. All-cause and ITP-related inpatient, emergency room, and observation visits (average number of visits per patient)

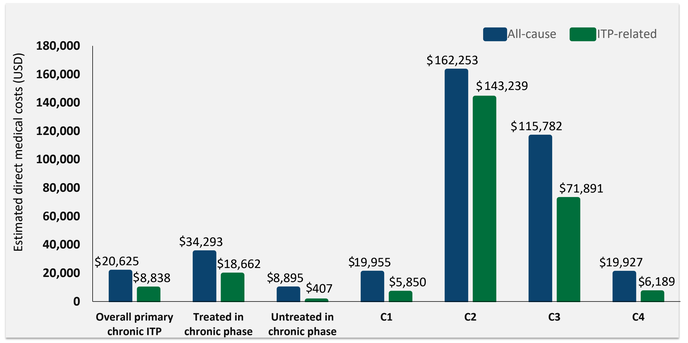
		IP visits, n (%)	ER visits, n (%)	Observation visits, n (%)
Overall primary chronic ITP	All-cause	0.5 (1.5)	0.6 (1.7)	0.0 (0.2)
	ITP-related	0.1 (0.4)	0.0 (0.2)	0.0 (0.1)
Treated in chronic phase	All-cause	0.3 (1.1)	0.4 (1.1)	0.0 (0.2)
	ITP-related	0.0 (0.2)	0.0 (0.1)	0.0 (0.1)
Untreated in chronic phase	All-cause	0.7 (1.8)	1.0 (2.2)	0.0 (0.3)
	ITP-related	0.2 (0.5)	0.0 (0.2)	0.0 (0.1)
C1	All-cause	0.4 (1.0)	0.5 (1.2)	0.0 (0.2)
	ITP-related	0.2 (0.5)	0.1 (0.3)	0.0 (0.0)
C2	All-cause	1.0 (1.8)	1.0 (1.7)	0.1 (0.6)
	ITP-related	0.5 (1.2)	0.0 (0.2)	0.1 (0.5)
C3	All-cause	0.8 (1.6)	1.0 (1.9)	0.0 (0.2)
	ITP-related	0.2 (0.8)	0.1 (0.3)	0.0 (0.1)
C4	All-cause	0.7 (1.7)	0.9 (2.1)	0.0 (0.3)
	ITP-related	0.2 (0.6)	0.0 (0.2)	0.0 (0.1)

C, cluster; ER, emergency room; IP, inpatient; ITP, immune thrombocytopenia.

Direct costs (all-cause and ITP-related) by cohort

- Overall, all-cause mean direct costs (medical and pharmacy) were $20,625, and ITP-related costs were $8,838 per-patient per-year (**Figure 5**).
- Mean all-cause costs were nearly 4-fold greater for patients receiving chronic ITP treatment ($34,293) versus those who were not treated ($8,895).
- In patients continuously treated with TPO RAs (C2), mean all-cause costs were nearly 8-fold higher ($162,253) compared with the overall cohort ($20,625).
- The significant increase in costs among patients continuously treated with TPO RAs (C2) and non-steroids (C3) was majorly driven by direct costs of TPO RAs and IVIg.

Figure 5. Estimated total direct costs (medical and pharmacy) for patients with primary chronic ITP during the chronic phase (all-cause and ITP-related)



C, cluster; ITP, immune thrombocytopenia; USD, United States dollar.

Conclusions

- **Considerable unmet need was identified in primary chronic ITP regardless of treatment pattern in the chronic phase, reflected by high HCRU and costs.**
- **Economic burden was markedly pronounced for those continuously treated with TPO RAs during the chronic phase. Further studies are required to evaluate potential unmet need associated with this group, including clinical and humanistic burden.**
- **These results highlight the necessity for targeted interventions that can reduce HCRU burden and costs associated with primary chronic ITP. Further research incorporating longitudinal data and comprehensive cost assessments is warranted to provide better understanding of the economic impact of ITP and inform healthcare decision-making.**

Limitations

- The study population included adults with primary chronic ITP in the US, limiting the generalizability of the findings to other populations or healthcare systems.
- As the study utilized a retrospective claims dataset, it did not capture disease severity, regional variations in healthcare practices, and access to treatment, which may influence the cost and HCRU.

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